

BELLICUM PHARMACEUTICALS, INC

Form 424B4

December 18, 2014

[Table of Contents](#)

Filed pursuant to Rule 424(b)(4)  
Registration Nos. 333-200328 and 333-201031

## PROSPECTUS

7,350,000 Shares

### Common Stock

Bellicum Pharmaceuticals, Inc. is offering 7,350,000 shares of its common stock. This is our initial public offering and no public market currently exists for our shares. The initial public offering price of our common stock is \$19.00 per share.

Our common stock has been approved for listing on The NASDAQ Global Market under the symbol BLCM.

We are an emerging growth company as that term is used in the Jumpstart Our Business Startups Act of 2012 and, as such, have elected to comply with certain reduced public company reporting requirements for this prospectus and future filings.

Investing in our common stock involves risks. See [Risk Factors](#) beginning on page 12.

Neither the Securities and Exchange Commission nor any state securities commission has approved or disapproved of these securities, or determined if this prospectus is truthful or complete. Any representation to the contrary is a criminal offense.

	PER SHARE	TOTAL
Public Offering Price	\$ 19.00	\$ 139,650,000
Underwriting Discounts and Commissions <sup>(1)</sup>	\$ 1.33	\$ 9,775,500
Proceeds to Bellicum Pharmaceuticals, Inc. (before expenses)	\$ 17.67	\$ 129,874,500

(1) We have agreed to reimburse the underwriters for certain expenses. See Underwriting. Certain of our existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering.

We have granted the underwriters an option for a period of 30 days to purchase up to an additional 1,102,500 shares of common stock. If the underwriters exercise the option in full, the total underwriting discounts and commissions payable by us will be \$11,241,825, and the total proceeds to us, before expenses will be \$149,355,675.

The underwriters expect to deliver the shares of common stock to purchasers on or about December 23, 2014.

*Joint Book-Running Managers*

**Jefferies**

**Citigroup**  
*Co-Manager*

**Piper Jaffray**

**Trout Capital**

Prospectus dated December 17, 2014

Table of Contents

TABLE OF CONTENTS

	<b>PAGE</b>
<u>Prospectus Summary</u>	1
<u>Risk Factors</u>	12
<u>Special Note Regarding Forward-Looking Statements</u>	45
<u>Use Of Proceeds</u>	46
<u>Dividend Policy</u>	47
<u>Capitalization</u>	48
<u>Dilution</u>	51
<u>Selected Financial Data</u>	54
<u>Management's Discussion And Analysis Of Financial Condition And Results Of Operations</u>	56
<u>Business</u>	67
<u>Management</u>	113
<u>Executive And Director Compensation</u>	120
<u>Certain Relationships And Related Party Transactions</u>	136
<u>Principal Stockholders</u>	143
<u>Description Of Capital Stock</u>	146
<u>Shares Eligible For Future Sale</u>	151
<u>Material U.S. Federal Income Tax Consequences To Non-U.S. Holders Of Our Common Stock</u>	153
<u>Underwriting</u>	156
<u>Notice to Investors</u>	160
<u>Legal Matters</u>	163

<u>Experts</u>	163
<u>Where You Can Find Additional Information</u>	163
<u>Index to Financial Statements</u>	F-1

**Table of Contents**

Neither we nor any of the underwriters has authorized anyone to provide you with information different from, or in addition to, that contained in this prospectus or any free writing prospectus prepared by or on behalf of us or to which we may have referred you in connection with this offering. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give you. Neither we nor any of the underwriters is making an offer to sell or seeking offers to buy these securities in any jurisdiction where or to any person to whom the offer or sale is not permitted. The information in this prospectus is accurate only as of the date on the front cover of this prospectus, regardless of the time of delivery of this prospectus or of any sale of shares of our common stock and the information in any free writing prospectus that we may provide you in connection with this offering is accurate only as of the date of that free writing prospectus. Our business, financial condition, results of operations and future growth prospects may have changed since those dates.

**Through and including January 11, 2015 (25 days after the date of this prospectus), all dealers that effect transactions in these securities, whether or not participating in this offering, may be required to deliver a prospectus. This delivery is in addition to a dealer's obligation to deliver a prospectus when acting as an underwriter and with respect to their unsold allotments or subscriptions.**

This prospectus includes statistical and other industry and market data that we obtained from industry publications and research, surveys and studies conducted by third parties. Industry publications and third-party research, surveys and studies generally indicate that their information has been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information.

We have obtained registered trademarks for Bellicum<sup>®</sup>, CaspaCIDE<sup>®</sup> and DeCIDE<sup>®</sup> based on an intent to use in the United States. We are currently prosecuting registrations for the GoCAR-T and GOCART marks. This prospectus contains references to our trademarks and to trademarks belonging to other entities. Solely for convenience, trademarks and trade names referred to in this prospectus, including logos, artwork and other visual displays, may appear without the <sup>®</sup> or TM symbols, but such references are not intended to indicate, in any way, that we will not assert, to the fullest extent under applicable law, our rights or the rights of the applicable licensor to these trademarks and trade names. We do not intend our use or display of other companies' trade names or trademarks to imply a relationship with, or endorsement or sponsorship of us by, any other companies.

**Table of Contents**

**PROSPECTUS SUMMARY**

*This summary highlights information contained in other parts of this prospectus. Because it is only a summary, it does not contain all of the information that you should consider before investing in shares of our common stock and it is qualified in its entirety by, and should be read in conjunction with, the more detailed information appearing elsewhere in this prospectus. You should read the entire prospectus carefully, especially Risk Factors beginning on page 12 and our financial statements and the related notes, before deciding to buy shares of our common stock.*

*Unless the context requires otherwise, references in this prospectus to we, us and our refer to Bellicum Pharmaceuticals, Inc.*

**Overview**

We are a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. Cellular immunotherapy has the potential to transform medicine by harnessing immune cells, principally T cells, to attack and eliminate harmful diseased cells in the body. Unlike traditional small molecule and biologic therapies which are predictably metabolized and eliminated from the body, cellular immunotherapies are unpredictable and uncontrollable. We are using our proprietary Chemical Induction of Dimerization, or CID, technology platform to engineer and then control components of the immune system in real time. By incorporating our CID platform, our product candidates may offer better safety and efficacy outcomes than are seen with current cellular immunotherapies.

Our lead clinical product candidate, BPX-501, is an adjunct T-cell therapy administered after allogeneic hematopoietic stem cell transplantation, or HSCT, and is currently being evaluated in multiple Phase 1/2 clinical trials. Our next clinical product candidate, BPX-201, is a dendritic cell cancer vaccine in a Phase 1 clinical trial for the treatment of metastatic castrate-resistant prostate cancer, or mCRPC, targeting the prostate-specific membrane antigen, or PSMA. Dendritic cells are specialized cells that are key regulators of the immune system that process and present antigens on the cell surface to T cells in order to activate the T cells. We are also focused on developing next-generation chimeric antigen receptor, or CAR, T-cell therapies and T-cell receptor, or TCR, therapies and are planning to advance several product candidates into human clinical trials, including: (1) BPX-401, a CAR-T product candidate for hematological cancers that express the CD19 antigen, (2) BPX-601, a CAR-T product candidate for solid tumors overexpressing the prostate stem cell antigen, or PSCA, and (3) BPX-701, a TCR product candidate for solid tumors expressing the preferentially-expressed antigen in melanoma, or PRAME.

**Table of Contents**

Our product candidate pipeline is set forth below:

**Our Proprietary CID Technology Platform**

We are developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including HSCT, CAR T cell therapy, and dendritic cell vaccines. HSCT, also known as bone marrow transplantation, has for decades been curative for many patients with hematological cancers or orphan inherited blood disorders. However, application of HSCT is limited by graft-versus-host-disease, or GvHD, a condition in which the transplanted immune cells recognize the host cells as foreign and attack them. Since the transplanted cells can persist indefinitely, GvHD does not resolve by itself and is a major cause of transplant-related morbidity and mortality. CAR T cell therapy is an innovative approach in which a patient's T cells are genetically modified to carry CARs, which redirect the T cells against cancer cells. While high objective response rates have been reported in some hematological malignancies, serious and sometimes fatal toxicities have arisen in patients treated with CAR T cell therapies. These toxicities include instances in which the CAR T cells have caused high levels of cytokines due to over-activation, referred to as cytokine release syndrome, frequent transient neurologic toxicities and cases in which they have attacked healthy organs instead of the targeted tumor, leading to death. In solid tumors, where the behavior of CAR T cells is particularly unpredictable and results have been inconsistent, researchers are developing enhanced CAR T cell approaches called armored CARs that raise even greater safety concerns. Lastly, despite the integral role that dendritic cells play in the immune system, they are difficult to activate appropriately and as a result their use has delivered only modest therapeutic benefit.

**Table of Contents**

Our proprietary CID technology is designed to address these challenges. Events inside a cell are controlled by cascades of specialized signaling proteins. CID consists of molecular switches, modified forms of these signaling proteins, which are triggered inside the patient by infusion of a small molecule, rimiducid (AP1903), instead of by natural upstream signals. We include these molecular switches in the appropriate immune cells and deliver the cells to the patient in the manner of conventional cellular immunotherapy. We have developed two such switches: a safety switch designed to initiate programmed cell death, or apoptosis, of the immunotherapy cells, and an activation switch designed to stimulate activation and in some cases proliferation of the immunotherapy cells. Each of our technologies incorporates one of these switches, for enhanced, real-time control of safety and efficacy:

- n **CaspaCIDE** is our safety switch, incorporated into our HSCT and TCR product candidates, where it is inactive unless the patient experiences a serious side effect. In that event, rimiducid is administered to fully or partially eliminate the cells, with the goal of terminating or attenuating the therapy and resolving the serious side effect.
- n **CIDeCAR** consists of CAR T cells modified to include our CaspaCIDE safety switch and in which the CAR incorporates the signaling domains of two proteins, MyD88 and CD40. Together, these form our proprietary dual co-stimulatory domain, MC, which is designed to activate T cells in the presence of cancer cells more potently than co-stimulatory molecules CD28 and 4-1BB, which are used in current CAR T cell therapy. Incorporation of CaspaCIDE in a CIDeCAR product candidate is intended to allow the enhanced potency of MC co-stimulation to be deployed safely in patients.
- n **GoCAR-T** consists of CAR T cells that are modified to include the proprietary dual co-stimulatory domain, MC. In contrast to CIDeCAR, MC is structured in GoCAR-T as a molecular switch, separate from the chimeric antigen receptor, which itself contains no co-stimulatory domains. GoCAR-T is designed to allow control of the activation and proliferation of the CAR T cells through the scheduled administration of a course of rimiducid infusions that may continue until the desired patient outcome is achieved. In the event of emergence of side effects, the level of activation of the GoCAR-T cells is designed to be attenuated by reducing the rimiducid administration schedule.
- n **DeCIDE** consists of dendritic cells that are modified to include the same MC switch used in GoCAR-T. Upon exposure to rimiducid, dendritic cells containing DeCIDE become highly activated in a process that is less susceptible to being turned off by the immune system's natural inhibitory processes. By administering rimiducid after the patient has been vaccinated and the dendritic cells have had time to migrate to the draining lymph nodes, our DeCIDE product candidates are designed to be activated in a potent and long-lasting manner.

By incorporating our novel switch technologies, we are developing product candidates with the potential to elicit positive clinical outcomes and ultimately change the treatment paradigm in various areas of cellular immunotherapy. Our clinical product candidates, each of which is a combination product of genetically modified immune cells and rimiducid, are described below.

n



**BPX-501.** We are developing a CaspaCIDE product candidate, BPX-501, as an adjunct T-cell therapy administered after allogeneic HSCT, using donor stem cells. In a typical allogeneic HSCT procedure, a patient receives a full complement of immune cells including both donor stem cells and donor T cells. T cells in the transplant often cause serious and potentially fatal side effects, such as GvHD. BPX-501 is designed to decrease the risk of including T cells with the transplant by enabling the elimination of donor T cells through the triggering of the CaspaCIDE safety switch upon emergence of GvHD. In a 10-patient Phase 1 clinical trial with CaspaCIDE modified T cells, conducted by an academic collaborator, four patients developed GvHD after donor T-cell infusion. A single dose of rimiducid rapidly eliminated over 90% of the modified T cells and resolved GvHD in all four patients without recurrence of GvHD. These findings have been replicated in preliminary data from three patients in a second clinical trial of CaspaCIDE-modified T cells. BPX-501 is currently being evaluated in multiple Phase 1/2 clinical trials in the United States and Europe, with the first top-line data expected in the second half of 2015.

---

## Table of Contents

n **BPX-201.** We are developing a DeCIDE product candidate, BPX-201, as a dendritic cell cancer vaccine made from the patient's own white blood cells, designed to treat mCRPC. It targets the prostate specific membrane antigen, or PSMA, and uses our DeCIDE activation switch technology. BPX-201 is currently being evaluated in an 18-patient Phase 1 clinical trial for mCRPC. We are evaluating opportunities for BPX-201 in combination with other cancer immunotherapies, such as checkpoint inhibitors, which are antibodies designed to block certain inhibitory receptors on the surface of T cells, and thus potentiate the T cells' ability to promote an immune response against cancer. We believe that the increased numbers of PSMA-specific T cells migrating to deposits of prostate cancer in the body that BPX-201 is designed to generate may serve as a substrate for checkpoint inhibitors, resulting in a synergistic, more potent anti-cancer immune response. In addition, our preclinical product candidates are designed to overcome the current limitations of CAR-T and TCR therapies and include the following:

- n **BPX-401.** We are developing a CIDE CAR product candidate, BPX-401, as a next-generation CAR T cell therapy for hematological cancers that express the CD19 antigen. CD19 is an antigen expressed in many hematological cancers, including acute lymphocytic leukemia, or ALL, chronic lymphocytic leukemia, or CLL, and certain non-Hodgkin's lymphomas. We believe that, while the activity of CAR T cell therapy has been demonstrated in early-stage clinical trials by third party researchers in these indications, safety issues, such as cytokine release syndrome, a systemic inflammatory response that is produced by elevated levels of cytokines that are associated with T-cell activation and proliferation, remain a major concern, which may be addressed by BPX-401.
- n **BPX-601.** We are developing a GoCAR-T product candidate, BPX-601, for solid tumors overexpressing PSCA, such as some prostate, pancreatic, bladder, esophageal and gastric cancers. We have obtained positive proof-of-principle data in an animal pancreatic tumor model, which we believe validate BPX-601's activity and rimiducid's ability to modulate therapeutic effect.
- n **BPX-701.** We are developing a CaspaCIDE TCR product candidate, BPX-701, in collaboration with Leiden University Medical Center, initially for the treatment of PRAME-expressing melanoma, sarcomas and neuroblastoma. Based on *in vitro* studies, BPX-701 has demonstrated strong affinity to panels of cancer cells presenting PRAME peptides and low affinity to non-tumor cells. In other *in vitro* studies, rimiducid administration has shown the ability to eliminate BPX-701 cells.

We expect to file investigational new drug applications, or INDs, for BPX-701 in the second half of 2015 and for BPX-401 and BPX-601 in 2016. Our IND-enabling activities for each of these preclinical product candidates include manufacturing key components and developing a robust process to produce cell products that comply with regulations of the U.S. Food and Drug Administration, or FDA, and other regulatory agencies. We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality and purity. This process is being implemented by our third-party contract manufacturers to produce BPX-501 for our clinical trials. We expect to leverage our resources, capabilities and expertise for the manufacture of our CAR-T and TCR product candidates.

## **Strategy**

Our goal is to become a leading innovator in the field of cellular immunotherapy by maximizing the inherent potential of this therapeutic modality and developing medicines with a differentiated combination of safety and efficacy. The key elements of our strategy to achieve this goal are as follows:

- n ***Pursue a broad development strategy that will maximize the market potential of BPX-501.*** We believe that BPX-501 will enable physicians to maximize the benefits of adjunct T-cell therapy for allogeneic HSCT, such as immune system recovery, prevention or treatment of relapse of underlying disease and improvement in stem cell engraftment, while mitigating safety issues associated with the therapy. Based on these attributes, BPX-501 may serve an integral role in the treatment paradigm for allogeneic HSCT in various diseases and increase the overall patient eligibility for the procedure. In

---

**Table of Contents**

order to make BPX-501 accessible to a broad group of patients and maximize the market potential of this product candidate, we are conducting multiple Phase 1/2 clinical trials that include U.S. and European protocols, adult and pediatric patients and different indications and usage of BPX-501. We expect to report data from these clinical trials and discuss registration trial design at an end-of-Phase 2 meeting with the FDA and European regulatory authorities in the first half of 2016.

- n ***Focus on developing proprietary CAR-T and TCR product candidates with an improved safety and efficacy profile.*** We intend to build a robust clinical pipeline of our own novel CAR-T and TCR product candidates, which incorporate our proprietary switch technologies, CIDE CAR, GoCAR-T and CaspaCIDE, and focus on indications in which current CAR-T and TCR therapies have significant shortcomings. To this end, we are developing BPX-401 for hematological cancers expressing the CD19 antigen, BPX-601 for solid tumors overexpressing PSCA and BPX-701 for solid tumors expressing PRAME. We believe that these product candidates may address serious safety concerns associated with conventional CAR-T and TCR therapies and achieve higher overall potency and efficacy, thereby widening the therapeutic window compared to other CAR-T and TCR product candidates. We intend to dedicate significant resources in the near term to advance BPX-401, BPX-601 and BPX-701 as well as our other product candidates toward human proof-of-concept data.
  
- n ***Selectively pursue partnerships and collaborations.*** Although our priority is to develop internal product candidates, we may pursue opportunistic partnerships and collaborations for our technologies, including CaspaCIDE and DeCIDE. In indications outside of our interest or expertise, we may structure transactions in which our molecular switches are incorporated into our partners' CAR-T or TCR product candidates. We intend to build on our existing strong relationships with premier cancer research centers around the world to identify new opportunities and position our company at the forefront of innovations in the field of cellular immunotherapy.
  
- n ***Continue to innovate around our proprietary CID platform.*** We believe that our CID platform can be further leveraged to discover other novel technologies and therapeutic applications to capitalize on additional market opportunities. We intend to evaluate BPX-201 and other product candidates based on our DeCIDE technology in combination with other cancer immunotherapy such as checkpoint inhibitors. We are also developing new switches and two-switch systems to provide greater control over cellular immunotherapy.
  
- n ***Continue to strengthen our intellectual property profile.*** We believe that having a comprehensive patent estate that provides strong barriers to entry is critical to the success of our business. As such, our management team has made a concerted effort to develop and secure our intellectual property since inception. We currently own or have exclusive licenses to 74 issued patents and 45 pending patent applications. These patents and patent applications include composition and/or method of use claims in the United States, Europe and other jurisdictions. We intend to continue to strengthen our patent estate by developing and filing for patents on various aspects of our technologies and product candidates as well as through in-licensing activities with research institutions and other biopharmaceutical companies.
  
- n ***Become a fully integrated cellular immunotherapy company.*** Developing product candidates for cellular immunotherapy is complex and requires significant in-house capabilities in various areas of drug

development. Over the years we have built a solid foundation from which to fulfill the highly demanding clinical and regulatory requirements of genetically modified cellular immunotherapy, with expertise in research and discovery, clinical trial management, data analysis, manufacturing, quality assurance and regulatory affairs. We intend to use a portion of the net proceeds from this offering to continue hiring staff with necessary expertise and investing in infrastructure to support the growth of our clinical development activities and to enable us to become the leading cellular immunotherapy company.

## **Table of Contents**

### **Recent Developments**

To enable further development of our proprietary technology and product candidates, we completed a private placement of \$55 million of Series C convertible preferred stock in August 2014. Investors in the transaction included, among others, Baker Brothers, RA Capital Management, LLC, Perceptive Advisors, LLC, Jennison Associates LLC (on behalf of certain clients), Sabby Capital, LLC, Ridgeback Capital Management, venBio Select, Redmile Group, LLC and AJU IB Investment, as well as our then current investors, including AVG Ventures and Remeditex Ventures.

Certain aspects of our platform technology are licensed from ARIAD Pharmaceuticals, Inc., or ARIAD. In October 2014, we amended our license agreement with ARIAD, pursuant to which we agreed to pay ARIAD \$50 million in three tranches payments, including an initial payment of \$15 million in connection with the execution of the amendment. In exchange, ARIAD gave us a fully paid-up license to its cell-signaling technology and agreed to return of all of the 677,463 shares of our common stock currently held by ARIAD at the time of the second tranche payment. The scope of the license and the field of use were also expanded as part of the amendment. The amended agreement gives us a worldwide exclusive license to ARIAD's cell-signaling technology for broad use in human cell therapies for all diseases on a royalty- and milestone-free basis. See [Business Our License Agreements](#).

### **Risks Associated With Our Business**

Our business is subject to numerous risks, as more fully described in the section entitled [Risk Factors](#) immediately following this prospectus summary. You should read these risks before you invest in our common stock. We may be unable, for many reasons, including those that are beyond our control, to implement our business strategy. In particular, risks associated with our business include:

- n We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.
- n Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty. We have never generated any revenue from product sales and may never be profitable.
- n We have concentrated our therapeutic product research and development efforts on our CID platform, a novel therapeutic approach, and our future success depends on the successful development of this therapeutic approach.
- n Our clinical trials may fail to demonstrate adequately the safety and efficacy of any of our product candidates, which would prevent or delay regulatory approval and commercialization.
- n We may not be successful in our efforts to use and expand our CID platform to build a pipeline of product candidates and develop marketable products.

n

The FDA regulatory approval process is lengthy and time-consuming, and we may experience significant delays in the clinical development and regulatory approval of our product candidates. Further, the FDA may disagree with our regulatory plans and we may fail to obtain regulatory approval of our product candidates.

- n Due to the novel nature of our technology and the potential for our product candidates to offer therapeutic benefit in a single administration, we face uncertainty related to pricing and reimbursement for these product candidates. Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our product candidates profitably.
  
- n We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.
  
- n We have identified a material weakness in our internal control over financial reporting. If our internal control over financial reporting is not effective, we may not be able to accurately report our financial results or file our periodic reports in a timely manner, which may cause investors to lose confidence in our reported financial information and may lead to a decline in our stock price.

## **Table of Contents**

### **Corporate Information**

We were incorporated in Delaware in July 2004. Our principal executive offices are located at 2130 W. Holcombe Blvd., Ste. 800, Houston, Texas and our telephone number is (832) 384-1100. Our corporate website address is [www.bellicum.com](http://www.bellicum.com). Information contained on or accessible through our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

### **Implications of Being an Emerging Growth Company**

As a company with less than \$1.0 billion in revenue during our last fiscal year, we qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. An emerging growth company may take advantage of reduced reporting requirements that are otherwise applicable to public companies. These provisions include, but are not limited to:

- n being permitted to present only two years of audited financial statements and only two years of related Management's Discussion and Analysis of Financial Condition and Results of Operations in this prospectus;
- n not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended;
- n reduced disclosure obligations regarding executive compensation in our periodic reports, proxy statements and registration statements; and
- n exemptions from the requirements of holding a nonbinding advisory vote on executive compensation and stockholder approval of any golden parachute payments not previously approved.

We may use these provisions until the last day of our fiscal year following the fifth anniversary of the closing of this offering. However, if certain events occur prior to the end of such five-year period, including if we become a large accelerated filer, our annual gross revenue exceed \$1.0 billion or we issue more than \$1.0 billion of non-convertible debt in any three-year period, we will cease to be an emerging growth company prior to the end of such five-year period.

We have elected to take advantage of certain of the reduced disclosure obligations in the registration statement of which this prospectus is a part and may elect to take advantage of other reduced reporting requirements in future filings. As a result, the information that we provide to our stockholders may be different than you might receive from other public reporting companies in which you hold equity interests.

The JOBS Act provides that an emerging growth company can take advantage of an extended transition period for complying with new or revised accounting standards. We have irrevocably elected not to avail ourselves of this exemption and, therefore, we will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies.





**Table of Contents**

**THE OFFERING**

Common stock offered by us	7,350,000 shares
Common stock to be outstanding after this offering	25,849,571 shares
Option to purchase additional shares	We have granted to the underwriters the option, exercisable for 30 days from the date of this prospectus, to purchase up to 1,102,500 additional shares of common stock.
Use of proceeds	We estimate that we will receive net proceeds of approximately \$127.1 million (or approximately \$146.5 million if the underwriters option to purchase additional shares is exercised in full) from the sale of the shares of common stock offered by us in this offering, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering for the following purposes: (1) \$21.0 million to fund our ongoing and planned Phase 1/2 clinical trials of BPX-501, (2) \$30.0 million to fund pre-clinical and Phase 1/2 clinical trial of BPX-401, BPX-601 and BPX-701 as well as preclinical development of our other CART and TCR programs, (3) \$4.0 million to fund our ongoing Phase 1/2 clinical trial and our planned Phase 1/2 clinical trial of BPX-201 in combination with checkpoint inhibitors, (4) \$11.0 million to fund the construction of tenant improvements and the purchase of capital equipment at our Houston facility, and (5) the remainder to fund other working capital purposes, including general operating expenses. See Use of Proceeds.
Risk factors	You should read the Risk Factors section of this prospectus for a discussion of certain of the factors to consider carefully before deciding to purchase any shares of our common stock.
NASDAQ Global Market symbol	BLCM
The number of shares of our common stock to be outstanding after this offering is based on 2,124,386 shares of common stock outstanding as of September 30, 2014, and assumes:	

n the issuance by us of 7,350,000 shares of our common stock in this offering;

- n the conversion of all of our convertible preferred stock outstanding into an aggregate of 12,224,819 shares of common stock upon the closing of this offering;
- n the net exercise of outstanding warrants to purchase common stock for an aggregate of 114,468 shares of common stock;
- n that all of the holders of Series B convertible preferred stock will elect to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock in connection with this offering, which will result in the issuance by us of 177,349 shares of common stock;
- n the issuance by us of 6,559,598 shares of Series C convertible preferred stock issuable upon the exercise of warrants issued by us in August 2014, pursuant to that certain Series C Preferred Stock and Warrant Purchase Agreement, or the Series C Purchase Agreement and the conversion of these shares of Series C convertible preferred stock into an aggregate of 3,858,549 shares of common stock;

**Table of Contents**

and excludes:

- n 1,602,339 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2014, at a weighted-average exercise price of \$2.33 per share;
- n 2,956,909 shares of our common stock reserved for future issuance under our 2014 equity incentive plan, or the 2014 Plan, which will become effective as of the date of the effectiveness of the registration statement of which this prospectus forms a part, which number includes the 258,823 shares subject to stock options and a stock award that will be granted upon the effective date of the 2014 Plan and includes the 1,382,481 shares of common stock reserved for issuance under our 2011 stock option plan, as amended, or the 2011 Plan as of September 30, 2014, reduced by the 1,031,454 shares of common stock issuable upon the exercise of the stock options granted under the 2011 Plan subsequent to September 30, 2014, which aggregate of 351,027 shares will be added to the shares reserved under the 2014 Plan when the 2014 Plan becomes effective;
- n 550,000 shares of common stock reserved for future issuance under our 2014 Employee Stock Purchase Plan, or the ESPP, which will become effective upon the execution and delivery of the underwriting agreement for this offering; and
- n 355,392 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2014, at an exercise price of \$0.0017 per share.

Unless otherwise indicated, all information contained in this prospectus assumes or gives effect to:

- n a 1-for-1.7 reverse stock split of our common stock effected on December 5, 2014;
- n the filing of our amended and restated certificate of incorporation and the adoption of our amended and restated bylaws immediately prior to the closing of this offering; and
- n no exercise by the underwriters of their option to purchase up to an additional 1,102,500 shares of our common stock.

Certain of our existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering.

**Table of Contents****SUMMARY FINANCIAL DATA**

The following summary financial data should be read together with our financial statements and related notes, Selected Financial Data and Management's Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. The summary financial data in this section are not intended to replace our financial statements and the related notes. We derived the summary statement of operations data for the years ended December 31, 2012 and 2013 from our audited financial statements and related notes appearing elsewhere in this prospectus. We derived the summary statement of operations data for the nine months ended September 30, 2013 and 2014 and the summary balance sheet data as of September 30, 2014, from our unaudited financial statements and related notes appearing elsewhere in this prospectus. The unaudited financial data, in management's opinion, have been prepared on the same basis as the audited financial statements and related notes included elsewhere in this prospectus, and include all adjustments, consisting only of normal recurring adjustments, that management considers necessary for a fair presentation of the information for the periods presented. Our historical results are not necessarily indicative of the results that may be expected in the future, and results from our interim period may not necessarily be indicative of the results of the entire year.

(in thousands, except share and per share data)	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2012	2013	2013 (unaudited)	2014 (unaudited)
<b>Statement of Operations Data:</b>				
Grant revenue	\$ 1,470	\$ 1,941	\$ 1,122	\$ 1,766
Operating expenses:				
Research and development	5,796	7,050	4,564	7,078
General and administrative	1,943	2,813	1,997	3,135
Total operating expenses	7,739	9,863	6,561	10,213
Loss from operations	(6,269)	(7,922)	(5,439)	(8,447)
Other income (expense):				
Interest income	7	4	2	15
Interest expense	(1)	(51)	(38)	(38)
Change in value of warrant liability				(1,197)
Total other income (expense)	6	(47)	(36)	(1,220)
Net loss	\$ (6,263)	\$ (7,969)	\$ (5,475)	\$ (9,667)
Preferred stock dividends	(757)	(1,094)	(695)	(1,432)

Net loss available to common stockholders	\$ (7,020)	\$ (9,063)	\$ (6,170)	\$ (11,099)
Net loss per share, basic and diluted <sup>(1)</sup>	\$ (4.26)	\$ (5.25)	\$ (3.58)	\$ (5.45)
Weighted-average shares outstanding, basic and diluted	1,648,198	1,725,992	1,725,992	2,036,025
Pro forma net loss (unaudited)		\$ (7,969)		\$ (9,667)
Pro forma net loss per share, basic and diluted (unaudited) <sup>(2)</sup>		\$ (1.32)		\$ (0.98)
Pro forma weighted-average shares outstanding, basic and diluted (unaudited) <sup>(2)</sup>		6,051,619		9,827,767

<sup>(1)</sup> See Note 2 to our financial statements appearing elsewhere in this prospectus for an explanation of the method used to calculate the basic and diluted net loss per common share and the number of shares used in the computation of the per share amounts.

**Table of Contents**

- (2) The calculations for the unaudited pro forma net loss per common share, basic and diluted, assume (1) the conversion of all our outstanding shares of convertible preferred stock as of September 30, 2014, into an aggregate of 12,224,819 shares of our common stock, (2) the net exercise of outstanding warrants to purchase common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, (3) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants, and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering. The calculations exclude the impact of the issuance by us of an aggregate of 177,349 shares of our common stock as payment of the accrued dividend payable to the holders of Series B convertible preferred stock in connection with this offering.

(in thousands)	AS OF SEPTEMBER 30, 2014		
	ACTUAL (unaudited)	PRO FORMA <sup>(1)(2)</sup>	PRO FORMA AS ADJUSTED <sup>(3)</sup>
<b>Balance Sheet Data:</b>			
Cash and cash equivalents	\$ 61,932	\$ 86,287	\$ 213,469
Working capital	49,849	65,269	192,369
Total assets	66,331	90,686	217,786
Convertible preferred stock	90,753		
Accumulated deficit	(38,646)	(83,559)	(83,559)
Total stockholders (deficit) equity	(38,794)	51,913	179,013

- (1) Pro forma amounts reflect (1) the conversion of all our outstanding shares of our convertible preferred stock as of September 30, 2014 into an aggregate of 12,224,819 shares of our common stock in connection with the closing of this offering, and (2) the net exercise of outstanding warrants to purchase shares of our common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, (3) the issuance by us of 177,349 shares of common stock, as payment of the accrued dividend on the outstanding shares of Series B convertible preferred stock payable to the holders of Series B convertible preferred stock in connection with this offering, (4) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants, and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering.
- (2) Pro forma amounts reflect the October 2014 amendment to our license agreement with Ariad, pursuant to which we agreed to pay \$50.0 million in exchange for expanded use of the license and the termination of all obligations to make milestone and royalty payments to Ariad in the future. We have reflected (1) a decrease in cash for the initial payment of \$15.0 million in October 2014, (2) a liability of \$35.0 million to recognize the promissory note for the remaining balance, (3) a \$5.1 million reduction in equity to reflect the estimated fair value, as of October 2014, of the common stock to be returned to us in connection with the second payment to Ariad and (4) a \$44.9 million charge to research and development expense. The final accounting treatment is still under review and may

ultimately result in different accounting treatment, including discounting the liability or less research and development expense.

- (3) Pro forma as adjusted amounts reflect the pro forma conversion adjustments described in footnotes (1) and (2) above, as well as the sale of 7,350,000 shares of our common stock in this offering at the initial public offering price of \$19.00 per share after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.



---

**Table of Contents**

**RISK FACTORS**

*Investing in our common stock involves a high degree of risk. You should carefully consider the risks described below, as well as the other information in this prospectus, including our financial statements and the related notes and Management's Discussion and Analysis of Financial Condition and Results of Operations, before deciding whether to invest in our common stock. The occurrence of any of the events or developments described below could harm our business, financial condition, results of operations and growth prospects. In such an event, the market price of our common stock could decline and you may lose all or part of your investment. Additional risks and uncertainties not presently known to us or that we currently deem immaterial also may impair our business operations.*

**Risks Related to Our Business and Industry**

*We have incurred net losses in every year since our inception and anticipate that we will continue to incur net losses in the future.*

We are a clinical stage biopharmaceutical company with a limited operating history. We are not profitable and have incurred losses in each period since our inception in 2004. To date, we have financed our operations primarily through private placements of convertible debt and preferred stock. For the years ended December 31, 2012 and 2013, we reported a net loss of \$6.3 million and \$8.0 million, respectively. For the nine months ended September 30, 2013 and 2014, we reported a net loss of \$5.5 million and \$9.7 million, respectively. As of September 30, 2014, we had an accumulated deficit of \$38.6 million. We expect to continue to incur significant losses for the foreseeable future, and we expect these losses to increase as we continue our research and development of, and seek regulatory approvals for, our product candidates.

Even if we succeed in commercializing one or more of our product candidates, we will continue to incur substantial research and development and other expenditures to develop and market additional product candidates. We may encounter unforeseen expenses, difficulties, complications, delays and other unknown factors that may adversely affect our business. The size of our future net losses will depend, in part, on the rate of future growth of our expenses and our ability to generate revenue. Our prior losses and expected future losses have had and will continue to have an adverse effect on our stockholders' equity and working capital.

*Biopharmaceutical product development is a highly speculative undertaking and involves a substantial degree of uncertainty. We have never generated any revenue from product sales and may never be profitable.*

We have devoted substantially all of our financial resources and efforts to developing our proprietary CID technology platform, identifying potential product candidates and conducting preclinical studies and clinical trials. We are still in the early stages of developing our product candidates, and we have not completed development of any products. Our ability to generate revenue and achieve profitability depends in large part on our ability, alone or with partners, to successfully complete the development of, obtain the necessary regulatory approvals for, and commercialize, product candidates. We do not anticipate generating revenues from sales of products for the foreseeable future. Our ability to generate future revenues from product sales depends heavily on our success in:

- n completing clinical trials through all phases of clinical development of our current product candidates, as well as the product candidates that are being developed by our partners and licensees;

- n seeking and obtaining marketing approvals for product candidates that successfully complete clinical trials;
- n launching and commercializing product candidates for which we obtain marketing approval, with a partner or, if launched independently, successfully establishing a sales force, marketing and distribution infrastructure;
- n identifying and developing new product candidates;
- n progressing our pre-clinical programs into human clinical trials;
- n establishing and maintaining supply and manufacturing relationships with third parties;
- n developing new molecular switches based on our proprietary CID technology platform;
- n maintaining, protecting, expanding and enforcing our intellectual property; and
- n attracting, hiring and retaining qualified personnel.

## **Table of Contents**

Because of the numerous risks and uncertainties associated with biologic product development, we are unable to predict the timing or amount of increased expenses and when we will be able to achieve or maintain profitability, if ever. In addition, our expenses could increase beyond expectations if we are required by the FDA, or foreign regulatory agencies, to perform studies and clinical trials in addition to those that we currently anticipate, or if there are any delays in our or our partners completing clinical trials or the development of any of our product candidates. If one or more of the product candidates that we independently develop is approved for commercial sale, we anticipate incurring significant costs associated with commercializing such product candidates. Even if we or our partners are able to generate revenues from the sale of any approved products, we may not become profitable and may need to obtain additional funding to continue operations, which may not be available to us on favorable terms, if at all. Even if we do achieve profitability, we may not be able to sustain or increase profitability on a quarterly or annual basis. Our failure to become and remain profitable would depress the value of our company and could impair our ability to raise capital, expand our business, maintain our research and development efforts, diversify our product offerings or even continue our operations. A decline in the value of our company could also cause you to lose all or part of your investment.

***We have concentrated our therapeutic product research and development efforts on our CID platform, and our future success depends on the successful development of this therapeutic approach.***

Our proprietary CID technology platform is novel and there are no approved products or product candidates in late-stage clinical trials based on this technology. Additionally, the safety and efficacy profile of rimiducid has not been subject to large scale clinical testing. If rimiducid is found to have a poor safety profile in clinical trials, or if our technology is not effective, we may be required to redesign all of our product candidates, which would require significant time and expense. In addition, our CID platform technology may not be applicable or effective in the development of additional cellular immunotherapies beyond our current programs which would adversely affect our business and prospects.

***CAR T cell therapies are novel and present significant challenges.***

CAR-T and TCR product candidates represent a relatively new field of cellular immunotherapy and there are no FDA-approved products in this area. Advancing this novel and personalized therapy creates significant challenges for us, including:

- n obtaining regulatory approval, as the FDA and other regulatory authorities have limited experience with commercial development of T-cell therapies for cancer;
- n sourcing clinical and, if approved, commercial supplies for the materials used to manufacture and process our product candidates;
- n developing a consistent and reliable process, while limiting contamination risks, for engineering and manufacturing T cells *ex vivo* and infusing the engineered T cells into the patient;
- n educating medical personnel regarding the potential safety benefits, as well as the challenges, of incorporating our product candidates into their treatment regimens; and

n establishing sales and marketing capabilities upon obtaining any regulatory approval to gain market acceptance of a novel therapy.

Our inability to successfully develop CAR-T and TCR cell therapies or develop processes related to the manufacture, sales and marketing of these therapies would adversely affect our business, results of operations and prospects. We believe that we have appropriately accounted for the above factors while pursuing the development and commercialization of our product candidates, but we cannot entirely eliminate the risks associated with novel technology.

***Failure to successfully develop and obtain approval of our lead product candidate BPX-501 or our other clinical product candidates could adversely affect our future success.***

Our business and future success depends, in part, on our ability to obtain regulatory approval of and then successfully commercialize our lead product candidate, BPX-501 and our other clinical product candidates. BPX-501 is in the early stages of development. All of our product candidates, including BPX-501, will require additional clinical and non-clinical development, regulatory review and approval in multiple jurisdictions, substantial investment, access to sufficient commercial manufacturing capacity and significant marketing efforts before we can generate any revenue from product sales. In addition, because BPX-501 is our most advanced product candidate,

## **Table of Contents**

and because many of our other product candidates are based on similar technology, if BPX-501 encounters safety or efficacy problems, developmental delays or regulatory issues or other problems, our development plans and business for our other product candidates would be significantly harmed. In addition, our product candidates that incorporate the CID safety switch combine genetically modified T cells that are used to enhance the patients immune system and a small molecule that leads to the death of these modified T cells if they cause safety issues.

***Our clinical trials may fail to adequately demonstrate the safety and efficacy of any of our product candidates, which would prevent or delay regulatory approval and commercialization.***

Before obtaining regulatory approvals for the commercial sale of our product candidates, we must demonstrate through lengthy, complex and expensive preclinical testing and clinical trials that our product candidates are both safe and effective for use in each target indication. Clinical testing is expensive and can take many years to complete, and its outcome is inherently uncertain. Failure can occur at any time during the clinical trial process. The results of preclinical studies and early clinical trials of our product candidates may not be predictive of the results of later-stage clinical trials. We expect there may be greater variability in results for cellular immunotherapy products processed and administered on a patient-by-patient basis, like all of our CID technology-based development and product candidates, than for off-the-shelf products, like many drugs. There is typically an extremely high rate of attrition from the failure of product candidates proceeding through clinical trials. Product candidates in later stages of clinical trials may fail to show the desired safety and efficacy profile despite having progressed through preclinical studies and initial clinical trials. A number of companies in the biopharmaceutical industry have suffered significant setbacks in advanced clinical trials due to lack of efficacy or unacceptable safety issues, notwithstanding promising results in earlier clinical trials. Most product candidates that commence clinical trials are never approved as products.

***We have not completed any clinical studies of our current product candidates. Success in early clinical studies may not be indicative of results obtained in later studies.***

Many of our current product candidates have not initiated evaluation in human clinical studies, and we may experience unexpected results in the future. Differences in cell processing, time of administration and patient conditioning, among other factors, may result in our experiencing different results in our clinical trials from those reported in trials by our collaborators, and may mean that we experience different results in our clinical trials. In addition, data from preclinical studies and investigator-led Phase 1 or Phase 1/2 clinical trials of BPX-501 therapy should not be relied upon as evidence that later or later-scale clinical trials will succeed. We have designed our planned Phase 1/2 single-arm multicenter clinical trial of BPX-501 primarily to assess safety and efficacy in a small number of adult patients with malignant disease. In addition, we are initiating additional Phase 1 and Phase 1/2 clinical trials of BPX-501 and there are a number of investigator-led clinical trials of BPX-501 ongoing and planned.

Similarly, results from preclinical studies, such as *in vitro* and *in vivo* studies, of BPX-401, BPX-601, BPX-701 and our other preclinical programs may not be indicative of the results of clinical trials of these product candidates. Furthermore, we may not be able to commence human clinical trials on any of our preclinical product candidates on the time frames we expect. Our failure to meet these expected targets would likely have an adverse effect on our stock price.

Even if the clinical trials are successfully completed, we cannot guarantee that the FDA or foreign regulatory authorities will interpret the results as we do, and more clinical trials could be required before we submit our product candidates for approval. To the extent that the results of the clinical trials are not satisfactory to the FDA or foreign regulatory authorities for support of a marketing application, approval of our product candidates may be significantly delayed, or we may be required to expend significant additional resources, which may not be available to us, to conduct additional clinical trials in support of potential approval of our product candidates.

***We may not be successful in our efforts to use and expand our CID platform to build a pipeline of product candidates and develop marketable products.***

We believe that our CID platform, which serves as the foundation of our CaspaCIDE, CIDE CAR, GoCAR-T and DeCIDE technologies, can be further leveraged to discover other novel technologies, therapeutic applications and market opportunities. For example, we are currently conducting research in applying our platform TCR therapies for solid tumors, where immune toxicities associated with treatment are even more severe than CAR-T therapies. We are also developing new molecular switches and two-switch systems to provide greater control over cellular immunotherapy.

## Table of Contents

We are at a very early stage of development and our platform has not yet, and may never lead to, approved or marketable products. Even if we are successful in continuing to build our pipeline, the potential product candidates that we identify may not be suitable for clinical development, including for reasons related to their harmful side effects, limited efficacy or other characteristics that indicate that they are unlikely to be products that will receive marketing approval and achieve market acceptance. If we do not successfully develop and commercialize product candidates based upon our technological approach, we may not be able to obtain product or partnership revenues in future periods, which would adversely affect our business, prospects, financial condition and results of operations.

***We rely and will continue to rely on third parties to conduct our clinical trials. If these third parties do not successfully carry out their contractual duties or meet expected deadlines, we may not be able to obtain regulatory approval of or commercialize our product candidates.***

We depend and will continue to depend upon independent investigators and collaborators, such as universities, medical institutions, and strategic partners to conduct our preclinical and clinical trials under agreements with us. Negotiations of budgets and contracts with study sites may result in delays to our development timelines and increased costs. We will rely heavily on these third parties over the course of our clinical trials, and we control only certain aspects of their activities. Nevertheless, we are responsible for ensuring that each of our studies is conducted in accordance with applicable protocol, legal, regulatory and scientific standards, and our reliance on third parties does not relieve us of our regulatory responsibilities. We and these third parties are required to comply with current good clinical practices, or cGCPs, which are regulations and guidelines enforced by the FDA and comparable foreign regulatory authorities for product candidates in clinical development. Regulatory authorities enforce these cGCPs through periodic inspections of clinical trial sponsors, principal investigators and clinical trial sites. If we or any of these third parties fail to comply with applicable cGCP regulations, the clinical data generated in our clinical trials may be deemed unreliable and the FDA or comparable foreign regulatory authorities may require us to perform additional clinical trials before approving our marketing applications. We cannot assure you that, upon inspection, such regulatory authorities will determine that any of our clinical trials comply with the cGCP regulations. In addition, our clinical trials must be conducted with biologic product produced under current good manufacturing practices, or cGMPs, regulations and will require a large number of test patients. Our failure or any failure by these third parties to comply with these regulations or to recruit a sufficient number of patients may require us to repeat clinical trials, which would delay the regulatory approval process. Moreover, our business may be implicated if any of these third parties violates federal or state fraud and abuse or false claims laws and regulations or healthcare privacy and security laws.

Any third parties conducting our clinical trials are and will not be our employees and, except for remedies available to us under our agreements with these third parties, we cannot control whether or not they devote sufficient time and resources to our ongoing preclinical, clinical and nonclinical programs. These third parties may also have relationships with other commercial entities, including our competitors, for whom they may also be conducting clinical trials or other drug development activities, which could affect their performance on our behalf. If these third parties do not successfully carry out their contractual duties or obligations or meet expected deadlines, if they need to be replaced or if the quality or accuracy of the clinical data they obtain is compromised due to the failure to adhere to our clinical protocols or regulatory requirements or for other reasons, our clinical trials may be extended, delayed or terminated and we may not be able to complete development of, obtain regulatory approval of or successfully commercialize our product candidates. As a result, our financial results and the commercial prospects for our product candidates would be harmed, our costs could increase and our ability to generate revenue could be delayed.

Switching or adding third parties to conduct our clinical trials involves substantial cost and requires extensive management time and focus. In addition, there is a natural transition period when a new third party commences work. As a result, delays occur, which can materially impact our ability to meet our desired clinical development timelines.

Additionally, we are conducting multiple clinical trials in Europe and may plan additional testing of our technology and product candidates in other foreign jurisdictions. We currently have limited staffing and capabilities in foreign countries, and may not be able to effectively resolve potential disputes with our independent investigators and collaborators.



**Table of Contents**

*If we encounter difficulties enrolling patients in our clinical trials, our clinical development activities could be delayed or otherwise adversely affected.*

We may experience difficulties in patient enrollment in our clinical trials for a variety of reasons. The timely completion of clinical trials in accordance with their protocols depends, among other things, on our ability to enroll a sufficient number of patients who remain in the study until its conclusion. The enrollment of patients depends on many factors, including:

- n the patient eligibility criteria defined in the protocol;
- n the size of the patient population required for analysis of the trial's primary endpoints;
- n the proximity of patients to study sites;
- n the design of the clinical trial;
- n our ability to recruit clinical trial investigators with the appropriate competencies and experience;
- n our ability to obtain and maintain patient consents; and

n the risk that patients enrolled in clinical trials will drop out of the clinical trials before completion. In particular, some of our clinical trials will look to enroll patients with characteristics which are found in a very small population, such as patients with CD19-expressing cancers, such as ALL, CLL and non-Hodgkin's lymphomas, and patients with orphan inherited blood disorders. Our clinical trials will compete with other clinical trials for product candidates that are in the same therapeutic areas as our product candidates, and this competition will reduce the number and types of patients available to us, because some patients who might have opted to enroll in our clinical trials may instead opt to enroll in a trial being conducted by one of our competitors. Since the number of qualified clinical investigators is limited, we expect to conduct some of our clinical trials at the same clinical trial sites that some of our competitors use, which will reduce the number of patients who are available for our clinical trials in these clinical trial sites. Moreover, because our product candidates represent a departure from more commonly used methods for cancer treatment, potential patients and their doctors may be inclined to use conventional therapies, such as chemotherapy and antibody therapy, rather than enroll patients in any of our future clinical trials. Patients may also be unwilling to participate in our clinical trials because of negative publicity from adverse events in the biotechnology or gene therapy industries.

Delays in patient enrollment may result in increased costs or may affect the timing or outcome of the planned clinical trials, which could prevent completion of these clinical trials and adversely affect our ability to advance the development of our product candidates.

***Any adverse developments that occur during any clinical trials conducted by academic investigators, our collaborators or other entities conducting clinical trials under independent INDs may affect our ability to obtain regulatory approval or commercialize our product candidates.***

BPX-501 and certain of our other CaspaCIDE product candidates are being used by third parties in clinical trials for which we are collaborating or in clinical trials which are completely independent of our development program. We have little to no control over the conduct of such clinical trials. If serious adverse events occur during these or any other clinical trials using our product candidates, the FDA and other regulatory authorities may delay, limit or deny approval of our product candidate or require us to conduct additional clinical trials as a condition to marketing approval, which would increase our costs. If we receive FDA approval for BPX-501 or any other CaspaCIDE product candidate and a new and serious safety issue is identified in connection with clinical trials conducted by third parties, the FDA and other regulatory authorities may withdraw their approval of the product or otherwise restrict our ability to market and sell our product. In addition, treating physicians may be less willing to administer our product due to concerns over such adverse events, which would limit our ability to commercialize our product.

***Adverse side effects or other safety risks associated with our product candidates could delay or preclude approval, cause us to suspend or discontinue clinical trials, abandon product candidates, limit the commercial profile of an approved label, or result in significant negative consequences following marketing approval, if any.***

Undesirable side effects caused by our product candidates could result in the delay, suspension or termination of clinical trials by us, the FDA or other regulatory authorities for a number of reasons. If we elect or are required to delay, suspend or terminate any clinical trial of any product candidates that we develop, the commercial prospects of such product candidates will be harmed and our ability to generate product revenues from any of these product

## **Table of Contents**

candidates will be delayed or eliminated. Serious adverse events observed in clinical trials could hinder or prevent market acceptance of the product candidate at issue. Any of these occurrences may harm our business, prospects, financial condition and results of operations significantly.

In other clinical trials involving CAR T cells, the most prominent acute toxicities included symptoms thought to be associated with the release of cytokines, such as fever, low blood pressure and kidney dysfunction. Some patients also experienced toxicity of the central nervous system, such as confusion, cranial nerve dysfunction and speech impairment. Adverse events by worst grade and attributed to CAR T cells were severe and life threatening in some patients. The life threatening events were related to kidney dysfunction and toxicities of the central nervous system. Severe and life threatening toxicities occurred mostly in the first two weeks after cell infusion and generally resolved within three weeks. In the past, several patients have also died in clinical trials by others involving CAR T cells.

### ***Clinical trials are expensive, time-consuming and difficult to design and implement.***

Human clinical trials are expensive and difficult to design and implement, in part because they are subject to rigorous regulatory requirements. Because our product candidates are based on new technology and engineered on a patient-by-patient basis, we expect that they will require extensive research and development and have substantial manufacturing and processing costs. In addition, costs to treat patients with relapsed/refractory cancer and to treat potential side effects that may result from therapies such as our current and future product candidates can be significant. Accordingly, our clinical trial costs are likely to be significantly higher than for more conventional therapeutic technologies or drug products. In addition, our proposed personalized product candidates involve several complex and costly manufacturing and processing steps, the costs of which will be borne by us. The costs of our clinical trials may increase if the FDA does not agree with our clinical development plans or requires us to conduct additional clinical trials to demonstrate the safety and efficacy of our product candidates.

### ***We face significant competition from other biotechnology and pharmaceutical companies, and our operating results will suffer if we fail to compete effectively.***

The biopharmaceutical industry is characterized by intense competition and rapid innovation. Our competitors may be able to develop other compounds or drugs that are able to achieve similar or better results. Our potential competitors include major multinational pharmaceutical companies, established biotechnology companies, specialty pharmaceutical companies and universities and other research institutions. Many of our competitors have substantially greater financial, technical and other resources, such as larger research and development staff and experienced marketing and manufacturing organizations and well-established sales forces. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large, established companies. Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated in our competitors. Competition may increase further as a result of advances in the commercial applicability of technologies and greater availability of capital for investment in these industries. Our competitors, either alone or with collaborative partners, may succeed in developing, acquiring or licensing on an exclusive basis drug or biologic products that are more effective, safer, more easily commercialized or less costly than our product candidates or may develop proprietary technologies or secure patent protection that we may need for the development of our technologies and products. We believe the key competitive factors that will affect the development and commercial success of our product candidates are efficacy, safety, tolerability, reliability, convenience of use, price and reimbursement.

Specifically, genetically engineering T cells faces significant competition in both the CAR and TCR technology space from multiple companies, including Adaptimmune Limited, bluebird bio, Inc., Celgene Corporation, Collectis SA, GlaxoSmithKline plc, Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc., Novartis AG and Pfizer Inc.

Even if we obtain regulatory approval of our product candidates, the availability and price of our competitors' products could limit the demand and the price we are able to charge for our product candidates. We may not be able to implement our business plan if the acceptance of our product candidates is inhibited by price competition or the reluctance of physicians to switch from existing methods of treatment to our product candidates, or if physicians switch to other new drug or biologic products or choose to reserve our product candidates for use in limited circumstances. For additional information regarding our competition, see [Business Competition](#).

## **Table of Contents**

***We are highly dependent on our key personnel, and if we are not successful in attracting and retaining highly qualified personnel, we may not be able to successfully implement our business strategy.***

Our ability to compete in the highly competitive biotechnology and pharmaceutical industries depends upon our ability to attract and retain highly qualified managerial, scientific and medical personnel. We are highly dependent on our management, scientific and medical personnel, including our Chief Executive Officer, our Chief Operating Officer, our Chief Medical Officer and Chief Technology Officer, and our Chief Scientific Officer. The loss of the services of any of our executive officers, other key employees, and other scientific and medical advisors, and our inability to find suitable replacements could result in delays in product development and harm our business.

To induce valuable employees to remain at our company, in addition to salary and cash incentives, we have provided stock options that vest over time. The value to employees of stock options that vest over time may be significantly affected by movements in our stock price that are beyond our control, and may at any time be insufficient to counteract more lucrative offers from other companies. Despite our efforts to retain valuable employees, members of our management, scientific and development teams may terminate their employment with us on short notice. Although we have employment agreements with our key employees, these employment agreements provide for at-will employment, which means that any of our employees could leave our employment at any time, with or without notice. We do not maintain key man insurance policies on the lives of these individuals or the lives of any of our other employees. Our success also depends on our ability to continue to attract, retain and motivate highly skilled scientific and medical personnel.

***We will need to grow the size of our organization, and we may experience difficulties in managing this growth.***

As of September 30, 2014, we had 30 employees, all of whom were full-time. As our development and commercialization plans and strategies develop, and as we transition into operating as a public company, we expect to need additional managerial, operational, sales, marketing, financial and other personnel. Future growth would impose significant added responsibilities on members of management, including:

- n identifying, recruiting, integrating, maintaining and motivating additional employees, including a Chief Financial Officer;
  
- n managing our internal development efforts effectively, including the clinical and FDA review process for our product candidates, while complying with our contractual obligations to contractors and other third parties; and
  
- n improving our operational, financial and management controls, reporting systems and procedures.

There are a small number of individuals with experience in cell therapy and the competition for such individuals is high. Our future financial performance and our ability to commercialize our product candidates will depend, in part, on our ability to effectively manage any future growth, and our management may also have to divert a disproportionate amount of its attention away from day-to-day activities in order to devote a substantial amount of time to managing these growth activities. We currently rely, and for the foreseeable future will continue to rely, in substantial part on certain independent organizations, advisors and consultants to provide certain services, including substantially all aspects of regulatory approval, clinical management, and manufacturing. There can be no assurance that the services of independent organizations, advisors and consultants will continue to be available to us on a timely

basis when needed, or that we can find qualified replacements. In addition, if we are unable to effectively manage our outsourced activities or if the quality or accuracy of the services provided by consultants is compromised for any reason, our clinical trials may be extended, delayed or terminated, and we may not be able to obtain regulatory approval of our product candidates or otherwise advance our business. There can be no assurance that we will be able to manage our existing consultants or find other competent outside contractors and consultants on economically reasonable terms, or at all.

If we are not able to effectively expand our organization by hiring new employees and expanding our groups of consultants and contractors, we may not be able to successfully implement the tasks necessary to further develop and commercialize our product candidates and, accordingly, may not achieve our research, development and commercialization goals.

In addition to expanding our organization, we expect to increase the size of our facility and build out our development and manufacturing capabilities, which will require significant capital expenditures. If these capital

## **Table of Contents**

expenditures are higher than expected, it may adversely affect our financial condition and capital resources. In addition, if the increase in the size of our facility is delayed, it may limit our ability to rapidly expand the size of our organization in order to meet our corporate goals.

***If we fail to obtain additional financing, we may be unable to complete the development and commercialization of our product candidates.***

Our operations have consumed substantial amounts of cash since inception. We expect to continue to spend substantial amounts to continue the clinical development of our product candidates, including our planned clinical development and preclinical studies of our product candidates and other programs. If approved, we will require significant additional amounts in order to launch and commercialize our product candidates.

As of September 30, 2014, we had cash and cash equivalents of approximately \$61.9 million. We estimate that our net proceeds from this offering will be approximately \$127.1 million, after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use the net proceeds from this offering to fund (1) our ongoing and planned Phase 1/2 clinical trials of BPX-501; (2) pre-clinical studies for BPX-401, BPX-601 and BPX-701 and fund the Phase 1/2 clinical trial of BPX-401, partially fund the planned Phase 1/2 clinical trial of BPX-601 and fund the Phase 1/2 clinical trial of BPX-701 as well as preclinical development of our other CAR T and TCR programs; (3) our planned Phase 1/2 clinical trials of BPX-201 in combination with checkpoint inhibitors; (4) the construction of tenant improvements and the purchase of capital equipment at our Houston facility, to accommodate our anticipated personnel needs for the next three years and to support our planned in-house new product discovery and development, as well as process development and manufacturing for U.S. clinical trials of planned product candidates or certain critical components thereof; and (5) other working capital purposes, including general operating expenses. We believe that such proceeds together with our existing cash and cash equivalents will be sufficient to fund our operations through at least 2016. However, changing circumstances may cause us to consume capital significantly faster than we currently anticipate, and we may need to spend more money than currently expected because of circumstances beyond our control. We may require additional capital for the further development and commercialization of our product candidates and may need to raise additional funds sooner if we choose to expand more rapidly than we presently anticipate.

Additional funding may not be available on acceptable terms, or at all. We have no committed source of additional capital and if we are unable to raise additional capital in sufficient amounts or on terms acceptable to us, we may have to significantly delay, scale back or discontinue the development or commercialization of our product candidates or other research and development initiatives. We could be required to seek collaborators for our product candidates at an earlier stage than otherwise would be desirable or on terms that are less favorable than might otherwise be available or relinquish or license on unfavorable terms our rights to our product candidates in markets where we otherwise would seek to pursue development or commercialization ourselves.

Any of the above events could significantly harm our business, prospects, financial condition and results of operations and cause the price of our common stock to decline.

***Raising additional capital may cause dilution to our existing stockholders, restrict our operations or require us to relinquish rights to our technologies or product candidates.***

We may seek additional capital through a combination of public and private equity offerings, debt financings, strategic partnerships and alliances and licensing arrangements. To the extent that we raise additional capital through the sale of equity or convertible debt securities, your ownership interest will be diluted, and the terms may include liquidation or other preferences that adversely affect your rights as a stockholder. The incurrence of indebtedness

would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us.



## **Table of Contents**

### ***We need to oversee manufacturing of a complex supply chain of cellular therapy product candidates, viral vectors and small molecule drugs.***

Because of the complex nature of our products, we need to oversee manufacture of multiple components that require a diverse knowledge base and appropriate manufacturing personnel. The supply chain for these components is separate and distinct, and no single manufacturer can supply more than one component of each of our products. Additionally, it is likely that the cell therapy products will need to be made within an appropriate geographic location for the area in which the products will be utilized, so one cell therapy manufacturing facility may not be able to supply diverse geographic areas. Any lack of capabilities to store, freeze, thaw and infuse our cell therapies would adversely affect our business and prospects.

### ***We expect to rely on third parties to manufacture a substantial portion of our clinical cell therapy product candidates, viral vectors and small molecule supplies in the United States and Europe.***

We do not currently own any facility that may be used as our clinical-scale manufacturing and processing facility, and must currently rely on outside vendors to manufacture supplies and process our product candidates, which is and will need to be done on a patient-by-patient basis. We have not yet caused our product candidates to be manufactured or processed on a commercial scale. We may not be able to scale patient-by-patient manufacturing and processing to satisfy clinical or commercial demands for any of our product candidates. In addition, our anticipated reliance on a limited number of third-party manufacturers exposes us to the following risks:

- n We may be unable to identify manufacturers on acceptable terms or at all because the number of potential manufacturers is limited and the FDA or an equivalent foreign regulatory agency must approve any replacement contractor. This approval would require new testing and compliance inspections. In addition, a new manufacturer would have to be educated in, or develop substantially equivalent processes for, production of our products after receipt of FDA approval, if any.
- n Our third-party manufacturers might be unable to timely formulate and manufacture our product or produce the quantity and quality required to meet our clinical and commercial needs, if any.
- n Contract manufacturers may not be able to execute our manufacturing procedures appropriately.
- n Our future contract manufacturers may not perform as agreed or may not remain in the contract manufacturing business for the time required to supply our clinical trials or to successfully produce, store and distribute our products.
- n Manufacturers are subject to ongoing periodic unannounced inspection by the FDA, the Drug Enforcement Administration, or corresponding agencies in other geographic locations, to ensure strict compliance with cGMP and other government regulations and corresponding foreign standards. We do not have control over third-party manufacturers' compliance with these regulations and standards.

n We may not own, or may have to share, the intellectual property rights to any improvements made by our third-party manufacturers in the manufacturing process for our products.

n Our third-party manufacturers could breach or terminate their agreement with us. Each of these risks could delay our clinical trials, the approval, if any of our product candidates by the FDA or the commercialization of our product candidates or result in higher costs or deprive us of potential product revenue. In addition, we will rely on third parties to perform release tests on our product candidates prior to delivery to patients. If these tests are not appropriately done and test data are not reliable, patients could be put at risk of serious harm.

***We expect to create our own manufacturing facility for supply of U.S. clinical and/or commercial cell therapy product candidate requirements, but there is no guarantee we will be able to do so.***

Our intent to create internal manufacturing infrastructure will rely upon finding personnel with an appropriate background and training to staff and operate the facility on a daily basis. Should we be unable to find such individuals, we may need to rely on external contractors longer than anticipated, and train additional personnel to fill the needed roles. There are a small number of individuals with experience in cell therapy and the competition for such individuals is high.

Specifically, the establishment of a cell-therapy manufacturing facility is a complex endeavor requiring knowledgeable individuals who have successful previous experience in cleanroom designs. Cell therapy facilities, like other biological agent manufacturing facilities, require appropriate commissioning and validation activities to

## **Table of Contents**

demonstrate that they operate as designed. Additionally, each manufacturing process must be validated through the performance of process validation runs to guarantee that the facility, personnel, equipment, and process work as designed. While we have developed our own manufacturing processes using an in-house process development team to maximize our understanding of our process, there is timing risk associated with in-house product manufacture.

***Cell-based therapies rely on the availability of specialty raw materials, which may not be available to us on acceptable terms or at all.***

Gene-modified cell therapy manufacture requires many specialty raw materials, some of which are manufactured by small companies with limited resources and experience to support a commercial product. Some suppliers typically support biomedical researchers or blood-based hospital businesses and may not have the capacity to support commercial products manufactured under cGMP by biopharmaceutical firms. The suppliers may be ill-equipped to support our needs, especially in non-routine circumstances like an FDA inspection or medical crisis, such as widespread contamination. We also do not have commercial supply arrangements with many of these suppliers, and may not be able to contract with them on acceptable terms or at all. Accordingly, we may experience delays in receiving key raw materials to support clinical or commercial manufacturing.

In addition, some raw materials are currently available from a single supplier, or a small number of suppliers. We cannot be sure that these suppliers will remain in business, or that they will not be purchased by one of our competitors or another company that is not interested in continuing to produce these materials for our intended purpose.

***We currently have no marketing and sales organization and have no experience in marketing products. If we are unable to establish marketing and sales capabilities or enter into agreements with third parties to market and sell our product candidates, we may not be able to generate product revenue.***

We currently have no sales, marketing or distribution capabilities and have no experience in marketing products. We intend to develop an in-house marketing organization and sales force, which will require significant capital expenditures, management resources and time. We will have to compete with other pharmaceutical and biotechnology companies to recruit, hire, train and retain marketing and sales personnel.

If we are unable or decide not to establish internal sales, marketing and distribution capabilities, we will pursue collaborative arrangements regarding the sales and marketing of our products, however, there can be no assurance that we will be able to establish or maintain such collaborative arrangements, or if we are able to do so, that they will have effective sales forces. Any revenue we receive will depend upon the efforts of such third parties, which may not be successful. We may have little or no control over the marketing and sales efforts of such third parties and our revenue from product sales may be lower than if we had commercialized our product candidates ourselves. We also face competition in our search for third parties to assist us with the sales and marketing efforts of our product candidates.

There can be no assurance that we will be able to develop in-house sales and distribution capabilities or establish or maintain relationships with third-party collaborators to commercialize any product in the United States or overseas.

***A variety of risks associated with marketing our product candidates internationally could materially adversely affect our business.***

We plan to seek regulatory approval of our product candidates outside of the United States and, accordingly, we expect that we will be subject to additional risks related to operating in foreign countries if we obtain the necessary approvals, including:

- n differing regulatory requirements in foreign countries;
- n unexpected changes in tariffs, trade barriers, price and exchange controls and other regulatory requirements;
- n economic weakness, including inflation, or political instability in particular foreign economies and markets;
- n compliance with tax, employment, immigration and labor laws for employees living or traveling abroad;
- n foreign taxes, including withholding of payroll taxes;
- n foreign currency fluctuations, which could result in increased operating expenses and reduced revenue, and other obligations incident to doing business in another country;

**Table of Contents**

- n difficulties staffing and managing foreign operations;
- n workforce uncertainty in countries where labor unrest is more common than in the United States;
- n potential liability under the Foreign Corrupt Practices Act of 1977 or comparable foreign regulations;
- n challenges enforcing our contractual and intellectual property rights, especially in those foreign countries that do not respect and protect intellectual property rights to the same extent as the United States;
- n production shortages resulting from any events affecting raw material supply or manufacturing capabilities abroad; and
- n business interruptions resulting from geo-political actions, including war and terrorism.

These and other risks associated with our international operations may materially adversely affect our ability to attain or maintain profitable operations.

***We may form or seek strategic alliances or enter into additional licensing arrangements in the future, and we may not realize the benefits of such alliances or licensing arrangements.***

We may form or seek strategic alliances, create joint ventures or collaborations or enter into additional licensing arrangements with third parties that we believe will complement or augment our development and commercialization efforts with respect to our product candidates and any future product candidates that we may develop. Any of these relationships may require us to incur non-recurring and other charges, increase our near and long-term expenditures, issue securities that dilute our existing stockholders or disrupt our management and business. In addition, we face significant competition in seeking appropriate strategic partners and the negotiation process is time-consuming and complex. Moreover, we may not be successful in our efforts to establish a strategic partnership or other alternative arrangements for our product candidates because they may be deemed to be at too early of a stage of development for collaborative effort and third parties may not view our product candidates as having the requisite potential to demonstrate safety and efficacy. If we license products or businesses, we may not be able to realize the benefit of such transactions if we are unable to successfully integrate them with our existing operations and company culture. We cannot be certain that, following a strategic transaction or license, we will achieve the revenue or specific net income that justifies such transaction. Any delays in entering into new strategic partnership agreements related to our product candidates could delay the development and commercialization of our product candidates in certain geographies for certain indications, which would harm our business prospects, financial condition and results of operations.

***Our reliance on third parties requires us to share our trade secrets, which increases the possibility that a competitor will discover them or that our trade secrets will be misappropriated or disclosed.***

Because we rely on third parties to manufacture our drug substance and our drug product, and because we collaborate with various organizations and academic institutions on the advancement of our technology platform, we must, at times, share trade secrets with them. We seek to protect our proprietary technology in part by entering into confidentiality agreements and, if applicable, material transfer agreements, collaborative research agreements, consulting agreements or other similar agreements with our collaborators, advisors, employees and consultants prior

to beginning research or disclosing proprietary information. These agreements typically limit the rights of the third parties to use or disclose our confidential information, such as trade secrets. Despite these contractual provisions, the need to share trade secrets and other confidential information increases the risk that such trade secrets become known by our competitors, are inadvertently incorporated into the technology of others, or are disclosed or used in violation of these agreements. Given that our proprietary position is based, in part, on our know-how and trade secrets, a competitor's discovery of our trade secrets or other unauthorized use or disclosure would impair our competitive position and may have a material adverse effect on our business.

***We may use our financial and human resources to pursue a particular research program or product candidate and fail to capitalize on programs or product candidates that may be more profitable or for which there is a greater likelihood of success.***

Because we have limited resources, we may forego or delay pursuit of opportunities with certain programs or product candidates or for indications that later prove to have greater commercial potential. Our resource allocation decisions may cause us to fail to capitalize on viable commercial products or profitable market opportunities. Our spending on current and future research and development programs for product candidates may not yield any commercially viable products. If we do not accurately evaluate the commercial potential or target market for a particular product

## **Table of Contents**

candidate, we may relinquish valuable rights to that product candidate through strategic collaboration, licensing or other royalty arrangements in cases in which it would have been more advantageous for us to retain sole development and commercialization rights to such product candidate, or we may allocate internal resources to a product candidate in a therapeutic area in which it would have been more advantageous to enter into a partnering arrangement. We are particularly susceptible to this risk because we are pursuing clinical and preclinical development program in each of our CaspaCIDE, DeCIDE, CIDE-CAR and GoCAR-T technologies. Resources spent on one of these programs could result in fewer resources to further develop the other programs.

***We have limited information available regarding the ultimate cost of our products, and cannot estimate what the cost of our products will be upon commercialization, should that occur.***

We do not yet have sufficient information to reliably estimate the cost of the commercial manufacturing and processing of our product candidates, and the actual cost to manufacture and process our product candidates could materially and adversely affect the commercial viability of our product candidates. As a result, we may never be able to develop a commercially viable product. Because of the patient-specific nature of our manufacturing process, it is not amenable to traditional scale up to manufacture larger lots as is performed for traditional drugs and biological agents.

***We and our contractors utilize hazardous materials in our business operations, and any claims relating to improper handling, storage, or disposal of these materials could harm our business.***

Our activities involve the controlled use of potentially hazardous substances, including chemical and biological materials, by our third-party manufacturers. Our manufacturers are subject to federal, state and local laws and regulations in the United States governing the use, manufacture, storage, handling and disposal of medical and hazardous materials, and similar laws in other geographic regions. Although we believe that our manufacturers procedures for using, handling, storing and disposing of these materials comply with legally prescribed standards, we cannot completely eliminate the risk of contamination or injury resulting from medical or hazardous materials. As a result of any such contamination or injury, we may incur liability or local, city, state or federal authorities may curtail the use of these materials and interrupt our business operations. In the event of an accident, we could be held liable for damages or penalized with fines, and the liability could exceed our resources. We do not have any insurance for liabilities arising from medical or hazardous materials. Compliance with applicable environmental laws and regulations is expensive, and current or future environmental regulations may impair our research, development and production efforts, which could harm our business, prospects, financial condition or results of operations.

***Our internal computer systems, or those used by our clinical investigators, contractors or consultants, may fail or suffer security breaches.***

Despite the implementation of security measures, our internal computer systems and those of our contractors and consultants are vulnerable to damage from computer viruses and unauthorized access. While we have not experienced any such material system failure or security breach to date, if such an event were to occur and cause interruptions in our operations, it could result in a material disruption of our development programs and our business operations. For example, the loss of clinical trial data from completed or future clinical trials could result in delays in our regulatory approval efforts and significantly increase our costs to recover or reproduce the data. To the extent that any disruption or security breach were to result in a loss of, or damage to, our data or applications, or inappropriate disclosure of confidential or proprietary information, we could incur liability and the further development and commercialization of our product candidates could be delayed.

***Business disruptions could seriously harm our future revenue and financial condition and increase our costs and expenses.***

Our operations, and those of our clinical investigators, contractors and consultants, could be subject to power shortages, telecommunications failures, water shortages, floods, earthquakes, hurricanes, typhoons, fires, extreme weather conditions, medical epidemics and other natural or man-made disasters or business interruptions, for which we are predominantly self-insured. The occurrence of any of these business disruptions could seriously harm our operations and financial condition and increase our costs and expenses. We rely on third-party manufacturers to produce and process our product candidates on a patient by patient basis. Our ability to obtain clinical supplies of our product candidates could be disrupted if the operations of these suppliers are affected by a man-made or natural disaster or other business interruption.



---

**Table of Contents**

***Our employees, independent contractors, consultants, commercial partners and vendors may engage in misconduct or other improper activities, including noncompliance with regulatory standards and requirements.***

We are exposed to the risk of employee fraud or other illegal activity by our employees, independent contractors, consultants, commercial partners and vendors. Misconduct by these parties could include intentional, reckless and/or negligent conduct that fails to: comply with the laws of the FDA and other similar foreign regulatory bodies; provide true, complete and accurate information to the FDA and other similar foreign regulatory bodies, comply with manufacturing standards we have established, comply with healthcare fraud and abuse laws in the United States and similar foreign fraudulent misconduct laws, or report financial information or data accurately or to disclose unauthorized activities to us. If we obtain FDA approval of any of our product candidates and begin commercializing those products in the United States, our potential exposure under such laws will increase significantly, and our costs associated with compliance with such laws are also likely to increase. These laws may impact, among other things, our current activities with principal investigators and research patients, as well as proposed and future sales, marketing and education programs. In particular, the promotion, sales and marketing of healthcare items and services, as well as certain business arrangements in the healthcare industry, are subject to extensive laws designed to prevent fraud, kickbacks, self-dealing and other abusive practices. These laws and regulations may restrict or prohibit a wide range of pricing, discounting, marketing and promotion, structuring and commission(s), certain customer incentive programs and other business arrangements generally. Activities subject to these laws also involve the improper use of information obtained in the course of patient recruitment for clinical trials. The laws that may affect our ability to operate include, but are not limited to:

- n the federal Anti-Kickback Statute, which prohibits, among other things, knowingly and willfully soliciting, receiving, offering or paying any remuneration (including any kickback, bribe, or rebate), directly or indirectly, overtly or covertly, in cash or in kind, to induce, or in return for, either the referral of an individual, or the purchase, lease, order or recommendation of any good, facility, item or service for which payment may be made, in whole or in part, under a federal healthcare program, such as the Medicare and Medicaid programs;
- n federal civil and criminal false claims laws and civil monetary penalty laws, which prohibit, among other things, individuals or entities from knowingly presenting, or causing to be presented, claims for payment or approval from Medicare, Medicaid, or other third-party payors that are false or fraudulent or knowingly making a false statement to improperly avoid, decrease or conceal an obligation to pay money to the federal government;
- n the federal Health Insurance Portability and Accountability Act of 1996, or HIPAA, which created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud any healthcare benefit program or obtain, by means of false or fraudulent pretenses, representations, or promises, any of the money or property owned by, or under the custody or control of, any healthcare benefit program, regardless of the payor (e.g., public or private) and knowingly and willfully falsifying, concealing or covering up by any trick or device a material fact or making any materially false statements in connection with the delivery of, or payment for, healthcare benefits, items or services relating to healthcare matters;

- n HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act of 2009, or HITECH, and their respective implementing regulations, which impose requirements on certain covered healthcare providers, health plans, and healthcare clearinghouses as well as their respective business associates that perform services for them that involve the use, or disclosure of, individually identifiable health information, relating to the privacy, security and transmission of individually identifiable health information without appropriate authorization;
  
- n the federal Physician Payment Sunshine Act, created under the Health Reform Law, and its implementing regulations, which requires manufacturers of drugs, devices, biologicals and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report annually to the United States Department of Health and Human Services, or HHS, information related to payments or other transfers of value made to physicians (defined to include doctors, dentists, optometrists, podiatrists and chiropractors) and teaching hospitals, as well as ownership and investment interests held by physicians and their immediate family members; and

## **Table of Contents**

- n federal consumer protection and unfair competition laws, which broadly regulate marketplace activities and activities that potentially harm consumers.

Additionally, we are subject to state and foreign equivalents of each of the healthcare laws described above, among others, some of which may be broader in scope and may apply regardless of the payor.

Effective upon the completion of this offering, we will adopt a code of business conduct and ethics, but it is not always possible to identify and deter employee misconduct, and the precautions we take to detect and prevent inappropriate conduct may not be effective in controlling unknown or unmanaged risks or losses or in protecting us from governmental investigations or other actions or lawsuits stemming from a failure to be in compliance with such laws or regulations. Efforts to ensure that our business arrangements will comply with applicable healthcare laws may involve substantial costs. It is possible that governmental and enforcement authorities will conclude that our business practices may not comply with current or future statutes, regulations or case law interpreting applicable fraud and abuse or other healthcare laws and regulations. If any such actions are instituted against us, and we are not successful in defending ourselves or asserting our rights, those actions could have a significant impact on our business, including the imposition of civil, criminal and administrative penalties, damages, disgorgement, monetary fines, possible exclusion from participation in Medicare, Medicaid and other federal healthcare programs, contractual damages, reputational harm, diminished profits and future earnings, and curtailment of our operations, any of which could adversely affect our ability to operate our business and our results of operations. In addition, the approval and commercialization of any of our product candidates outside the United States will also likely subject us to foreign equivalents of the healthcare laws mentioned above, among other foreign laws.

***If product liability lawsuits are brought against us, we may incur substantial liabilities and may be required to limit commercialization of our product candidates.***

We face an inherent risk of product liability as a result of the clinical testing of our product candidates and will face an even greater risk if we commercialize any products. For example, we may be sued if our product candidates cause or are perceived to cause injury or are found to be otherwise unsuitable during clinical testing, manufacturing, marketing or sale. Any such product liability claims may include allegations of defects in manufacturing, defects in design, a failure to warn of dangers inherent in the product, negligence, strict liability or a breach of warranties. Claims could also be asserted under state consumer protection acts. If we cannot successfully defend ourselves against product liability claims, we may incur substantial liabilities or be required to limit commercialization of our product candidates. Even successful defense would require significant financial and management resources. Regardless of the merits or eventual outcome, liability claims may result in:

- n decreased demand for our product candidates;
- n injury to our reputation;
- n withdrawal of clinical trial participants;
- n initiation of investigations by regulators;

- n costs to defend the related litigation;
- n a diversion of management's time and our resources;
- n substantial monetary awards to clinical trial participants or patients;
- n product recalls, withdrawals or labeling, marketing or promotional restrictions;
- n loss of revenue;
- n exhaustion of any available insurance and our capital resources;
- n the inability to commercialize any product candidate; and
- n a decline in our share price.

Our inability to obtain sufficient product liability insurance at an acceptable cost to protect against potential product liability claims could prevent or inhibit the commercialization of any products we develop, alone or with corporate collaborators. We currently carry \$10.0 million of product liability insurance covering our clinical trials. Although we maintain such insurance, our insurance policies may have various exclusions, and we may be subject to a product liability claim for which we have no coverage. We may have to pay any amounts awarded by a court or negotiated in a settlement that exceed our coverage limitations or that are not covered by our insurance, and we may not have, or be

## **Table of Contents**

able to obtain, sufficient capital to pay such amounts. Even if our agreements with any future corporate collaborators entitle us to indemnification against losses, such indemnification may not be available or adequate should any claim arise.

### ***Our ability to utilize our net operating loss carryforwards and certain other tax attributes may be limited.***

Under Sections 382 and 383 of the Internal Revenue Code of 1986, as amended, if a corporation undergoes an ownership change (generally defined as a greater than 50% change (by value) in its equity ownership over a three-year period), the corporation's ability to use its pre-change net operating loss carryforwards and other pre-change tax attributes to offset its post-change income may be limited. As a result of our most recent private placements and other transactions that have occurred over the past three years, we may have experienced, and, upon completion of this offering, may experience, an ownership change. We may also experience ownership changes in the future as a result of subsequent shifts in our stock ownership. As of December 31, 2013, we had U.S. net operating loss carryforwards of approximately \$26.9 million, which begin to expire in 2024, and U.S. research and development credits of \$0.8 million, which could be limited if we experience an ownership change.

## **Risks Related to Government Regulation**

### ***The FDA regulatory approval process is lengthy and time-consuming, and we may experience significant delays in the clinical development and regulatory approval of our product candidates.***

We have not previously submitted a Biologics License Application, or BLA, to the FDA, or similar approval filings to comparable foreign authorities. A BLA must include extensive preclinical and clinical data and supporting information to establish the product candidate's safety, purity and potency for each desired indication. The BLA must also include significant information regarding the chemistry, manufacturing and controls for the product. We expect the novel nature of our product candidates to create further challenges in obtaining regulatory approval. For example, the FDA has limited experience with commercial development of T cell therapies for cancer. In addition, the cell and gene therapy office of the FDA has limited experience with combination products that include a small molecule component. Approval of our product candidates, including BPX-501, will require this FDA office to consult with another division of the FDA, which may result in further challenges in obtaining regulatory approval, including in developing final product labeling. The regulatory approval pathway for our product candidates may be uncertain, complex, expensive and lengthy, and approval may not be obtained.

We may also experience delays in completing planned clinical trials for a variety of reasons, including delays related to:

- n the availability of financial resources to commence and complete our planned clinical trials;
- n reaching agreement on acceptable terms with prospective clinical trial sites, the terms of which can be subject to extensive negotiation and may vary significantly among different clinical trial sites;
- n obtaining approval at each clinical trial site by an independent institutional review board, or IRB;

- n recruiting suitable patients to participate in a clinical trial;
- n having patients complete a clinical trial or return for post-treatment follow-up;
- n clinical trial sites deviating from clinical trial protocol, failing to follow GCPs, or dropping out of a clinical trial;
- n adding new clinical trial sites; or
- n manufacturing sufficient quantities of qualified materials under cGMPs and applying them on a subject by subject basis for use in clinical trials.

We could also encounter delays if physicians encounter unresolved ethical issues associated with enrolling patients in clinical trials of our product candidates in lieu of prescribing existing treatments that have established safety and efficacy profiles. Further, a clinical trial may be suspended or terminated by us, the IRBs for the institutions in which such clinical trials are being conducted, the Data Monitoring Committee for such clinical trial, or by the FDA or other regulatory authorities due to a number of factors, including failure to conduct the clinical trial in accordance with regulatory requirements or our clinical protocols, inspection of the clinical trial operations or clinical trial site by the FDA or other regulatory authorities resulting in the imposition of a clinical hold, unforeseen safety issues or adverse side effects, failure to demonstrate a benefit from using a product candidate, changes in governmental

## **Table of Contents**

regulations or administrative actions or lack of adequate funding to continue the clinical trial. If we experience termination of, or delays in the completion of, any clinical trial of our product candidates, the commercial prospects for our product candidates will be harmed, and our ability to generate product revenue will be delayed. In addition, any delays in completing our clinical trials will increase our costs, slow down our product development and approval process and jeopardize our ability to commence product sales and generate revenue.

Many of the factors that cause, or lead to, a delay in the commencement or completion of clinical trials may ultimately lead to the denial of regulatory approval of our product candidates.

***The FDA may disagree with our regulatory plans and we may fail to obtain regulatory approval of our product candidates.***

Our ongoing and planned Phase 1 and Phase 1/2 clinical trials of BPX-501 are designed to show enhanced immune system recovery in patients following an allogeneic (donor cells as opposed to the patient's own cells) HSCT. Following the completion of those clinical trials, and if the results are satisfactory, we plan to meet with the FDA in an end of phase two meeting to discuss our clinical trial design that could serve as the registration trial for our BLA for BPX-501 in that indication. We, or our institutional collaborators, are conducting and planning additional Phase 1 and Phase 1/2 clinical trials of BPX-501 in clinical trials designed to evaluate BPX-501 as a treatment for patients with recurrent disease (relapse) after an allogeneic HSCT. Following the completion of those clinical trials, and if the results are satisfactory, we plan to meet with the FDA in another end of phase two meeting to discuss whether our planned clinical trial design could serve as the registration trial for our BLA for BPX-501 in that indication. However, the general approach for FDA approval of a new biologic or drug is dispositive data from two adequate and well-controlled, Phase 3 clinical trials of the relevant biologic or drug in the relevant patient population. Phase 3 clinical trials typically involve hundreds of patients, have significant costs and take years to complete. We believe that a single Phase 3 clinical trial strategy is warranted given the limited alternatives for patients for which BPX-501 therapy is potentially beneficial, but the FDA may ultimately require more than one Phase 3 clinical trial and may limit clinical trial designs allowed to serve as a registration trial.

Our clinical trials results may not support approval. In addition, BPX-501 and our other product candidates could fail to receive regulatory approval for many reasons, including the following:

- n the FDA or comparable foreign regulatory authorities may disagree with the design or implementation of our clinical trials;
- n we may be unable to demonstrate to the satisfaction of the FDA or comparable foreign regulatory authorities that our product candidates have the necessary safety, purity, and potency for any of their proposed indications;
- n the results of clinical trials may not meet the level of statistical significance required by the FDA or comparable foreign regulatory authorities for approval;
- n we may be unable to demonstrate that our product candidates' clinical and other benefits outweigh their safety risks;

- n we may encounter serious and unexpected adverse events during clinical trials that render our products unsafe for use in humans;
- n the FDA or comparable foreign regulatory authorities may disagree with our interpretation of data from preclinical studies or clinical trials;
- n the data collected from clinical trials of our product candidates may not be sufficient to the satisfaction of the FDA or comparable foreign regulatory authorities to support the submission of a BLA or other comparable submission in foreign jurisdictions or to obtain regulatory approval in the United States or elsewhere;
- n the FDA or comparable foreign regulatory authorities may fail to approve our manufacturing processes and/or facilities of third-party manufacturers with which we contract for clinical and commercial supplies; and
- n the approval policies or regulations of the FDA or comparable foreign regulatory authorities may significantly change in a manner rendering our clinical data insufficient for approval.



## **Table of Contents**

***Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not mean that we will be successful in obtaining regulatory approval of our product candidates in other jurisdictions.***

Obtaining and maintaining regulatory approval of our product candidates in one jurisdiction does not guarantee that we will be able to obtain or maintain regulatory approval in any other jurisdiction, while a failure or delay in obtaining regulatory approval in one jurisdiction may have a negative effect on the regulatory approval process in others. For example, even if the FDA grants marketing approval of a product candidate, comparable regulatory authorities in foreign jurisdictions must also approve the manufacturing, marketing and promotion of the product candidate in those countries. Approval procedures vary among jurisdictions and can involve requirements and administrative review periods different from, and greater than, those in the United States, including additional preclinical studies or clinical trials. Studies and clinical trials conducted in one jurisdiction may not be accepted by regulatory authorities in other jurisdictions. In many jurisdictions outside the United States, a product candidate must be approved for reimbursement before it can be approved for sale in that jurisdiction. In some cases, the price that we intend to charge for our products is also subject to approval.

We may also submit marketing applications in other countries. Regulatory authorities in jurisdictions outside of the United States have requirements for approval of product candidates with which we must comply prior to marketing in those jurisdictions. Obtaining foreign regulatory approvals and compliance with foreign regulatory requirements could result in significant delays, difficulties and costs for us and could delay or prevent the introduction of our products in certain countries. If we fail to comply with the regulatory requirements in international markets and/or receive applicable marketing approvals, our target market will be reduced and our ability to realize the full market potential of our product candidates will be harmed.

***Even if we receive regulatory approval of our product candidates, we will be subject to ongoing regulatory obligations and continued regulatory review, which may result in significant additional expense and we may be subject to penalties and/or withdrawal of product approval if we fail to comply with regulatory requirements or experience unanticipated problems with our product candidates.***

Any regulatory approvals that we receive for our product candidates will require surveillance to monitor the safety and efficacy of the product candidate. The FDA may also require a risk evaluation and mitigation strategy, or REMS, in order to approve our product candidates, which could entail requirements for a medication guide, physician communication plans or additional elements to ensure safe use, such as restricted distribution methods, patient registries and other risk minimization tools. In addition, if the FDA or a comparable foreign regulatory authority approves our product candidates, the manufacturing processes, labeling, packaging, distribution, adverse event reporting, storage, advertising, promotion, import, export and recordkeeping for our product candidates will be subject to extensive and ongoing regulatory requirements. These requirements include, among other things, submissions of safety and other post-marketing information and reports, registration, as well as continued compliance with cGMPs and cGCPs for any clinical trials that we conduct post-approval. Later discovery of previously unknown problems with our product candidates, including adverse events of unanticipated severity or frequency, or our or with our third-party manufacturers or manufacturing processes, or failure to comply with regulatory requirements, may result in, among other things:

- n restrictions on the marketing or manufacturing of our product candidates, withdrawal of the product from the market, or voluntary or mandatory product recalls;

- n fines, warning letters or holds on clinical trials;
  
- n refusal by the FDA to approve pending applications or supplements to approved applications filed by us or suspension or revocation of license approvals;
  
- n suspension or termination of manufacturing at one or more manufacturing facilities;
  
- n product seizure or detention, or refusal to permit the import or export of our product candidates; and
  
- n injunctions or the imposition of civil or criminal penalties.

The FDA's and other regulatory authorities' policies may change and additional government regulations may be enacted that could prevent, limit or delay regulatory approval of our product candidates. We cannot predict the likelihood, nature or extent of government regulation that may arise from future legislation or administrative action, either in the United States or abroad. If we are slow or unable to adapt to changes in existing requirements or the adoption of new requirements or policies, or if we are not able to maintain regulatory compliance, we may lose any marketing approval that we may have obtained and we may not achieve or sustain profitability.

**Table of Contents**

*Even if we obtain regulatory approval of our product candidates, the products may not gain market acceptance among physicians, patients, hospitals, cancer treatment centers, third-party payors and others in the medical community.*

The use of engineered T cells as a potential cancer treatment is a recent development and may not become broadly accepted by physicians, patients, hospitals, cancer treatment centers, third-party payors and others in the medical community. We expect physicians in the large bone marrow transplant centers to be particularly influential and we may not be able to convince them to use our product candidates for many reasons. Factors will influence whether our product candidates are accepted in the market, including:

- n the clinical indications for which our product candidates are approved;
- n physicians, hospitals, cancer treatment centers and patients considering our product candidates as a safe and effective treatment;
- n the potential and perceived advantages of our product candidates over alternative treatments;
- n the prevalence and severity of any side effects;
- n product labeling or product insert requirements of the FDA or other regulatory authorities;
- n limitations or warnings contained in the labeling approved by the FDA;
- n the timing of market introduction of our product candidates as well as competitive products;
- n the cost of treatment in relation to alternative treatments;
- n the availability of adequate coverage, reimbursement and pricing by third-party payors and government authorities;
- n the willingness of patients to pay out-of-pocket in the absence of coverage by third-party payors, including government authorities;
- n relative convenience and ease of administration, including as compared to alternative treatments and competitive therapies;

n confusion or lack of understanding regarding the effects of rimiducid and the timing and size of dosing of rimiducid after immune cell therapy; and

n the effectiveness of our sales and marketing efforts.

In addition, although we are not utilizing embryonic stem cells or replication competent vectors, adverse publicity due to the ethical and social controversies surrounding the therapeutic use of such technologies, and reported side effects from any clinical trials using these technologies or the failure of such clinical trials to demonstrate that these therapies are safe and effective may limit market acceptance our product candidates. If our product candidates are approved but fail to achieve market acceptance among physicians, patients, hospitals, cancer treatment centers or others in the medical community, we will not be able to generate significant revenue.

Even if our products achieve market acceptance, we may not be able to maintain that market acceptance over time if new products or technologies are introduced that are more favorably received than our products, are more cost effective or render our products obsolete.

***We plan to seek orphan drug designation for BPX-501 and rimiducid as a combination therapy, but we may be unable to obtain such designation or maintain the benefits associated with orphan drug status, including market exclusivity.***

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition or for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for a disease or condition will be recovered from sales in the United States for that drug or biologic. If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a BLA to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity.

Rimiducid has orphan drug designation for the treatment of acute graft-versus-host-disease, or GvHD, in patients undergoing bone marrow transplantation. Since BPX-501 and rimiducid are considered a combination product, we

## **Table of Contents**

are currently discussing the designation of orphan for the combination of BPX-501 and rimiducid for treatment of immunodeficiency after allogeneic transplant with the FDA, but exclusive marketing rights in the United States may be limited if we seek approval for an indication broader than the orphan designated indication and may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition. In addition, while we intend to seek orphan drug designation for other product candidates, we may never receive such designations.

***Coverage and reimbursement may be limited or unavailable in certain market segments for our product candidates, which could make it difficult for us to sell our product candidates profitably.***

Successful sales of our product candidates, if approved, depend on the availability of coverage and adequate reimbursement from third-party payors. In addition, because our product candidates represent new approaches to the treatment of cancer, we cannot accurately estimate the potential revenue from our product candidates.

Patients who are provided medical treatment for their conditions generally rely on third-party payors to reimburse all or part of the costs associated with their treatment. Coverage and adequate reimbursement from governmental healthcare programs, such as Medicare and Medicaid, and commercial payors is critical to new product acceptance.

Government authorities and other third-party payors, such as private health insurers and health maintenance organizations, decide which drugs and treatments they will cover and the amount of reimbursement. Reimbursement by a third-party payor may depend upon a number of factors, including, but not limited to, the third-party payor's determination that use of a product is:

- n a covered benefit under its health plan;
- n safe, effective and medically necessary;
- n appropriate for the specific patient;
- n cost-effective; and

- n neither experimental nor investigational.

Obtaining coverage and reimbursement approval of a product from a government or other third-party payor is a time-consuming and costly process that could require us to provide to the payor supporting scientific, clinical and cost-effectiveness data for the use of our products. Even if we obtain coverage for a given product, the resulting reimbursement levels might not be adequate for us to achieve or sustain profitability or may require co-payments that patients find unacceptably high. Patients are unlikely to use our product candidates unless coverage is provided and reimbursement is adequate to cover a significant portion of the cost of our product candidates.

In the United States, no uniform policy of coverage and reimbursement for products exists among third-party payors. Therefore, coverage and reimbursement for products can differ significantly from payor to payor. As a result, the

coverage determination process is often a time-consuming and costly process that will require us to provide scientific and clinical support for the use of our product candidates to each payor separately, with no assurance that coverage and adequate reimbursement will be obtained.

We intend to seek approval to market our product candidates in both the United States and in selected foreign jurisdictions. If we obtain approval in one or more foreign jurisdictions for our product candidates, we will be subject to rules and regulations in those jurisdictions. In some foreign countries, particularly those in the EU, the pricing of biologics is subject to governmental control. In these countries, pricing negotiations with governmental authorities can take considerable time after obtaining marketing approval of a product candidate. In addition, market acceptance and sales of our product candidates will depend significantly on the availability of coverage and adequate reimbursement from third-party payors for our product candidates and may be affected by existing and future health care reform measures.

Third-party payors, whether domestic or foreign, or governmental or commercial, are developing increasingly sophisticated methods of controlling healthcare costs. In both the United States and certain foreign jurisdictions, there have been a number of legislative and regulatory changes to the health care system that could impact our ability to sell our products profitably. In particular, in 2010, the Patient Protection and Affordable Care Act, as

---

**Table of Contents**

amended by the Health Care and Education Reconciliation Act, collectively, the Healthcare Reform Act, was enacted in the United States. The Healthcare Reform Act and its implementing regulations, among other things, revised the methodology by which rebates owed by manufacturers to the state and federal government for covered outpatient drugs and certain biologics, including our product candidates, under the Medicaid Drug Rebate Program are calculated, increased the minimum Medicaid rebates owed by most manufacturers under the Medicaid Drug Rebate Program, extended the Medicaid Drug Rebate program to utilization of prescriptions of individuals enrolled in Medicaid managed care organizations, subjected manufacturers to new annual fees and taxes for certain branded prescription drugs, and provided incentives to programs that increase the federal government's comparative effectiveness research.

Other legislative changes have been proposed and adopted in the United States since the Healthcare Reform Act was enacted. In August 2011, the Budget Control Act of 2011, among other things, created measures for spending reductions by Congress. A Joint Select Committee on Deficit Reduction, tasked with recommending a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, was unable to reach required goals, thereby triggering the legislation's automatic reduction to several government programs. This includes aggregate reductions of Medicare payments to providers up to 2% per fiscal year, which went into effect on April 1, 2013, and will stay in effect through 2024 unless Congressional action is taken. In January 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, or the ATRA, which, among other things, reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers, and increased the statute of limitations period for the government to recover overpayments to providers from three to five years.

There have been, and likely will continue to be, legislative and regulatory proposals at the foreign, federal and state levels directed at broadening the availability of healthcare and containing or lowering the cost of healthcare. We cannot predict the initiatives that may be adopted in the future. The continuing efforts of the government, insurance companies, managed care organizations and other payors of healthcare services to contain or reduce costs of healthcare and/or impose price controls may adversely affect:

- n the demand for our product candidates, if we obtain regulatory approval;
- n our ability to set a price that we believe is fair for our products;
- n our ability to generate revenue and achieve or maintain profitability;
- n the level of taxes that we are required to pay; and
- n the availability of capital.

Any reduction in reimbursement from Medicare or other government programs may result in a similar reduction in payments from private payors, which may adversely affect our future profitability.

Third party payors may also have difficulty in determining the appropriate coverage of our product candidates, if approved, due to the fact that they are combination products that include a small molecule drug (rimiducid). To the extent there are any delays in determining such coverage or inadequate coverage for all aspects of our combination

therapies, it would adversely affect the market acceptance of our product candidates.

***Due to the novel nature of our technology and the small size of our target patient populations, we face uncertainty related to pricing and reimbursement for these product candidates.***

Our target patient populations are relatively small, as a result, the pricing and reimbursement of our product candidates, if approved, must be adequate to support commercial infrastructure. If we are unable to obtain adequate levels of reimbursement, our ability to successfully market and sell our product candidates will be adversely affected. The manner and level at which reimbursement is provided for services related to our product candidates (e.g., for administration of our product to patients) is also important. Inadequate reimbursement for such services may lead to physician resistance and adversely affect our ability to market or sell our products.



---

**Table of Contents**

**Risks Related to Our Intellectual Property**

*We depend on intellectual property licensed from third parties and termination of any of these licenses could result in the loss of significant rights, which would harm our business.*

We are dependent on patents, know-how and proprietary technology, both our own and licensed from others. Technology that we license from others includes rimiducid, which is the small molecule activating agent that forms a part of our current and future product candidates and that we license from ARIAD. ARIAD may terminate or modify our license upon a material breach by us that remains uncured following the date that is 30 days after written notice of a payment breach and 90 days for any other breach, and effective immediately upon certain insolvency events. In addition, ARIAD may terminate our license, upon notice to us, if we do not make certain lump sum installment payments within specific timeframes from the date such payments are due. In addition, ARIAD in-licenses some of the intellectual property rights it licenses to us. To the extent ARIAD fails to meet its obligations under its license agreements, which we are not in control of, we may lose the benefits of our license agreement with ARIAD. We license from Baylor College of Medicine, or Baylor, certain intellectual property related to methods for activating antigen presenting cells and to certain genetic constructs. Baylor may terminate or modify our licenses in the event of a material breach by us that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. In addition, we have funded certain of our ongoing clinical development and will fund certain of our future clinical development with funds from the state of Texas. The state may have rights to commercialize the results of those clinical trials if it determines that we have failed, after notice and an opportunity to cure, to use diligent and commercially reasonable efforts to commercialize or otherwise bring to practical application the results of the funded clinical trials. We are in advanced negotiations with Baylor for an exclusive license to intellectual property concerning the use of inducible caspase technology for elimination of transplanted cells.

Any termination of these agreements could result in the loss of significant rights and could harm our ability to commercialize our product candidates. See [Business Our License Agreements](#) for additional information regarding our license agreements.

Disputes may also arise between us and our licensors and other partners regarding intellectual property subject to a license agreement, including:

- n the scope of rights granted under the license agreement and other interpretation-related issues;
- n whether and the extent to which our technology and processes infringe on intellectual property of the licensor that is not subject to the licensing agreement;
- n our right to sublicense patent and other rights to third parties under collaborative development relationships;
- n our diligence obligations with respect to the use of the licensed technology in relation to our development and commercialization of our product candidates, and what activities satisfy those diligence obligations; and

n the ownership of inventions and know-how resulting from the joint creation or use of intellectual property by our licensors and us and our partners.

If disputes over intellectual property that we have licensed prevent or impair our ability to maintain our current licensing arrangements on acceptable terms, we may be unable to successfully develop and commercialize the affected product candidates.

We are generally also subject to all of the same risks with respect to protection of intellectual property that we license, as we are for intellectual property that we own, which are described below. If we or our licensors fail to adequately protect this intellectual property, our ability to commercialize products could suffer.

***If our efforts to protect the proprietary nature of our technologies are not adequate, we may not be able to compete effectively in our market.***

Any disclosure to or misappropriation by third parties of our confidential proprietary information could enable competitors to quickly duplicate or surpass our technological achievements, thus eroding our competitive position in our market. Certain intellectual property which is covered by our in-license agreements has been developed at academic institutions which have retained non-commercial rights to such intellectual property.

---

**Table of Contents**

There are several pending U.S. and foreign patent applications in our portfolio, and we anticipate additional patent applications will be filed both in the United States and in other countries, as appropriate. However, we cannot predict:

- n if and when patents will issue;
  
- n the degree and range of protection any issued patents will afford us against competitors including whether third parties will find ways to invalidate or otherwise circumvent our patents;
  
- n whether or not others will obtain patents claiming aspects similar to those covered by our patents and patent applications; or
  
- n whether we will need to initiate litigation or administrative proceedings which may be costly whether we win or lose.

Composition of matter patents for biological and pharmaceutical products are generally considered to be the strongest form of intellectual property. We cannot be certain that the claims in our pending patent applications directed to compositions of matter for our product candidates will be considered patentable by the United States Patent and Trademark Office, or the USPTO, or by patent offices in foreign countries, or that the claims in any of our issued patents will be considered valid by courts in the United States or foreign countries. Method of use patents have claims directed to the use of a product for the specified method. This type of patent does not prevent a competitor from making and marketing a product that is identical to our product for an indication that is outside the scope of the patented method. Moreover, even if competitors do not actively promote their product for our targeted indications, physicians may prescribe these products off-label. Although off-label prescriptions may infringe or contribute to the infringement of method of use patents, the practice is common and such infringement is difficult to prevent or prosecute.

The strength of patents in the biotechnology and pharmaceutical field involves complex legal and scientific questions and can be uncertain. The patent applications that we own or in-license may fail to result in issued patents with claims that cover our product candidates or uses thereof in the United States or in other foreign countries. Even if the patents do successfully issue, third parties may challenge the validity, enforceability or scope thereof, which may result in such patents being narrowed, invalidated or held unenforceable. Furthermore, even if they are unchallenged, patents in our portfolio may not adequately exclude third parties from practicing relevant technology or prevent others from designing around our claims. If the breadth or strength of our intellectual property position with respect to our product candidates is threatened, it could dissuade companies from collaborating with us to develop, and threaten our ability to commercialize, our product candidates. Further, if we encounter delays in our clinical trials, the period of time during which we could market our product candidates under patent protection would be reduced. Since patent applications in the United States and most other countries are confidential for a period of time after filing, we cannot be certain that patent applications in our portfolio were the first filed patent applications related to our product candidates. Furthermore, for United States applications in which all claims are entitled to a priority date before March 16, 2013, an interference proceeding can be provoked by a third-party or instituted by the USPTO, to determine who was the first to invent any of the subject matter covered by the patent claims of our applications. For United States applications containing a claim not entitled to priority before March 16, 2013, there is a greater level of uncertainty in the patent law with the passage of the America Invents Act (2012) which brings into effect significant changes to the U.S. patent laws that are yet untried and untested, and which introduces new procedures for challenging pending

patent applications and issued patents. A primary change under this reform is creating a first to file system in the U.S. This will require us to be cognizant going forward of the time from invention to filing of a patent application.

We are aware that patent coverage on rimiducid, the dimerization molecule AP1903, will expire in 2016. Any additional barriers to entry for competitors to use rimiducid after patent expiration may not be effective in preventing such use. There remain significant questions regarding how the FDA will interpret the biosimilar provisions recently added to the Public Health Service Act as applied to complex biological products such as our investigational products. Depending on how the FDA ultimately interprets these provisions, if our investigational products incorporating rimiducid receive FDA approval through a combination product BLA, then a biosimilar of these combination products could be approved by the FDA 12 years from the date that we receive FDA approval for our application. In addition, if a third party were able to obtain FDA approval of a new drug application for rimiducid on

## **Table of Contents**

its own, then it is possible that other third parties could later seek approval of an abbreviated new drug application for rimiducid.

We rely on trade secret protection and confidentiality agreements to protect proprietary know-how that is not patentable, processes for which patents are difficult to enforce and any other elements of our product discovery and development processes that involve proprietary know-how, information, or technology that is not covered by patents. We require all of our employees to assign their inventions to us, and require all of our employees, consultants, advisors and any third parties who have access to our proprietary know-how, information, or technology to enter into confidentiality agreements; however, we cannot be certain that our trade secrets and other confidential proprietary information will not be disclosed or that competitors will not otherwise gain access to our trade secrets or independently develop substantially equivalent information and techniques. Furthermore, the laws of some foreign countries do not protect proprietary rights to the same extent or in the same manner as the laws of the United States. As a result, we may encounter significant problems in protecting and defending our intellectual property both in the United States and abroad. If we are unable to prevent unauthorized material disclosure of our intellectual property to third parties, we will not be able to establish or maintain a competitive advantage in our market, which could materially adversely affect our business, operating results and financial condition.

### ***Third-party claims of intellectual property infringement may prevent or delay our product discovery and development efforts.***

Our commercial success depends in part on our avoiding infringement of the patents and proprietary rights of third parties. There is a substantial amount of litigation involving patents and other intellectual property rights in the biotechnology and pharmaceutical industries, as well as administrative proceedings for challenging patents, including interference and reexamination proceedings before the USPTO or oppositions and other comparable proceedings in foreign jurisdictions. Recently, under U.S. patent reform, new procedures including *inter partes* review and post grant review have been implemented. As stated above, this reform is untried and untested and will bring uncertainty to the possibility of challenge to our patents in the future. Numerous U.S. and foreign issued patents and pending patent applications, which are owned by third parties, exist in the fields in which we are developing our product candidates. As the biotechnology and pharmaceutical industries expand and more patents are issued, the risk increases that our product candidates may give rise to claims of infringement of the patent rights of others.

Third parties may assert that we are employing their proprietary technology without authorization. There may be third-party patents, of which we are currently unaware or have not sufficiently analyzed with claims to materials, formulations, methods of manufacture or methods for treatment related to the use or manufacture of our product candidates. Because patent applications can take many years to issue, there may be currently pending patent applications which may later result in issued patents that our product candidates may infringe. In addition, third parties may obtain patents in the future and claim that use of our technologies infringes upon these patents. If any third-party patents were held by a court of competent jurisdiction to cover aspects of our formulations, the manufacturing process of our product candidates, constructs or molecules used in or formed during the manufacturing process, methods of use, including combination therapy or patient selection methods or any final product itself, the holders of any such patents may be able to block our ability to develop and commercialize the product candidate unless we obtained a license under the applicable patents, or until such patents expire or they are finally determined to be held invalid or unenforceable. In either case, such a license may not be available on commercially reasonable terms or at all. If we are unable to obtain a necessary license to a third-party patent on commercially reasonable terms, or at all, our ability to commercialize our product candidates may be impaired or delayed, which could in turn significantly harm our business.

Parties making claims against us may seek and obtain injunctive or other equitable relief, which could effectively block our ability to further develop and commercialize our product candidates. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure. We cannot predict whether any such license would be available at all or whether it would be available on commercially reasonable terms. Furthermore, even in the absence of litigation, we may need to obtain licenses from third parties to advance our research or allow commercialization of our product candidates. We may fail to obtain any of these licenses at a reasonable cost or on reasonable terms, if at all. In that event, we would

## **Table of Contents**

be unable to further develop and commercialize our product candidates, which could harm our business significantly.

We are aware of a third party patent having claims directed to chimeric DNA comprising DNA segments encoding (1) a single chain antibody domain and (2) transmembrane and cytoplasmic domains of an endogenous protein. Even though we have reason to believe that our BPX-401 and BPX-601 technologies are not covered by claims of this patent, an owner or licensee of the patent still might bring a patent infringement suit against us. If the patent is asserted against us, we may not prevail in defending against claims of infringement and/or challenging the validity of claims in the patent. We may not successfully develop alternative technologies or enter into an agreement by which we obtain rights to the patent. These rights, if necessary, may not be available on terms acceptable to us.

Also, while we are aware there are third party patents having claims that may be considered relevant to BPX-201, BPX-401 and BPX-601 technologies for which we are seeking, or plan to seek, regulatory approval, we believe those patents have a patent term that may expire prior to the time we expect to obtain regulatory approval for these technologies. The estimated expiration dates for those patents were determined according to information on the face pages of the patents, and certain factors that could influence patent term, such as patent term adjustment and patent term extension, for example, were not factored into these estimates. Accordingly, the estimated expiration dates of those patents may not be accurate and one or more of those patents may not expire before we obtain regulatory approval for an applicable technology. Owners or licensees of one or more of those patents may bring a patent infringement suit against us. If one or more of those patents are asserted against us, we may be able to assert a defense for a safe harbor to patent infringement under 35 U.S.C. 271(e)(1) if certain requirements are met. It is possible that (1) certain of these requirements may not be met, and/or (2) one or more of the third party patents might expire after one or more of our technologies obtain regulatory approval, and consequently we may not successfully assert such a defense to patent infringement. If we are unsuccessful in asserting a defense under 35 U.S.C. 271(e)(1), it is possible we may not prevail in defending against claims of infringement and/or challenging the validity of claims in those patents. We may not successfully develop alternative technologies or enter into agreements by which we obtain rights to applicable patents. These rights, if necessary, may not be available on terms acceptable to us.

We are also aware of third party patents having claims directed to single-chain antibody fragments that bind to prostate stem cell antigen, or PSCA, and those patents may be considered relevant to BPX-601 technologies we are developing. We are currently evaluating whether or not a license may be obtained for rights to those patents. If we determine it is necessary to obtain rights to one or more of those patents, we may not successfully enter into an agreement or agreements required for obtaining rights to those patents, and these rights may not be available on terms acceptable to us. We also may not successfully develop alternative technologies if we cannot secure rights to those patents.

***We may not be successful in obtaining or maintaining necessary rights to product components and processes for our development pipeline through acquisitions and in-licenses.***

Because our programs may involve additional product candidates that may require the use of proprietary rights held by third parties, the growth of our business will likely depend in part on our ability to acquire, in-license or use these proprietary rights.

Our product candidates may also require specific formulations to work effectively and efficiently and these rights may be held by others. We may be unable to acquire or in-license any compositions, methods of use, processes or other third-party intellectual property rights from third parties that we identify. Even if we are able to obtain a license, it may be non-exclusive, thereby giving our competitors access to the same technologies licensed to us. In that event, we may be required to expend significant time and resources to develop or license replacement technology.

The licensing and acquisition of third-party intellectual property rights is a competitive area, and companies, which may be more established, or have greater resources than we do, may also be pursuing strategies to license or acquire third-party intellectual property rights that we may consider necessary or attractive in order to commercialize our product candidates. More established companies may have a competitive advantage over us due to their size, cash resources and greater clinical development and commercialization capabilities.

There can be no assurance that we will be able to successfully complete negotiations and ultimately acquire the rights to the intellectual property that we may seek to acquire in the future.



## **Table of Contents**

***We may be involved in lawsuits to protect or enforce our patents or the patents of our licensors, which could be expensive, time-consuming and unsuccessful.***

Competitors may infringe our patents or the patents of our licensors. To counter infringement or unauthorized use, we may be required to file infringement claims, which can be expensive and time-consuming. In addition, in an infringement proceeding, a court may decide that one or more of our patents is not valid or is unenforceable, or may refuse to stop the other party from using the technology at issue on the grounds that our patents do not cover the technology in question. An adverse result in any litigation or defense proceedings could put one or more of our patents at risk of being invalidated, held unenforceable, or interpreted narrowly and could put our patent applications at risk of not issuing. Defense of these claims, regardless of their merit, would involve substantial litigation expense and would be a substantial diversion of employee resources from our business. In the event of a successful claim of infringement against us, we may have to pay substantial damages, including treble damages and attorneys' fees for willful infringement, obtain one or more licenses from third parties, pay royalties or redesign our infringing products, which may be impossible or require substantial time and monetary expenditure.

Interference proceedings provoked by third parties or brought by the USPTO may be necessary to determine the priority of inventions with respect to our patents or patent applications or those of our licensors. An unfavorable outcome could result in a loss of our current patent rights and could require us to cease using the related technology or to attempt to license rights to it from the prevailing party. Our business could be harmed if the prevailing party does not offer us a license on commercially reasonable terms. Litigation or interference proceedings may result in a decision adverse to our interests and, even if we are successful, may result in substantial costs and distract our management and other employees. We may not be able to prevent, alone or with our licensors, misappropriation of our trade secrets or confidential information, particularly in countries where the laws may not protect those rights as fully as in the United States.

Furthermore, because of the substantial amount of discovery required in connection with intellectual property litigation, there is a risk that some of our confidential information could be compromised by disclosure during this type of litigation. In addition, there could be public announcements of the results of hearings, motions or other interim proceedings or developments. If securities analysts or investors perceive these results to be negative, it could have a substantial adverse effect on the price of our common stock.

***Obtaining and maintaining our patents depends on compliance with various procedural, document submission, fee payment and other requirements imposed by governmental patent agencies, and our patent position could be reduced or eliminated for non-compliance with these requirements.***

Periodic maintenance fees on any issued patent are due to be paid to the USPTO and foreign patent agencies in several stages over the lifetime of the patent. The USPTO and various foreign governmental patent agencies require compliance with a number of procedural, documentary, fee payment and other similar provisions during the patent application process. While an inadvertent lapse can in many cases be cured by payment of a late fee or by other means in accordance with the applicable rules, there are situations in which noncompliance can result in abandonment or lapse of the patent or patent application, resulting in partial or complete loss of patent rights in the relevant jurisdiction. Noncompliance events that could result in abandonment or lapse of a patent or patent application include, but are not limited to, failure to respond to official actions within prescribed time limits, non-payment of fees and failure to properly legalize and submit formal documents. Such noncompliance events are outside of our direct control for (1) non-U.S. patents and patent applications owned by us, and (2) patents and patent applications licensed to us by another entity. In such an event, our competitors might be able to enter the market, which would have a material adverse effect on our business.

***Issued patents covering our product candidates could be found invalid or unenforceable if challenged in court or the USPTO.***

If we or one of our licensing partners initiate legal proceedings against a third party to enforce a patent covering one of our product candidates, the defendant could counterclaim that the patent covering our product candidate, as applicable, is invalid and/or unenforceable. In patent litigation in the United States, defendant counterclaims alleging invalidity and/or unenforceability are commonplace, and there are numerous grounds upon which a third party can assert invalidity or unenforceability of a patent. Third parties may also raise similar claims before administrative bodies in the United States or abroad, even outside the context of litigation. Such mechanisms include re-examination, post grant review, and equivalent proceedings in foreign jurisdictions (e.g., opposition

---

**Table of Contents**

proceedings). Such proceedings could result in revocation or amendment to our patents in such a way that they no longer cover our product candidates. The outcome following legal assertions of invalidity and unenforceability is unpredictable. With respect to the validity question, for example, we cannot be certain that there is no invalidating prior art and that prior art that was cited during prosecution, but not relied on by the patent examiner, will not be revisited. If a defendant were to prevail on a legal assertion of invalidity and/or unenforceability, we would lose at least part, and perhaps all, of the patents directed to our product candidates. Such a loss of patent rights could have a material adverse impact on our business.

***Changes in U.S. patent law could diminish the value of patents in general, thereby impairing our ability to protect our products.***

As is the case with other biopharmaceutical companies, our success is heavily dependent on intellectual property, particularly patents. Obtaining and enforcing patents in the biopharmaceutical industry involve both technological and legal complexity, and is therefore costly, time-consuming and inherently uncertain. In addition, the United States has recently enacted and is currently implementing wide-ranging patent reform legislation. Recent U.S. Supreme Court rulings have narrowed the scope of patent protection available in certain circumstances and weakened the rights of patent owners in certain situations. In addition to increasing uncertainty with regard to our ability to obtain patents in the future, this combination of events has created uncertainty with respect to the value of patents, once obtained. Depending on decisions by the U.S. Congress, the federal courts, and the USPTO, the laws and regulations governing patents could change in unpredictable ways that would weaken our ability to obtain new patents or to enforce our existing patents and patents that we might obtain in the future. For example, in the recent case, *Assoc. for Molecular Pathology v. Myriad Genetics, Inc.*, the U.S. Supreme Court held that certain claims to DNA molecules are not patentable. While we do not believe that any of the patents owned or licensed by us will be found invalid based on this decision, we cannot predict how future decisions by the courts, the U.S. Congress or the USPTO may impact the value of our patents.

***We have limited foreign intellectual property rights and may not be able to protect our intellectual property rights throughout the world.***

We have limited intellectual property rights outside the United States. Filing, prosecuting and defending patents on product candidates in all countries throughout the world would be prohibitively expensive, and our intellectual property rights in some countries outside the United States can be less extensive than those in the United States. In addition, the laws of some foreign countries do not protect intellectual property to the same extent as federal and state laws in the United States. Consequently, we may not be able to prevent third parties from practicing our inventions in all countries outside the United States, or from selling or importing products made using our inventions in and into the United States or other jurisdictions. Competitors may use our technologies in jurisdictions where we have not obtained patents to develop their own products and further, may export otherwise infringing products to territories where we have patents, but enforcement is not as strong as that in the United States. These products may compete with our products and our patents or other intellectual property rights may not be effective or sufficient to prevent them from competing.

Many companies have encountered significant problems in protecting and defending intellectual property in foreign jurisdictions. The legal systems of certain countries, particularly China and certain other developing countries, do not favor the enforcement of patents, trade secrets and other intellectual property, particularly those relating to biopharmaceutical products, which could make it difficult for us to stop the infringement of our patents or marketing of competing products in violation of our proprietary rights generally. To date, we have not sought to enforce any issued patents in these foreign jurisdictions. Proceedings to enforce our patent rights in foreign jurisdictions could result in substantial costs and divert our efforts and attention from other aspects of our business, could put our patents

at risk of being invalidated or interpreted narrowly and our patent applications at risk of not issuing and could provoke third parties to assert claims against us. We may not prevail in any lawsuits that we initiate and the damages or other remedies awarded, if any, may not be commercially meaningful. The requirements for patentability may differ in certain countries, particularly developing countries. Furthermore, generic drug manufacturers or other competitors may challenge the scope, validity or enforceability of our or our licensors' patents, requiring us or our licensors to engage in complex, lengthy and costly litigation or other proceedings. Certain countries in Europe and developing countries, including China and India, have compulsory licensing laws under which a patent owner may be compelled to grant licenses to third parties. In those countries, we and our licensors may have limited remedies if patents are infringed or if we or our licensors are compelled to grant a license to a third party, which could materially

## **Table of Contents**

diminish the value of those patents. This could limit our potential revenue opportunities. Accordingly, our efforts to enforce our intellectual property rights around the world may be inadequate to obtain a significant commercial advantage from the intellectual property that we develop or license.

***We may be subject to claims that our employees, consultants or independent contractors have wrongfully used or disclosed confidential information of third parties.***

We have received confidential and proprietary information from third parties. In addition, we employ individuals who were previously employed at other biotechnology or pharmaceutical companies. We may be subject to claims that we or our employees, consultants or independent contractors have inadvertently or otherwise used or disclosed confidential information of these third parties or our employees' former employers. Litigation may be necessary to defend against these claims. Even if we are successful in defending against these claims, litigation could result in substantial cost and be a distraction to our management and employees.

## **Risks Related to This Offering and Ownership of our Common Stock**

***We do not know whether an active, liquid and orderly trading market will develop for our common stock or what the market price of our common stock will be and as a result it may be difficult for you to sell your shares of our common stock.***

Prior to this offering there has been no public market for shares of our common stock. Although we have applied to list our common stock on The NASDAQ Global Market, an active trading market for our shares may never develop or be sustained following this offering. You may not be able to sell your shares quickly or at the market price if trading in shares of our common stock is not active. The initial public offering price for our common stock was determined through negotiations with the underwriters, and the negotiated price may not be indicative of the market price of the common stock after the offering. As a result of these and other factors, you may be unable to resell your shares of our common stock at or above the initial public offering price. Further, an inactive market may also impair our ability to raise capital by selling shares of our common stock and may impair our ability to enter into strategic partnerships or acquire companies or products by using our shares of common stock as consideration.

***The price of our stock may be volatile, and you could lose all or part of your investment.***

The trading price of our common stock following this offering is likely to be highly volatile and could be subject to wide fluctuations in response to various factors, some of which are beyond our control, including limited trading volume. In addition to the factors discussed in this Risk Factors section and elsewhere in this prospectus, these factors include:

- n the commencement, enrollment or results of the planned clinical trials of our product candidates or any future clinical trials we may conduct, or changes in the development status of our product candidates;
- n any delay in our regulatory filings for our product candidates and any adverse development or perceived adverse development with respect to the applicable regulatory authority's review of such filings, including without limitation the FDA's issuance of a refusal to file letter or a request for additional information;

- n adverse results or delays in clinical trials;
- n our decision to initiate a clinical trial, not to initiate a clinical trial or to terminate an existing clinical trial;
- n adverse regulatory decisions, including failure to receive regulatory approval of our product candidates;
- n changes in laws or regulations applicable to our products, including but not limited to clinical trial requirements for approvals;
- n adverse developments concerning our CID technology platform and our small molecule drug rimiducid;
- n adverse developments concerning our manufacturers;
- n our inability to obtain adequate product supply for any approved product or inability to do so at acceptable prices;
- n our inability to establish collaborations if needed;
- n our failure to commercialize our product candidates;
- n additions or departures of key scientific or management personnel;
- n unanticipated serious safety concerns related to the use of our product candidates;
- n introduction of new products or services offered by us or our competitors;

**Table of Contents**

- n announcements of significant acquisitions, strategic partnerships, joint ventures or capital commitments by us or our competitors;
- n our ability to effectively manage our growth;
- n the size and growth of our initial target markets;
- n our ability to successfully treat additional types of diseases and cancers or at different stages;
- n actual or anticipated variations in quarterly operating results;
- n our cash position;
- n our failure to meet the estimates and projections of the investment community or that we may otherwise provide to the public;
- n publication of research reports about us or our industry, or immunotherapy in particular, or positive or negative recommendations or withdrawal of research coverage by securities analysts;
- n changes in the market valuations of similar companies;
- n overall performance of the equity markets;
- n sales of our common stock by us or our stockholders in the future;
- n trading volume of our common stock;
- n changes in accounting practices;
- n ineffectiveness of our internal controls;
- n disputes or other developments relating to proprietary rights, including patents, litigation matters and our ability to obtain patent protection for our technologies;

n significant lawsuits, including patent or stockholder litigation;

n general political and economic conditions; and

n other events or factors, many of which are beyond our control.

In addition, the stock market in general, and The NASDAQ Global Market and biopharmaceutical companies in particular, have experienced extreme price and volume fluctuations that have often been unrelated or disproportionate to the operating performance of these companies. Broad market and industry factors may negatively affect the market price of our common stock, regardless of our actual operating performance. If the market price of our common stock after this offering does not exceed the initial public offering price, you may not realize any return on your investment in us and may lose some or all of your investment. In the past, securities class action litigation has often been instituted against companies following periods of volatility in the market price of a company's securities. This type of litigation, if instituted, could result in substantial costs and a diversion of management's attention and resources, which would harm our business, operating results or financial condition.

***We do not intend to pay dividends on our common stock so any returns will be limited to the value of our stock.***

We currently anticipate that we will retain future earnings for the development, operation and expansion of our business and do not anticipate declaring or paying any cash dividends for the foreseeable future. Any return to stockholders will therefore be limited to the appreciation of their stock.

***Our principal stockholders and management own a significant percentage of our stock and will be able to exert significant control over matters subject to stockholder approval.***

Prior to this offering, our executive officers, directors, and 5% stockholders beneficially owned approximately 79.4% of our voting stock as of September 30, 2014, and, upon the closing of this offering, that same group will hold approximately 44.5% of our outstanding voting stock, assuming (1) conversion of all outstanding shares of convertible preferred stock into 12,224,819 shares of common stock, (2) no exercise of the underwriters' option to purchase additional shares, (3) election by all of our executive officers, directors or 5% stockholders that are holders of Series B convertible preferred stock to have their accrued dividends converted into common stock in connection with this offering, (4) net exercise by all of our executive officers, directors or 5% stockholders that are holders of warrants to purchase common stock of such warrants in full in connection with this offering, and (5) exercise by all of our executive officers, directors or 5% stockholders that are holders of warrants to purchase Series C convertible preferred stock of such warrants in full in connection with this offering, in each case based on the initial public offering price of \$19.00 per share. Therefore, even after this offering, these stockholders will have the ability to influence us through this ownership position. These stockholders may be able to determine all matters requiring



## **Table of Contents**

stockholder approval. For example, these stockholders may be able to control elections of directors, amendments of our organizational documents, or approval of any merger, sale of assets, or other major corporate transaction. This may prevent or discourage unsolicited acquisition proposals or offers for our common stock that you may feel are in your best interest as one of our stockholders.

***If you purchase our common stock in this offering, you will incur immediate and substantial dilution in the book value of your shares.***

The initial public offering price is substantially higher than the net tangible book value per share of our common stock. Investors purchasing common stock in this offering will pay a price per share that substantially exceeds the book value of our tangible assets after subtracting our liabilities. As a result, investors purchasing common stock in this offering will incur immediate dilution of \$12.07 per share, based on the initial public offering price of \$19.00 per share. Further, investors purchasing common stock in this offering will contribute approximately 50% of the total amount invested by stockholders since our inception, but will own only approximately 28.4% of the shares of common stock outstanding after giving effect to this offering.

This dilution is due to our investors who purchased shares prior to this offering having paid substantially less when they purchased their shares than the price offered to the public in this offering and the exercise of stock options granted to our employees. To the extent outstanding options or warrants are exercised, there will be further dilution to new investors. As a result of the dilution to investors purchasing shares in this offering, investors may receive significantly less than the purchase price paid in this offering, if anything, in the event of our liquidation. For a further description of the dilution that you will experience immediately after this offering, see Dilution.

***We are an emerging growth company, and we cannot be certain if the reduced reporting requirements applicable to emerging growth companies will make our common stock less attractive to investors.***

We are an emerging growth company, as defined in the Jumpstart Our Business Startups Act, or the JOBS Act, enacted in April 2012. For as long as we continue to be an emerging growth company, we may take advantage of exemptions from various reporting requirements that are applicable to other public companies that are not emerging growth companies, including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements and exemptions from the requirements of holding nonbinding advisory votes on executive compensation and stockholder approval of any golden parachute payments not previously approved. We could be an emerging growth company for up to five years following the year in which we complete this offering, although circumstances could cause us to lose that status earlier. We will remain an emerging growth company until the earlier of (1) the last day of the fiscal year (a) following the fifth anniversary of the completion of this offering, (b) in which we have total annual gross revenue of at least \$1 billion or (c) in which we are deemed to be a large accelerated filer, which requires the market value of our common stock that is held by non-affiliates to exceed \$700 million as of the prior June 30th, and (2) the date on which we have issued more than \$1 billion in non-convertible debt during the prior three-year period.

Even after we no longer qualify as an emerging growth company, we may still qualify as a smaller reporting company which would allow us to take advantage of many of the same exemptions from disclosure requirements including not being required to comply with the auditor attestation requirements of Section 404 of the Sarbanes-Oxley Act and reduced disclosure obligations regarding executive compensation in this prospectus and our periodic reports and proxy statements. We cannot predict if investors will find our common stock less attractive because we may rely on these exemptions. If some investors find our common stock less attractive as a result, there may be a less active trading market for our common stock and our stock price may be more volatile.

Under the JOBS Act, emerging growth companies can also delay adopting new or revised accounting standards until such time as those standards apply to private companies. We have irrevocably elected not to avail ourselves of this exemption from new or revised accounting standards and, therefore, will be subject to the same new or revised accounting standards as other public companies that are not emerging growth companies. As a result, changes in rules of U.S. generally accepted accounting principles or their interpretation, the adoption of new guidance or the application of existing guidance to changes in our business could significantly affect our financial position and results of operations.

---

**Table of Contents**

***We will incur significant increased costs as a result of operating as a public company, and our management will be required to devote substantial time to new compliance initiatives.***

As a public company, we will incur significant legal, accounting and other expenses that we did not incur as a private company. We will be subject to the reporting requirements of the Securities Exchange Act of 1934, which will require, among other things, that we file with the Securities and Exchange Commission, or the SEC, annual, quarterly and current reports with respect to our business and financial condition. In addition, the Sarbanes-Oxley Act, as well as rules subsequently adopted by the SEC and The NASDAQ Global Market to implement provisions of the Sarbanes-Oxley Act, impose significant requirements on public companies, including requiring establishment and maintenance of effective disclosure and financial controls and changes in corporate governance practices. Further, in July 2010, the Dodd-Frank Wall Street Reform and Consumer Protection Act, or the Dodd-Frank Act, was enacted. There are significant corporate governance and executive compensation related provisions in the Dodd-Frank Act that require the SEC to adopt additional rules and regulations in these areas such as say on pay and proxy access. Recent legislation permits emerging growth companies to implement many of these requirements over a longer period and up to five years from the pricing of this offering. We intend to take advantage of this new legislation but cannot guarantee that we will not be required to implement these requirements sooner than budgeted or planned and thereby incur unexpected expenses. Stockholder activism, the current political environment and the current high level of government intervention and regulatory reform may lead to substantial new regulations and disclosure obligations, which may lead to additional compliance costs and impact the manner in which we operate our business in ways we cannot currently anticipate.

We expect the rules and regulations applicable to public companies to substantially increase our legal and financial compliance costs and to make some activities more time-consuming and costly. If these requirements divert the attention of our management and personnel from other business concerns, they could have a material adverse effect on our business, financial condition and results of operations. The increased costs will decrease our net income or increase our net loss, and may require us to reduce costs in other areas of our business or increase the prices of our products or services. For example, we expect these rules and regulations to make it more difficult and more expensive for us to obtain director and officer liability insurance and we may be required to incur substantial costs to maintain the same or similar coverage. We cannot predict or estimate the amount or timing of additional costs we may incur to respond to these requirements. The impact of these requirements could also make it more difficult for us to attract and retain qualified persons to serve on our board of directors, our board committees or as executive officers.

***We have identified a material weakness in our internal control over financial reporting. If our internal control over financial reporting is not effective, we may not be able to accurately report our financial results or file our periodic reports in a timely manner, which may cause investors to lose confidence in our reported financial information and may lead to a decline in our stock price.***

Effective internal control over financial reporting is necessary for us to provide reliable financial reports in a timely manner. In connection with the audits of our financial statements for the years ended December 31, 2012 and 2013, we concluded that there was a material weakness in our internal control over financial reporting. A material weakness is a significant deficiency, or a combination of significant deficiencies, in internal control over financial reporting such that it is reasonably possible that a material misstatement of the annual or interim financial statements will not be prevented or detected on a timely basis.

The material weakness related to (1) a lack of internal controls over accounting and financial reporting, particularly surrounding nonroutine transactions and financial reporting, (2) a lack of sufficient staff, including the lack of a chief financial officer or other senior finance executive, and (3) a lack of formalized accounting policy and procedure documentation that is followed by accounting personnel. The material weakness resulted in adjustments that were

primarily related to non-routine transactions and impacted intangible assets, prepaid manufacturing costs, accrued liabilities, equity, expenses and income taxes.

In an attempt to remediate our resource weakness, we have hired a chief financial officer and plan to hire additional finance and accounting personnel to augment our accounting staff and to provide more resources for complex accounting matters and financial reporting. In addition, we are working to establish a standard accounting and operation procedures manual outlining the corporate policies and accounting practices to be followed. However, we cannot assure you that these efforts will remediate our material weakness in a timely manner, or at all, or prevent

## **Table of Contents**

restatements of our financial statements in the future. If we are unable to successfully remediate our material weakness or identify any future significant deficiencies or material weaknesses, the accuracy and timing of our financial reporting may be adversely affected, we may be unable to maintain compliance with securities law requirements regarding timely filing of periodic reports, and our stock price may decline as a result.

In addition, even if we remediate our material weakness, following the completion of this offering, we will be required to expend significant time and resources to further improve our internal controls over financial reporting, including by further expanding our finance and accounting staff. If we fail to adequately staff our accounting and finance function to remediate our material weaknesses and our significant deficiency or otherwise to meet the demands that will be placed upon us as a public company, including the requirements of the Sarbanes-Oxley Act, or fail to maintain adequate internal control over financial reporting, any new or recurring material weakness could prevent our management from concluding our internal control over financial reporting is effective and impair our ability to prevent material misstatements in our financial statements, which could cause our business to suffer.

### ***Changes in accounting rules, assumptions and/or judgments could materially and adversely affect us.***

Accounting rules and interpretations for certain aspects of our operations are highly complex and involve significant assumptions and judgment. These complexities could lead to a delay in the preparation and dissemination of our financial statements. Furthermore, changes in accounting rules and interpretations or in our accounting assumptions and/or judgments, such as asset impairments, could significantly impact our financial statements. In some cases, we could be required to apply a new or revised standard retroactively, resulting in restating prior period financial statements. Any of these circumstances could have a material adverse effect on our business, prospects, liquidity, financial condition and results of operations.

### ***Sales of a substantial number of shares of our common stock by our existing stockholders in the public market could cause our stock price to fall.***

If our existing stockholders sell, or indicate an intention to sell, substantial amounts of our common stock in the public market after the lock-up and other legal restrictions on resale discussed in this prospectus lapse, the trading price of our common stock could decline. Based on shares of common stock outstanding as of September 30, 2014, upon the closing of this offering we will have outstanding a total of 25,849,571 shares of common stock. Of these shares, only the shares of common stock sold in this offering by us, plus any shares sold upon exercise of the underwriters' option to purchase additional shares, will be freely tradable without restriction in the public market immediately following this offering. Jefferies LLC, and Citigroup Global Markets Inc. however, may, in their sole discretion, permit our officers, directors and other stockholders who are subject to these lock-up agreements to sell shares prior to the expiration of the lock-up agreements.

We expect that the lock-up agreements pertaining to this offering will expire 180 days from the date of this prospectus. After the lock-up agreements expire, up to an additional 14,349,205 shares of common stock will be eligible for sale in the public market, of which 5,698,431 shares are held by directors, executive officers and other affiliates and will be subject to volume limitations under Rule 144 under the Securities Act of 1933, as amended, or the Securities Act. In addition, shares of common stock that are either subject to outstanding options or reserved for future issuance under our employee benefit plans will become eligible for sale in the public market to the extent permitted by the provisions of various vesting schedules, the lock-up agreements and Rule 144 and Rule 701 under the Securities Act. If these additional shares of common stock are sold, or if it is perceived that they will be sold, in the public market, the trading price of our common stock could decline.

After this offering, the holders of approximately 16.9 million shares of our common stock as of September 30, 2014 will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the 180-day lock-up agreements described above. See Description of Capital Stock Registration Rights. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares held by affiliates, as defined in Rule 144 under the Securities Act. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock.

---

**Table of Contents**

***Future sales and issuances of our common stock or rights to purchase common stock, including pursuant to our equity incentive plan, could result in additional dilution of the percentage ownership of our stockholders and could cause our stock price to fall.***

We expect that significant additional capital may be needed in the future to continue our planned operations, including conducting clinical trials, commercialization efforts, expanded research and development activities and costs associated with operating a public company. To raise capital, we may sell common stock, convertible securities or other equity securities in one or more transactions at prices and in a manner we determine from time to time. If we sell common stock, convertible securities or other equity securities, investors may be materially diluted by subsequent sales. Such sales may also result in material dilution to our existing stockholders, and new investors could gain rights, preferences and privileges senior to the holders of our common stock, including shares of common stock sold in this offering.

***We have broad discretion in the use of the net proceeds from this offering and may not use them effectively.***

Our management will have broad discretion in the application of the net proceeds from this offering, including for any of the purposes described in the section entitled Use of Proceeds, and you will not have the opportunity as part of your investment decision to assess whether the net proceeds are being used appropriately. Because of the number and variability of factors that will determine our use of the net proceeds from this offering, their ultimate use may vary substantially from their currently intended use. Our management might not apply our net proceeds in ways that ultimately increase the value of your investment. We intend to use the net proceeds from this offering to fund (1) our ongoing and planned Phase 1/2 clinical trials of BPX-501; (2) pre-clinical and Phase 1/2 clinical trials of BPX-401, BPX-601 and BPX-701 and preclinical development of our other CAR T and TCR programs; (3) our planned Phase 1/2 clinical trials of BPX-201 in combination with checkpoint inhibitors; (4) build-out of our development and manufacturing capabilities; and (5) other working capital purposes, including general operating expenses. The failure by our management to apply these funds effectively could harm our business. Pending their use, we may invest the net proceeds from this offering in short-term, investment-grade, interest-bearing securities. These investments may not yield a favorable return to our stockholders. If we do not invest or apply the net proceeds from this offering in ways that enhance stockholder value, we may fail to achieve expected financial results, which could cause our stock price to decline.

***Anti-takeover provisions under our charter documents and Delaware law could delay or prevent a change of control which could limit the market price of our common stock and may prevent or frustrate attempts by our stockholders to replace or remove our current management.***

Our amended and restated certificate of incorporation and amended and restated bylaws, which are to become effective at or prior to the closing of this offering, contain provisions that could delay or prevent a change of control of our company or changes in our board of directors that our stockholders might consider favorable. Some of these provisions include:

- n a board of directors divided into three classes serving staggered three-year terms, such that not all members of the board will be elected at one time;
- n a prohibition on stockholder action through written consent, which requires that all stockholder actions be taken at a meeting of our stockholders;

- n a requirement that special meetings of stockholders be called only by the chairman of the board of directors, the chief executive officer, or by a majority of the total number of authorized directors;
- n advance notice requirements for stockholder proposals and nominations for election to our board of directors;
- n a requirement that no member of our board of directors may be removed from office by our stockholders except for cause and, in addition to any other vote required by law, upon the approval of not less than two-thirds of all outstanding shares of our voting stock then entitled to vote in the election of directors;
- n a requirement of approval of not less than two-thirds of all outstanding shares of our voting stock to amend any bylaws by stockholder action or to amend specific provisions of our certificate of incorporation; and
- n the authority of the board of directors to issue convertible preferred stock on terms determined by the board of directors without stockholder approval and which convertible preferred stock may include rights superior to the rights of the holders of common stock.



**Table of Contents**

In addition, because we are incorporated in Delaware, we are governed by the provisions of Section 203 of the Delaware General Corporate Law, which may prohibit certain business combinations with stockholders owning 15% or more of our outstanding voting stock. These anti-takeover provisions and other provisions in our amended and restated certificate of incorporation and amended and restated bylaws could make it more difficult for stockholders or potential acquirors to obtain control of our board of directors or initiate actions that are opposed by the then-current board of directors and could also delay or impede a merger, tender offer or proxy contest involving our company. These provisions could also discourage proxy contests and make it more difficult for you and other stockholders to elect directors of your choosing or cause us to take other corporate actions you desire. Any delay or prevention of a change of control transaction or changes in our board of directors could cause the market price of our common stock to decline.

***If securities or industry analysts do not publish research or publish inaccurate or unfavorable research about our business, our stock price and trading volume could decline.***

The trading market for our common stock will depend in part on the research and reports that securities or industry analysts publish about us or our business. Securities and industry analysts do not currently, and may never, publish research on our company. If no securities or industry analysts commence coverage of our company, the trading price for our stock would likely be negatively impacted. In the event securities or industry analysts initiate coverage, if one or more of the analysts who covers us downgrades our stock or publishes inaccurate or unfavorable research about our business, our stock price may decline. If one or more of these analysts ceases coverage of our company or fails to publish reports on us regularly, demand for our stock could decrease, which might cause our stock price and trading volume to decline.

**Table of Contents**

**SPECIAL NOTE REGARDING FORWARD-LOOKING STATEMENTS**

This prospectus, including the sections entitled Prospectus Summary, Risk Factors, Management's Discussion and Analysis of Financial Condition and Results of Operations and Business, contains forward-looking statements. We may, in some cases, use words such as anticipate, believe, could, estimate, expect, intend, may, plan, predict, project, should, will, would or the negative of those terms, and similar expressions that convey uncertainty about future events or outcomes to identify these forward-looking statements. Any statements contained herein that are not statements of historical facts may be deemed to be forward-looking statements. Forward-looking statements in this prospectus include, but are not limited to, statements about:

- n the success, cost and timing of our product development activities and clinical trials;
- n our ability to advance CID-based technologies, including CaspaCIDE, CIDE CAR, GoCAR-T and DeCIDE;
- n our ability to obtain and maintain regulatory approval of BPX-501 and any other product candidates, and any related restrictions, limitations and/or warnings in the label of an approved product candidate;
- n our ability to obtain funding for our operations, including funding necessary to complete further development and commercialization of our product candidates;
- n the commercialization of our product candidates, if approved;
- n our plans to research, develop and commercialize our product candidates;
- n our ability to attract collaborators with development, regulatory and commercialization expertise;
- n future agreements with third parties in connection with the commercialization of our product candidates and any other approved product;
- n the size and growth potential of the markets for our product candidates, and our ability to serve those markets;
- n the rate and degree of market acceptance of our product candidates;
- n regulatory developments in the United States and foreign countries;

- n our ability to contract with third-party suppliers and manufacturers and their ability to perform adequately;
- n the success of competing therapies that are or may become available;
- n our ability to attract and retain key scientific or management personnel, including a Chief Financial Officer;
- n our ability to grow our organization and increase the size of our facilities to meet our anticipated growth;
- n the accuracy of our estimates regarding expenses, future revenue, capital requirements and needs for additional financing;
- n our expectations regarding the period during which we qualify as an emerging growth company under the JOBS Act;
- n our use of the proceeds from this offering; and
- n our expectations regarding our ability to obtain and maintain intellectual property protection for our product candidates.

These forward-looking statements reflect our management's beliefs and views with respect to future events and are based on estimates and assumptions as of the date of this prospectus and are subject to risks and uncertainties. We discuss many of these risks in greater detail under Risk Factors. Moreover, we operate in a very competitive and rapidly changing environment. New risks emerge from time to time. It is not possible for our management to predict all risks, nor can we assess the impact of all factors on our business or the extent to which any factor, or combination of factors, may cause actual results to differ materially from those contained in any forward-looking statements we may make. Given these uncertainties, you should not place undue reliance on these forward-looking statements.

You should read this prospectus and the documents that we reference in this prospectus and have filed as exhibits to the registration statement, of which this prospectus is a part, completely and with the understanding that our actual future results may be materially different from what we expect. We qualify all of the forward-looking statements in this prospectus by these cautionary statements. Except as required by law, we undertake no obligation to publicly update any forward-looking statements, whether as a result of new information, future events or otherwise.

**Table of Contents**

**USE OF PROCEEDS**

We estimate that we will receive net proceeds of approximately \$127.1 million (or approximately \$146.5 million if the underwriters' option to purchase additional shares is exercised in full) from the sale of the shares of common stock offered by us in this offering after deducting the underwriting discounts and commissions and estimated offering expenses payable by us.

The principal purposes of this offering are to obtain additional capital to support our operations, to establish a public market for our common stock and to facilitate our future access to the public equity markets. We intend to use the net proceeds from this offering for the following purposes:

- n approximately \$21.0 million to fund our ongoing and planned Phase 1/2 clinical trials of BPX-501;
- n approximately \$30.0 million to fund pre-clinical studies for BPX-401, BPX-601 and BPX-701 and fund the Phase 1/2 clinical trial of BPX-401, partially fund the planned Phase 1/2 clinical trial of BPX-601 and fund the Phase 1/2 clinical trial of BPX-701 as well as preclinical development of our other CAR T and TCR programs;
- n approximately \$4.0 million to fund our ongoing Phase 1/2 clinical trial and our planned Phase 1/2 clinical trial of BPX-201 in combination with checkpoint inhibitors;
- n approximately \$11.0 million to fund the construction of tenant improvements and the purchase of capital equipment at our Houston facility, to accommodate our anticipated personnel needs for the next three years and to support our planned in-house new product discovery and development, as well as process development and manufacturing for U.S. clinical trials of planned product candidates or certain critical components thereof; and
- n the remainder to fund other working capital purposes, including general operating expenses.

We may also use a portion of the remaining net proceeds to in-license, acquire, or invest in complementary businesses, technologies, intellectual property, products or assets. However, we have no current commitments or obligations to do so.

Furthermore, we anticipate that we will use our cash and cash equivalents on hand to pay our obligations under a promissory note held by ARIAD, or the ARIAD note, including \$20 million of which we expect to pay upon the closing of this offering and \$15 million of which we intend to pay within nine months of the closing of this offering.

Our expected use of the net proceeds from this offering represents our current intentions based upon our present plans and business condition. As of the date of this prospectus, we cannot predict with certainty all of the particular uses for the net proceeds to be received upon the completion of this offering, or the amounts that we will actually spend on the uses set forth above. The amounts and timing of our actual use of the net proceeds will vary depending on numerous factors, including our ability to gain access to additional financing, the relative success and cost of our research, preclinical and clinical development programs and whether we are able to enter into future licensing arrangements. As

a result, our management will have broad discretion in the application of the net proceeds, and investors will be relying on our judgment regarding the application of the net proceeds of this offering. In addition, we might decide to postpone or not pursue clinical trials or preclinical activities if the net proceeds from this offering and any other sources of cash are less than expected.

Pending their use, we plan to invest the net proceeds from this offering in short- and intermediate-term, interest-bearing obligations, investment-grade instruments, certificates of deposit or direct or guaranteed obligations of the U.S. government.

**Table of Contents**

**DIVIDEND POLICY**

Prior to the filing of our amended and restated certificate of incorporation in connection with our Series C financing in August 2014, the holders of our Series B convertible preferred stock were entitled to receive an annual accrued dividend equal to 6% of the original purchase price for shares of our Series B convertible preferred stock. These accrued dividends are payable upon conversion of the Series B convertible preferred stock, which will occur in connection with the closing of this offering, and will be paid in cash, unless a holder requests that such dividend be paid in shares of our common stock. The aggregate accrued dividend is approximately \$3.4 million. Except for the foregoing, we do not anticipate declaring or paying, in the foreseeable future, any cash dividends on our capital stock. We currently intend to retain all available funds and any future earnings to support our operations and finance the growth and development of our business. Any future determination related to our dividend policy will be made at the discretion of our board of directors and will depend upon, among other factors, our results of operations, financial condition, capital requirements, contractual restrictions, business prospects and other factors our board of directors may deem relevant. In addition, the terms of our existing line of credit prohibits us from paying dividends. We intend to request a waiver of this prohibition in connection with the payment of the accrued dividend to holders of our Series B convertible preferred stock referred to above.

**Table of Contents**

**CAPITALIZATION**

The following table sets forth our cash and cash equivalents, and our capitalization as of September 30, 2014:

- n on an actual basis;
  
- n a 1-for-1.7 reverse stock split of our common stock to be effected prior to the effectiveness of the registration statement of which this prospectus is a part;
  
- n on a pro forma basis, giving effect to (1) the conversion of all our outstanding shares of our convertible preferred stock as of September 30, 2014 into an aggregate of 12,224,819 shares of our common stock in connection with the closing of this offering, (2) the net exercise of outstanding warrants to purchase shares of our common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, (3) the issuance by us of 177,349 shares of common stock, as payment of the accrued dividends on the outstanding shares of Series B convertible preferred stock payable to the holders of Series B convertible preferred stock in connection with this offering, and (4) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants (which will expire upon the date immediately following the date of effectiveness of the registration statement of which this prospectus forms a part if not exercised), and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering;
  
- n pro forma and pro forma as adjusted amounts reflect the October 2014 amendment to our license agreement with Ariad, pursuant to which we agreed to pay \$50.0 million in exchange for expanded use of the license and the termination of all obligations to make milestone and royalty payments to Ariad in the future. We have reflected (i) a decrease in cash for the initial payment of \$15.0 million in October 2014, (ii) a liability of \$35.0 million to recognize the promissory note for the remaining balance, (iii) a \$5.1 million reduction in equity to reflect the estimated fair value, as of October 2014, of the common stock to be returned to us in connection with the second payment to Ariad and (iv) a \$44.9 million charge to research and development expense. The final accounting treatment is still under review and may ultimately result in different accounting treatment, including discounting the liability or less research and development expense; and
  
- n pro forma as adjusted amounts reflect the pro forma conversion adjustments described above as well as the sale of 7,350,000 shares of our common stock in this offering at the initial public offering price of \$19.00 per share and after deducting the estimated underwriting discounts and commissions and estimated offering expenses payable by us.

**Table of Contents**

The pro forma information below is illustrative only and our capitalization following the closing of this offering will be adjusted based on the actual initial public offering price and other terms of this offering determined at pricing. You should read this table together with Selected Financial Data and Management's Discussion and Analysis of Financial Condition and Results of Operations and our financial statements and the related notes appearing elsewhere in this prospectus.

(in thousands, except per share data)	AS OF SEPTEMBER 30, 2014		
	ACTUAL (unaudited)	PRO FORMA <sup>(1)(2)(3)</sup>	PRO FORMA AS ADJUSTED <sup>(1)(2)(3)</sup>
Cash and cash equivalents	\$61,932	\$ 86,287	\$ 213,469
Series C warrant liability	10,598		
Note payable to ARIAD		35,000	35,000
Convertible redeemable preferred stock:			
Series A preferred stock, \$0.01 par value: 2,600,000 shares authorized and 2,544,539 shares issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	7,634		
Series B preferred stock, \$0.01 par value: 8,200,000 shares authorized and 8,145,988 issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted	41,045		
Series C preferred stock, \$0.01 par value: 16,700,000 shares authorized and 10,091,743 issued and outstanding, actual; no shares authorized, issued or outstanding, pro forma and pro forma as adjusted .	42,074		
Stockholders' (deficit) equity:			
Common stock, \$0.01 par value: 37,500,000 shares authorized and 2,124,386 shares issued and outstanding, actual; 200,000,000 shares authorized and 18,499,571 shares issued and outstanding, pro forma; and 200,000,000 shares authorized and 25,849,571 shares issued and outstanding, pro forma as adjusted	21	185	258
Additional paid-in capital	(169)	135,287	262,314
Accumulated deficit	(38,646)	(83,559)	(83,559)
Total stockholders' (deficit) equity	(38,794)	51,913	179,013
Total capitalization	\$ 62,557	\$ 86,913	\$ 214,013



- (1) If any holder of Series B convertible preferred stock elects to receive their accrued dividends in cash, the amount of cash and cash equivalents will decrease by the amount of cash paid as a dividend (up to a maximum decrease of \$3.4 million) and the paid-in-capital, total stockholders' (deficit) equity and total capitalization will decrease correspondingly to reflect the issuance of fewer shares of common stock (up to a maximum of 177,349 fewer shares, if all dividends are paid in cash).
- (2) If any holder of a warrant to purchase common stock elects to exercise such holder's common stock warrant for cash, instead of net exercise, the amount of cash and cash equivalents will increase by the amount of cash paid for such exercise (up to a maximum increase of \$60,001 if all warrants to purchase common stock are exercised in full for cash) and the additional paid-in capital, total stockholders' (deficit) equity and total capitalization will be correspondingly increased to reflect the issuance of additional shares of common stock (up to a maximum of 3,171 additional shares of common stock if all warrants to purchase common stock are exercised in full for cash). If any holder of a warrant to purchase common stock does not elect to exercise such holder's common stock warrant, the common stock outstanding, additional paid in capital, total stockholders' (deficit) equity and total capitalization will be correspondingly decreased to reflect the issuance of less shares of common stock upon exercise of such warrants. If any holder of a warrant to purchase Series C convertible preferred stock does not elect to exercise such holder's warrant prior to this offering, the amount of cash and cash equivalents will decrease by such amount and the additional paid-in capital, total stockholders' (deficit) equity and total capitalization will correspondingly decrease to reflect the issuance of a lower number of shares for cash by us.

**Table of Contents**

- (3) Pro forma amounts reflect the October 2014 amendment to our license agreement with Ariad, pursuant to which we agreed to pay \$50.0 million in exchange for expanded use of the license and the termination of all obligations to make milestone and royalty payments to Ariad in the future. We have reflected (i) a decrease in cash for the initial payment of \$15.0 million in October 2014, (ii) a liability of \$35.0 million to recognize the promissory note for the remaining balance, (iii) a \$5.1 million reduction in equity to reflect the estimated fair value, as of October 2014, of the common stock to be returned to us in connection with the second payment to Ariad and (iv) a \$44.9 million charge to research and development expense. The final accounting treatment is still under review and may ultimately result in different accounting treatment, including discounting the liability or less research and development expense.

The number of shares of our common stock to be outstanding after this offering is based on 2,124,386 shares of common stock outstanding as of September 30, 2014, and assumes:

- n the issuance by us of 7,350,000 shares of our common stock in this offering;
- n the conversion of all of our convertible preferred stock outstanding into an aggregate of 12,224,819 shares of common stock upon the closing of this offering;
- n the net exercise of outstanding warrants to purchase common stock (which warrants will expire upon the closing of this offering if not exercised) for an aggregate of 114,468 shares of common stock;
- n that all of the holders of Series B convertible preferred stock will elect to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock in connection with this offering, which will result in the issuance by us of 177,349 shares of common stock; and
- n the issuance by us of 6,559,598 shares of Series C convertible preferred stock issued upon the exercise of warrants issued by us in August 2014, pursuant to that certain Series C Preferred Stock and Warrant Purchase Agreement, or the Series C Purchase Agreement (which warrants will expire upon the date immediately following the date of effectiveness of the registration statement of which this prospectus forms a part if not exercised), and the conversion of these shares of Series C convertible preferred stock into an aggregate of 3,858,549 shares of common stock;

and excludes:

- n 1,602,339 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2014, at a weighted-average exercise price of \$2.33 per share;
- n 2,956,909 shares of our common stock reserved for future issuance under the 2014 Plan, which will become effective as of the date of the effectiveness of the registration statement to which this prospectus is a part which number includes the 258,823 shares subject to stock options and a stock award that will be granted

upon the effective date of the 2014 Plan and includes the 1,382,481 shares of common stock reserved for issuance under the 2011 Plan as of September 30, 2014, reduced by the 1,031,454 shares of common stock issuable upon the exercise of the stock options granted under the 2011 Plan subsequent to September 30, 2014, which aggregate of 351,027 shares will be added to the shares reserved under the 2014 Plan when the 2014 Plan becomes effective;

- n 550,000 shares of common stock reserved for future issuance under the ESPP, which will become effective upon the execution and delivery of the underwriting agreement for this offering; and
  
- n 355,392 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2014, at an exercise price of \$0.0017 per share.

Table of Contents**DILUTION**

If you invest in our common stock in this offering, your ownership interest will be immediately diluted to the extent of the difference between the initial public offering price per share of our common stock and the pro forma as adjusted net tangible book value per share of our common stock after this offering.

Our historical net tangible book value as of September 30, 2014, was approximately \$(51.72) million, or \$(24.35) per share of our common stock. Our historical net tangible book value is the amount of our total tangible assets less our liabilities and the redemption value of convertible preferred stock which is not included within stockholders' equity. Historical net tangible book value per share is our historical net tangible book value divided by the number of shares of common stock outstanding as of September 30, 2014.

Our pro forma net tangible book value as of September 30, 2014, was \$51.9 million, or \$2.81 per share of common stock. Pro forma net tangible book value gives effect to (1) the conversion of all our outstanding shares of our convertible preferred stock as of September 30, 2014 into an aggregate of 12,224,819 shares of our common stock in connection with the closing of this offering, (2) the net exercise of outstanding warrants to purchase shares of our common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, (3) the issuance by us of 177,349 shares of common stock, based on the initial public offering price of \$19.00 per share as payment of the accrued dividends on the outstanding shares of Series B convertible preferred stock payable to the holders of Series B convertible preferred stock in connection with this offering, and (4) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants (which will expire upon the date immediately following the date of effectiveness of the registration statement of which this prospectus forms a part, if not exercised), and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering.

Pro forma as adjusted net tangible book value is our pro forma net tangible book value (deficit), plus the effect of the sale of 7,350,000 shares of our common stock in this offering at the initial public offering price of \$19.00 per share, and after deducting the underwriting discounts and commissions and estimated offering expenses payable by us. This amount represents an immediate increase in pro forma as adjusted net tangible book value of \$4.12 per share to our existing stockholders, and an immediate dilution of \$12.07 per share to new investors participating in this offering.

The following table illustrates this dilution on a per share basis:

Initial public offering price per share	\$ 19.00
Historical net tangible book value per share as of September 30, 2014	\$ (24.35)
Pro forma increase in net tangible book value per share as of September 30, 2014 attributable to the conversion of outstanding preferred stock	27.15
Pro forma net tangible book value per share as of September 30, 2014	2.81
Increase in pro forma net tangible book value per share attributable to investors participating in this offering	4.12

Pro forma as adjusted net tangible book value per share after this offering	6.93
Pro forma as adjusted dilution per share to investors participating in this offering	\$ 12.07

If the underwriters exercise in full their option to purchase 1,102,500 additional shares of our common stock in this offering, the pro forma as adjusted net tangible book value will increase to \$7.36 per share, representing an immediate increase in pro forma as adjusted net tangible book value to existing stockholders of \$0.44 per share and an immediate decrease of dilution of \$11.64 per share to new investors participating in this offering.

**Table of Contents**

The following table summarizes, on a pro forma as adjusted basis as of September 30, 2014, the number of shares purchased or to be purchased from us, the total consideration paid or to be paid to us, and the average price per share paid or to be paid to us by existing stockholders and investors participating in this offering at the initial public offering price of \$19.00 per share, before deducting estimated underwriting discounts and commissions and estimated offering expenses payable by us. As the table below shows, investors participating in this offering will pay an average price per share substantially higher than our existing stockholders paid.

	SHARES PURCHASED		TOTAL CONSIDERATION		AVERAGE PRICE
	NUMBER	PERCENT	AMOUNT	PERCENT	PER SHARE
Existing stockholders before this offering	18,499,571	71.6%	\$ 139,666,367	50.0%	\$ 7.54
Investors participating in this offering	7,350,000	28.4	139,650,000	50.0	19.00
<b>Total</b>	<b>25,849,571</b>	<b>100%</b>	<b>\$ 279,316,367</b>	<b>100%</b>	

Certain of existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering.

If the underwriters exercise in full their option to purchase 1,102,500 additional shares of our common stock in this offering, the number of shares of common stock held by existing stockholders will be reduced to 68.6% of the total number of shares of common stock to be outstanding after this offering, and the number of shares of common stock held by investors participating in this offering will be further increased to 8,452,500, or 31.4% of the total number of shares of common stock to be outstanding after this offering.

The number of shares of our common stock to be outstanding after this offering is based on 2,124,386 shares of common stock outstanding as of September 30, 2014, and assumes:

- n the issuance by us of 7,350,000 shares of our common stock in this offering;
- n the conversion of all of our convertible preferred stock outstanding into an aggregate of 12,224,819 shares of common stock upon the closing of this offering;

- n the net exercise of outstanding warrants to purchase common stock for an aggregate of 114,468 shares of common stock;
  - n that all of the holders of Series B convertible preferred stock will elect to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock in connection with this offering, which will result in the issuance by us of 177,349 shares of common stock, based on the initial public offering price of \$19.00; and
  - n the issuance by us of 6,559,598 shares of our Series C convertible preferred stock issuable upon the exercise of warrants, issued by us in August 2014, pursuant to the Series C Purchase Agreement, and the conversion of these shares of Series C convertible preferred stock into an aggregate of 3,858,549 shares of common stock;
- and excludes:
- n 1,602,339 shares of common stock issuable upon the exercise of outstanding stock options as of September 30, 2014, at a weighted-average exercise price of \$2.33 per share;
  - n 2,956,909 shares of our common stock reserved for future issuance under our 2014 equity incentive plan, or the 2014 Plan, which will become effective as of the date of the effectiveness of the registration statement of which this prospectus forms a part, which number includes the 258,823 shares subject to stock options and a stock award that will be granted upon the effective date of the 2014 Plan and includes the 1,382,481 shares of common stock reserved for issuance under our 2011 stock option plan, as amended, or the 2011 Plan as of September 30, 2014, reduced by the 1,031,454 shares of common stock

**Table of Contents**

issuable upon the exercise of the stock options granted under the 2011 Plan subsequent to September 30, 2014, which aggregate of 351,027 shares will be added to the shares reserved under the 2014 Plan when the 2014 Plan becomes effective;

- n 550,000 shares of common stock reserved for future issuance under the ESPP, which will become effective upon the execution and delivery of the underwriting agreement for this offering; and
  
- n 355,392 shares of common stock issuable upon the exercise of outstanding warrants as of September 30, 2014, at an exercise price of \$0.0017 per share.

We may choose to raise additional capital through the sale of equity or convertible debt securities due to market conditions or strategic considerations even if we believe we have sufficient funds for our current or future operating plans. To the extent we issue additional shares of common stock or other equity or convertible debt securities in the future, there will be further dilution to investors participating in this offering.



**Table of Contents****SELECTED FINANCIAL DATA**

This section should be read together with our financial statements and related notes and Management's Discussion and Analysis of Financial Condition and Results of Operations appearing elsewhere in this prospectus. We derived the selected statement of operations data for the years ended December 31, 2012 and 2013 and the selected balance sheet data as of December 31, 2012 and 2013 from our audited financial statements and related notes appearing elsewhere in this prospectus. We derived the selected statement of operations data for the nine months ended September 30, 2013 and 2014 and the selected balance sheet data as of September 30, 2014 from our unaudited financial statements and related notes appearing elsewhere in this prospectus. The selected financial data in this section are not intended to replace our financial statements and the related notes. The unaudited financial data, in management's opinion, have been prepared on the same basis as the audited financial statements and related notes included elsewhere in this prospectus, and include all adjustments, consisting only of normal recurring adjustments, that management considers necessary for a fair presentation of the information for the periods presented. Our historical results are not necessarily indicative of the results that may be expected in the future and results from our interim period may not necessarily be indicative of the results of the entire year.

	YEAR ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,	
	2012	2013	2013	2014
<b>(in thousands, except share and per share data)</b>			<b>(unaudited)</b>	<b>(unaudited)</b>
<b>Statement of Operations Data:</b>				
Grant revenue	\$ 1,470	\$ 1,941	\$ 1,122	\$ 1,766
Operating expenses:				
Research and development	5,796	7,050	4,564	7,078
General and administrative	1,943	2,813	1,997	3,135
Total operating expenses	7,739	9,863	6,561	10,213
Loss from operations	(6,269)	(7,922)	(5,439)	(8,447)
Other income (expense):				
Interest income	7	4	2	15
Interest expense	(1)	(51)	(38)	(38)
Change in value of warrant				(1,197)
Total other income (expense)	6	(47)	(36)	(1,220)
Net loss	\$ (6,263)	\$ (7,969)	\$ (5,475)	\$ (9,667)
Preferred stock dividend	(757)	(1,094)	(695)	(1,432)

Edgar Filing: BELLICUM PHARMACEUTICALS, INC - Form 424B4

Net loss available to common stockholders	\$ (7,020)	\$ (9,063)	\$ (6,170)	\$ (11,099)
Net loss per share, basic and diluted	\$ (4.26)	\$ (5.25)	\$ (3.58)	\$ (5.45)
Weighted-average shares outstanding, basic and diluted <sup>(1)</sup>	1,648,198	1,725,992	1,725,992	2,036,025
Pro forma net loss (unaudited)		\$ (7,969)		\$ (9,667)
Pro forma net loss per share, basic and diluted (unaudited) <sup>(2)</sup>		\$ (1.32)		\$ (0.98)
Pro forma weighted-average shares outstanding, basic and diluted (unaudited) <sup>(2)</sup>		6,051,619		9,827,767

- <sup>(1)</sup> See Note 2 to our financial statements appearing elsewhere in this prospectus for an explanation of the method used to calculate the basic and diluted net loss per common share and the number of shares used in the computation of the per share amounts.

**Table of Contents**

- (2) The calculations for the unaudited pro forma net loss per common share, basic and diluted, assume (1) the conversion of all our outstanding shares of convertible preferred stock as of September 30, 2014 into an aggregate of 12,224,819 shares of our common stock, (2) the net exercise of outstanding warrants to purchase common stock (which will expire upon the closing of this offering if not exercised) into 114,468 shares of our common stock, and (3) the issuance of 6,559,598 shares of Series C convertible preferred stock upon the exercise of warrants, and the conversion of such shares into 3,858,549 shares of common stock in connection with the closing of this offering.

(in thousands)	AS OF DECEMBER 31,		AS OF
	2012	2013	SEPTEMBER 30, 2014 (unaudited)
<b>Balance Sheet Data:</b>			
Cash and cash equivalents	\$ 1,632	\$ 11,168	\$ 61,932
Working capital	256	9,963	49,849
Total assets	5,186	14,942	66,331
Convertible preferred stock	21,658	39,926	90,753
Accumulated deficit	(21,010)	(28,979)	(38,646)
Total stockholders' deficit	(19,473)	(28,152)	(38,794)

**Table of Contents****MANAGEMENT'S DISCUSSION AND ANALYSIS OF FINANCIAL CONDITION AND RESULTS OF OPERATIONS**

*The following discussion and analysis should be read in conjunction with Selected Financial Data and our financial statements and related notes included elsewhere in this prospectus. This discussion and analysis and other parts of this prospectus contain forward-looking statements based upon current beliefs, plans and expectations that involve risks, uncertainties and assumptions, such as statements regarding our plans, objectives, expectations, intentions and projections. Our actual results and the timing of selected events could differ materially from those anticipated in these forward-looking statements as a result of several factors, including those set forth under Risk Factors and elsewhere in this prospectus. You should carefully read the Risk Factors section of this prospectus to gain an understanding of the important factors that could cause actual results to differ materially from our forward-looking statements. Please also see the section entitled Special Note Regarding Forward-Looking Statements.*

**Overview**

We are a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. We are using our proprietary Chemical Induction of Dimerization, or CID, technology platform to engineer and then control components of the immune system in real time. By incorporating our CID platform, our product candidates may offer better safety and efficacy outcomes than are seen with current cellular immunotherapies.

We are developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including HSCT, CAR T cell therapy, and dendritic cell vaccines. By incorporating our novel switch technologies, we are developing product candidates with the potential to elicit positive clinical outcomes and ultimately change the treatment paradigm in various areas of cellular immunotherapy. Our clinical product candidates, each of which is a combination product of genetically modified immune cells and rimiducid, are described below.

- n **BPX-501.** We are developing a CaspaCIDE product candidate, BPX-501, as an adjunct T-cell therapy administered after allogeneic HSCT, using donor stem cells. BPX-501 is designed to decrease the risk of including T cells with the transplant by enabling the elimination of donor T cells through the triggering of the CaspaCIDE safety switch upon emergence of GvHD. BPX-501 is currently being evaluated in multiple Phase 1/2 clinical trials in the United States and Europe, with the first top-line data expected in the second half of 2015.
- n **BPX-201.** We are developing a DeCIDE product candidate, BPX-201, as a dendritic cell cancer vaccine made from the patient's own white blood cells, designed to treat mCRPC. It targets the prostate specific membrane antigen, or PSMA, and uses our DeCIDE activation switch technology. BPX-201 is currently being evaluated in an 18-patient Phase 1 clinical trial for mCRPC. We are evaluating opportunities for BPX-201 in combination with other cancer immunotherapies, such as checkpoint inhibitors.

In addition, our preclinical product candidates are designed to overcome the current limitations of CAR-T and TCR therapies and include the following:

- n **BPX-401.** We are developing a CIDE CAR product candidate, BPX-401, as a next-generation CAR T cell therapy for hematological cancers that express the CD19 antigen.
  
- n **BPX-601.** We are developing a GoCAR-T product candidate, BPX-601, for solid tumors overexpressing PSCA, such as some prostate, pancreatic, bladder, esophageal and gastric cancers.
  
- n **BPX-701.** We are developing a CaspaCIDE TCR product candidate, BPX-701, in collaboration with Leiden University Medical Center, initially for the treatment of PRAME-expressing melanoma, sarcomas and neuroblastoma.

We expect to file INDs for BPX-701 in the second half of 2015 and for BPX-401 and BPX-601 in 2016. Our IND-enabling activities for each of these preclinical product candidates include manufacturing key components and developing a robust process to produce cell products that comply with regulations of the FDA, and other regulatory agencies. We have developed an efficient and scalable process to manufacture genetically modified T cells of high

## **Table of Contents**

quality and purity. This process is being implemented by our third-party contract manufacturers to produce BPX-501 for our clinical trials. We expect to leverage our resources, capabilities and expertise for the manufacture of our CAR-T and TCR product candidates.

## **Recent Developments**

To enable further development of our proprietary technology and product candidates, we completed a private placement of \$55 million of Series C convertible preferred stock and warrants to purchase Series C convertible preferred stock in August 2014. Investors in the transaction included, among others, Baker Brothers, RA Capital Management, LLC, Perceptive Advisors, LLC, Jennison Associates LLC (on behalf of certain clients), Sabby Capital, LLC, Ridgeback Capital Management, venBio Select, Redmile Group, LLC and AJU IB Investment, as well as our then current investors, including AVG Ventures and Remeditex Ventures.

Certain aspects of our platform technology are inlicensed from ARIAD. In October 2014, we amended our license agreement with ARIAD, pursuant to which we agreed to pay ARIAD \$50 million in three tranches payments, including an initial payment of \$15 million in connection with the execution of the amendment. In exchange, ARIAD gave us a fully paid-up license to its cell-signaling technology and agreed to return of all of the 677,463 shares of our common stock currently held by ARIAD at the time of the second tranche payment. The scope of the license and the field of use were also expanded as part of the amendment. The amended agreement gives us a worldwide exclusive license to ARIAD's cell-signaling technology for broad use in human cell therapies for all diseases on a royalty- and milestone-free basis.

## **Financial Operations Overview**

### ***Revenue***

To date, we have only recognized revenue from government grants and we have not generated any product revenue. We receive funds from the Cancer Prevention and Research Institute of Texas, or CPRIT, and the National Institute of Health, or NIH, which are awarded based on the progress of the program being funded. Grant money is not received until expenses for the program are incurred. We accrue the revenue based on the costs incurred for the programs associated with the grant.

During 2011, we entered into a grant agreement with CPRIT for approximately \$5.7 million covering a three year period from July 1, 2011 through June 30, 2014. The grant allowed us to receive funds in advance of costs and allowable expenses being incurred. On a quarterly basis, we are required to submit a financial reporting package outlining the nature and extent of reimbursed costs under the grant. At the end of each period, any excess funds received in advance, or paid prior to reimbursement, result in a deferred liability or grant receivable.

In the future, we may generate revenue from a combination of product sales, government or other third-party grants, marketing and distribution arrangements and other collaborations, strategic alliances and licensing arrangements or a combination of these approaches. We expect that any revenue we generate will fluctuate from quarter to quarter as a result of the timing and amount of license fees, milestone and other payments, and the amount and timing of payments that we receive upon the sale of our products, to the extent any are successfully commercialized. If we fail to complete the development of our product candidates in a timely manner or obtain regulatory approval of them, our ability to generate future revenue, and our results of operations and financial position, would be materially adversely affected.

Funds from the NIH are granted based on the progress of the program being funded. Grant money is not received until expenses for the program are incurred. We accrue the revenue based on the costs incurred for the programs associated

with the grant.

***Research and Development Expenses***

To date, our research and development expenses have related primarily to the development of our CID platform and the identification and development of our product candidates. Research and development expenses consist of expenses incurred in performing research and development activities, including compensation and benefits for research and development employees and consultants, facilities expenses, overhead expenses, cost of laboratory supplies, manufacturing expenses, fees paid to third parties and other outside expenses.

**Table of Contents**

Research and development costs are expensed as incurred. Clinical trial and other development costs incurred by third parties are expensed as the contracted work is performed. We accrue for costs incurred as the services are being provided by monitoring the status of the clinical trial or project and the invoices received from our external service providers. We adjust our accrual as actual costs become known. Where contingent milestone payments are due to third parties under research and development arrangements, the milestone payment obligations are expensed when the milestone results are achieved.

Research and development activities are central to our business model. Product candidates in later stages of clinical development generally have higher development costs than those in earlier stages of clinical development, primarily due to the increased size and duration of later-stage clinical trials. We expect our research and development expenses to increase over the next several years as we seek to conduct our ongoing and planned clinical trials for BPX-501, BPX-201, BPX-401, BPX-601 and BPX-701 and as we selectively develop additional product candidates. However, it is difficult to determine with certainty the duration and completion costs of our current or future preclinical programs and clinical trials of our product candidates.

The duration, costs and timing of clinical trials and development of our product candidates will depend on a variety of factors that include, but are not limited to, the following:

- n per patient clinical trial costs;
- n the number of patients that participate in the clinical trials;
- n the number of sites included in the clinical trials;
- n the process of collection, differentiation, selection and expansion of immune cells for our cellular immuno-therapies;
- n the countries in which the clinical trials are conducted;
- n the length of time required to enroll eligible patients;
- n the number of doses that patients receive;
- n the drop-out or discontinuation rates of patients;
- n potential additional safety monitoring or other studies requested by regulatory agencies;



n the duration of patient follow-up; and

n the efficacy and safety profile of the product candidates.

The following table indicates our research and development expense by project/category for the periods indicated:

	YEARS ENDED DECEMBER 31,		NINE MONTHS ENDED SEPTEMBER 30,		TOTAL PROJECT INCEPTION THROUGH SEPTEMBER 30,
	2012	2013	2013	2014	2014
BPX-101					6,478,453
BPX-201	1,943,433	1,563,324	932,315	1,516,158	6,096,499
BPX-501	2,239,482	3,061,500	2,186,924	4,029,508	10,017,316
General	1,613,318	2,424,596	1,444,537	1,532,272	6,935,928
<b>Total</b>	<b>5,796,233</b>	<b>7,049,420</b>	<b>4,563,776</b>	<b>7,077,938</b>	<b>29,528,196</b>

In addition, the probability of success for each product candidate will depend on numerous factors, including competition, manufacturing capability and commercial viability. We will determine which programs to pursue and how much to fund each program in response to the scientific and clinical success of each product candidate, as well as an assessment of each product candidate's commercial potential.

#### *General and Administrative Expenses*

General and administrative expenses consist primarily of salaries and other related costs, including stock-based compensation, for personnel in executive, finance, accounting, business development and human resources functions. Other significant costs include facility costs not otherwise included in research and development expenses, legal fees relating to patent and corporate matters and fees for accounting and consulting services.

**Table of Contents**

We anticipate that our general and administrative expenses will increase in the future to support our continued research and development activities, potential commercialization of our product candidates and the increased costs of operating as a public company. These increases will likely include increased costs related to the hiring of additional personnel and fees to outside consultants, lawyers and accountants, among other expenses. Additionally, we anticipate increased costs associated with being a public company, including expenses related to services associated with maintaining compliance with NASDAQ listing rules and SEC requirements, insurance and investor relations costs.

**Other Income (Expense)**

Other income (expense), net consists of interest income, interest expense and the change in the value of the warrant liability.

**Results of Operations****Comparison of Nine Months Ended September 30, 2013 and 2014**

The following table sets forth our results of operations for the nine months ended September 30, 2013 and 2014:

(in thousands)	NINE MONTHS ENDED SEPTEMBER 30,		CHANGE \$
	2013 (unaudited)	2014 (unaudited)	
Grant revenues	\$ 1,122	\$ 1,766	\$ 644
Operating expenses:			
Research and development	4,564	7,078	2,514
General and administrative	1,997	3,135	1,138
Total operating expenses	6,561	10,213	3,652
Loss from operations	(5,439)	(8,447)	(3,008)
Other income (expense):			
Interest income	2	15	13
Interest expense	(38)	(38)	
Change in value of warrant		(1,197)	(1,197)
Total other income (expense)	(36)	(1,220)	(1,184)
Net loss	\$ (5,475)	\$ (9,667)	\$ (4,192)

*Grant Revenues*

Grant revenues were \$1.1 million and \$1.8 million for the nine months ended September 30, 2013 and 2014, respectively. The increase in grant revenues is primarily due to additional costs associated with the grant from CPRIT due to patient enrollment which began in August 2013. The increase is also due to the addition of the grant from the NIH. Funds were awarded in April 2013 and April 2014.

*Research and Development Expenses*

Research and development expenses were \$4.6 million and \$7.1 million for the nine months ended September 30, 2013 and 2014, respectively. The increase in research and development expenses is primarily due to the increase in manufacturing of \$1.1 million and \$0.3 million and clinical expenses of \$0.2 million and \$0.2 million as a result of increased patient enrollment in our clinical trials for BPX-501 and BPX-201, respectively.

*General and Administrative Expenses*

General and administrative expenses were \$2.0 million and \$3.1 million for the nine months ended September 30, 2013 and 2014, respectively. The increase in general and administrative expenses during this period of \$1.1 million was due to our overall growth, including an increase in personnel, legal and accounting expenses, costs related to facilities, travel and entertainment expenses and depreciation expense related to equipment.

*Other Income (Expense)*

Other expense was \$36,000 and \$1.2 million for the nine months ended September 30, 2013 and 2014, respectively. The increase in other income (expense) is primarily due to the change in value of the warrant liability.

**Table of Contents*****Comparison of the Years Ended December 31, 2012 and 2013***

The following table sets forth our results of operations for the years ended December 31, 2012 and 2013:

<b>(in thousands)</b>	<b>YEAR ENDED DECEMBER 31,</b>		<b>CHANGE</b>
	<b>2012</b>	<b>2013</b>	<b>\$</b>
Grant revenue	\$ 1,470	\$ 1,941	\$ 471
Operating expenses:			
Research and development	5,796	7,050	1,254
General and administrative	1,943	2,813	870
<b>Total operating expenses</b>	<b>7,739</b>	<b>9,863</b>	<b>2,124</b>
Loss from operations	(6,269)	(7,922)	(1,653)
Other income (expense):			
Interest income	7	4	(3)
Interest expense	(1)	(51)	(50)
<b>Total other income (expense)</b>	<b>6</b>	<b>(47)</b>	<b>(53)</b>
<b>Net loss</b>	<b>\$ (6,263)</b>	<b>\$ (7,969)</b>	<b>\$ (1,706)</b>

***Grant Revenues***

Grants revenues were \$1.5 million and \$1.9 million for the years ended December 31, 2012 and 2013, respectively. The increase in grant revenues is due to the addition of the grant from the NIH, received in April 2013.

***Research and Development Expenses***

Research and development expenses were \$5.8 million and \$7.1 million for the years ended December 31, 2012 and 2013, respectively. The increase in research and development expenses is primarily due to the increase in personnel and clinical expenses as a result of increased patient enrollment in the clinical trials of BPX-501 and BPX-201, as well as an increase in total patient costs of \$0.5 million, which includes \$0.2 million of clinical site costs and \$0.2 million of specific patient treatment costs. BPX-501 clinical and manufacturing costs increased by \$0.3 million and \$0.4 million, respectively, for the year ended December 31, 2013 when compared to the previous year. BPX-201 clinical costs increased by \$0.2 million and manufacturing costs decreased by \$0.6 million, for the year ended December 31, 2013 when compared to the previous year.

*General and Administrative Expenses*

General and administrative expenses were \$1.9 million and \$2.8 million for the years ended December 31, 2012 and 2013, respectively. The increase in expenses is due to our overall growth, including an increase in personnel, legal and accounting expenses, costs related to facilities, travel and entertainment expenses, and depreciation expense related to equipment.

*Other Income (Expense)*

Other income was \$6,000 and other expense was \$47,000 for the years ended December 31, 2012 and 2013, respectively. The decrease is primarily due to interest expense incurred on the increase in the outstanding amount under the line of credit described below.

*Liquidity and Capital Resources*

*Sources of Liquidity*

We are a clinical stage biopharmaceutical company with a limited operating history. To date, we have financed our operations primarily through private placements of convertible debt and preferred stock and receipt of grants to fund our research and development programs. We have not generated any revenue from the sale of any products. As of December 31, 2013 and September 30, 2014, we had available cash and cash equivalents of \$11.2 million and \$61.9 million, respectively. Our cash and cash equivalents are held in cash and money market accounts. Cash in excess of immediate requirements is invested with a view toward liquidity and capital preservation, and we seek to minimize the potential effects of concentration and degrees of risk.

## **Table of Contents**

We are party to a line of credit which was executed in 2012 for \$1 million. The annual interest rate is equal to the prime rate plus 2.75%. We take advances under this line of credit to fund equipment purchases and other capital expenditures. During 2013, we were advanced \$550,223 under the line of credit. Principal payments on the \$1 million line of credit began in July 2013 and will be paid over 30 months. During 2014, the line of credit was amended to include a credit extension up to \$500,000. Interest accrues at a rate of prime plus 2.75% from the date of each advance. Any advances that are outstanding on the credit extension are payable in 24 equal monthly installments of principal, plus all accrued interest, beginning on April 1, 2015. During the nine months ended September 30, 2014, \$81,548 was advanced under the credit extension.

On February 12, 2013, we received \$3.5 million of cash proceeds through the issuance of promissory notes, bearing interest at 0.21% per annum from February 12, 2013 through July 31, 2013. On July 31, 2013, in connection with the issuance of Series B convertible preferred stock, we repaid the notes with 757,497 shares of Series B convertible preferred stock at a conversion price of \$4.625 per share. The converted balances consisted of \$3.5 million of principal and \$3,426 of outstanding interest payable.

During the nine months ended September 30, 2014, we sold 1,582,705 shares of our Series B convertible preferred stock for net proceeds of \$7.3 million, and received \$0.2 million pursuant to the exercise of warrants.

In August 2014, we completed a private placement of 10,091,743 shares of Series C convertible preferred stock and received gross proceeds of \$55 million, resulting in net proceeds of \$51.5 million. In connection with the Series C convertible preferred stock financing, we also issued warrants to purchase up to 6,559,598 shares of Series C convertible preferred stock at an exercise price of \$6.00 per share. The warrants are exercisable for five years and will terminate upon the earlier of a merger or sale of the Company or upon the date immediately following the date of effectiveness of the registration statement of which this prospectus forms a part. If this offering closes prior to March 31, 2015, we would expect substantially all of the warrants to be exercised for cash, which would provide additional proceeds of up to approximately \$39 million.

## ***Funding Requirements***

Our primary uses of capital are, and we expect will continue to be, compensation and related expenses, third-party clinical research and development services, laboratory and related supplies, clinical costs, legal and other regulatory expenses and general overhead costs. Specifically, we expect to use capital to repay the ARIAD note and expand our manufacturing capabilities.

The successful development of any product candidate is highly uncertain. As such, at this time, we cannot reasonably estimate or know the nature, timing and costs of the efforts that will be necessary to complete the development of BPX-501 or our other current and future product candidates. We are also unable to predict when, if ever, material net cash inflows will commence from the sale of product candidates. This is due to the numerous risks and uncertainties associated with developing medical treatments, including the uncertainty of:

- n successful enrollment in, and completion of, clinical trials;
  
- n receipt of marketing approvals from applicable regulatory authorities;

- n making arrangements with third-party manufacturers;
  
- n obtaining and maintaining patent and trade secret protection and regulatory exclusivity; and
  
- n launching commercial sales of our products, if and when approved, whether alone or in collaboration with others.

A change in the outcome of any of these variables with respect to the development of any of our product candidates would significantly change the costs and timing associated with the development of that product candidate.

Because all of our product candidates are in the early stages of clinical and preclinical development and the outcome of these efforts is uncertain, we cannot estimate the actual amounts necessary to successfully complete the development and commercialization of product candidates or whether, or when, we may achieve profitability. Until such time, if ever, as we can generate substantial product revenue, we expect to finance our cash needs through a combination of equity or debt financings and collaboration arrangements.

## **Table of Contents**

In addition to the amounts necessary to fund development and commercialization of our product candidates, we will also need funds to pay our obligations under the ARIAD note. We anticipate using our cash and cash equivalents to pay such obligations, including \$20 million which we expect to pay upon closing of this offering and \$15 million which we intend to pay within nine months of the closing of this offering.

We plan to continue to fund our operations and capital funding needs through equity and/or debt financing. We may also consider new collaborations or selectively partnering our technology. To the extent that we raise additional capital through the sale of equity or convertible debt securities, the ownership interests of our stockholders will be diluted, and the terms may include liquidation or other preferences that adversely affect the rights of our existing stockholders. The incurrence of indebtedness would result in increased fixed payment obligations and could involve certain restrictive covenants, such as limitations on our ability to incur additional debt, limitations on our ability to acquire or license intellectual property rights and other operating restrictions that could adversely impact our ability to conduct our business. If we raise additional funds through strategic partnerships and alliances and licensing arrangements with third parties, we may have to relinquish valuable rights to our technologies or product candidates, or grant licenses on terms unfavorable to us. Any of these actions could harm our business, results of operations and future prospects.

## ***Outlook***

Based on our research and development plans and our timing expectations related to the progress of our programs, we expect that the net proceeds from this offering, together with our existing cash and cash equivalents as of September 30, 2014 and the net proceeds from the issuance and sale of our Series C convertible preferred stock in August 2014, will enable us to fund our operating expenses and capital expenditure requirements into 2016. We have based this estimate on assumptions that may prove to be wrong, and we could utilize our available capital resources sooner than we currently expect. Furthermore, our operating plan may change, and we may need additional funds to meet operational needs and capital requirements for product development and commercialization sooner than planned. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates and the extent to which we may enter into additional collaborations with third parties to participate in their development and commercialization, we are unable to estimate the amounts of increased capital outlays and operating expenditures associated with our current and anticipated clinical trials. Our future funding requirements will depend on many factors, as we:

- n initiate or continue clinical trials of BPX-501 and any other product candidates;
- n continue the research and development of our product candidates;
- n seek to discover additional product candidates;
- n seek regulatory approvals for our product candidates that successfully complete clinical trials;
- n establish a sales, marketing and distribution infrastructure and scale-up manufacturing capabilities to commercialize products which receive regulatory approval;



n enhance operational, financial and information management systems and hire additional personnel, including personnel to support development of our product candidates and, if a product candidate is approved, our commercialization efforts; and

n incur additional costs associated with becoming a public company.

### **Cash Flows**

The following table set forth a summary of our cash flows for the nine months ended September 30, 2014 and 2013, respectively:

	FOR THE YEAR ENDED DECEMBER 31		FOR THE NINE MONTHS ENDED	
	2012	2013	2013	2014
(in thousands)			(unaudited)	(unaudited)
Net cash used in operating activities	(7,744)	(7,613)	\$ (5,567)	\$ (7,158)
Net cash used in investing activities	(2,047)	(366)	(283)	(401)
Net cash provided by financing activities	3,516	17,515	10,144	58,323
Net cash (outflow) inflow	(6,275)	9,536	\$ 4,294	\$ 50,764

**Table of Contents***Operating Activities*

Net cash used in operating activities was \$7.2 million for the nine months ended September 30, 2014, which was derived from a net loss of \$9.7 million, in addition to the following primary components: an increase in grant receivables of \$0.5 million, an increase in accounts payable of \$1.1 million, a decrease in accrued payroll of \$0.5 million driven primarily by payment of accrued bonuses, and stock-based compensation of \$0.2 million.

Net cash used in operating activities was \$5.6 million for the nine months ended September 30, 2013, which was derived from a net loss of \$5.5 million, in addition to the following primary components: a decrease in prepaid expenses and other current assets of \$0.6 million, a decrease in accounts payable, accrued payroll, and accrued liabilities of \$0.4 million, a decrease in deferred revenue-grants of \$1.0 million, and stock-based compensation of \$0.3 million.

*Investing Activities*

Net cash used in investing activities for the nine months ended September 30, 2014 was \$0.4 million, which was derived solely from the purchase of property and equipment. Net cash used in investing activities for the nine months ended September 30, 2013 was \$0.3 million, which was derived solely from the purchase of property and equipment.

*Financing Activities*

Net cash provided by financing activities for the nine months ended September 30, 2014 was \$58.3 million, which was derived from proceeds from the issuance of convertible preferred stock of \$62.3 million offset by \$3.5 million of issuance costs, proceeds from issuance of common stock of \$0.2 million, and proceeds from the line of credit of \$82,000, which were offset by payments on the line of credit of \$0.3 million. Net cash provided by financing activities for the nine months ended September 30, 2013 was \$10.1 million, which was derived from proceeds from issuance of preferred stock of \$6.2 million, proceeds from notes payable of \$3.5 million and proceeds from the line of credit of \$0.6 million, offset by payments on the line of credit of \$0.1 million.

**Contractual Obligations**

Our contractual obligations as of December 31, 2013 were as follows:

	<b>PAYMENTS DUE BY PERIOD</b>			
	<b>TOTAL</b>	<b>LESS THAN 1 YEAR</b>	<b>1 - 3 YEARS</b>	<b>MORE THAN 5 YEARS</b>
Long term debt	\$ 800,000	\$ 400,000	\$ 400,000	\$
Operating lease agreements	1,803,170	601,057	1,202,113	
Contract manufacturing agreements	2,967,600	789,600	2,178,000	
Facility lease agreement	240,000	192,000	48,000	

License Agreements	200,000	50,000	150,000		
Total contractual obligation	\$ 6,010,770	\$ 2,032,657	\$ 3,978,113	\$	\$

### Critical Accounting Policies and Significant Estimates

Our financial statements are prepared in accordance with U.S. generally accepted accounting principles, or GAAP. The preparation of these financial statements requires us to make estimates and assumptions that affect the reported amounts of assets, liabilities, revenues, costs and expenses and related disclosures. We base our estimates on historical experience and on various other assumptions that we believe to be reasonable under the circumstances. In many instances, we could have reasonably used different accounting estimates, and in other instances changes in the accounting estimates are reasonably likely to occur from period to period. Accordingly, actual results could differ significantly from management's estimates. To the extent that there are material differences between these estimates and actual results, our future financial statement presentation, financial condition, results of operations and cash flows will be affected. While our significant accounting policies are described in the Notes to our financial statements, we believe that the accounting policies discussed below are critical to understanding our historical and future performance, as these policies related to the more significant areas involving management's judgments and estimates.

## **Table of Contents**

**Revenue Recognition** We have not yet generated any revenue from product sales. Our sole source of revenue is grant revenue related to a \$5.7 million research grant received from CPRIT, covering a three-year period from July 1, 2011 through June 30, 2014 and a \$0.7 million research grant from NIH. Grant payments received prior to our performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred.

**Licenses and Patents** Licenses and patent costs are expensed as incurred. Costs related to the license of patents from third parties and internally developed patents are classified as research and development expenses. Legal costs related to patent applications and maintenance are classified as general and administrative expenses.

**Research and Development** Research and development expenses include salaries, related payroll expenses, consulting fees, laboratory costs, manufacturing costs, and clinical trial expenses. All costs for research and development are expensed as incurred.

**Contract Manufacturing Services** Contract manufacturing services are expensed as incurred. Prepaid costs are capitalized and amortized as services are performed.

**Stock-Based Compensation** Stock-based compensation cost is measured at the date of grant based on the estimated fair value of the award, net of estimated forfeitures. We estimate the grant date fair value and the resulting stock-based compensation expense using the Black-Scholes option pricing model. The grant date fair value of a stock-based award is recognized as an expense over the requisite service period of the award on a straight-line basis.

We account for stock-based compensation arrangements with non-employees using a fair value approach. The fair value of these options is measured using the Black-Scholes option pricing model reflecting an expected life that is assumed to be the remaining contractual life of the option. The compensation costs of these arrangements are subject to re-measurement over the vesting terms as earned.

We determine the fair value of each grant of stock options using the estimated fair value of our common stock and the assumptions set forth below. Each of these inputs is subjective and generally requires significant judgment.

Our board of directors intends all options granted to be exercisable at a price per share not less than the per share fair value of our common stock underlying those options on the date of grant. The estimated fair value of our common stock was determined at each valuation date in accordance with the guidelines outlined in the American Institute of Certified Public Accountants Practice Aid, Valuation of Privately-Held-Company Equity Securities Issued as Compensation. Our board of directors, with the assistance of management, developed these valuations using significant judgment and taking into account numerous factors, including developments at our company, market conditions and independent third-party valuations as of December 31, 2011, 2012 and 2013, July 31, 2014, and October 15, 2014.

For all option grant dates through September 30, 2014, the enterprise value was determined based on a Probability Weighted Expected Return Method, or PWERM, Option Pricing Method, or the OPM backsolve method. The allocation of these enterprise values to each part of our capital structure, including our common stock, was done based on OPM. OPM treats the rights of the holders of preferred and common shares as equivalent to call options on any value of the enterprise above certain break points of value based upon the liquidation preferences of the holders of preferred shares, as well as their rights to participation and conversion. Thus, the estimated value of the common stock can be determined by estimating the value of its portion of each of these call option rights. The OPM backsolve method derives the implied equity value of a company from a recent transaction involving the company's own securities issued on an arms-length basis. The Discounted Cash Flow method estimates value based on the expectation

of future net cash flows, which are then discounted back to the present using a rate of return derived from alternative companies of similar type and risk profile. Under the PWERM the value is estimated based upon analysis of future values for the enterprise under varying scenarios, probabilities are ascribed to these scenarios based on expected future outcomes. Following the closing of this offering, the fair value of our common stock will be determined based on the closing price of our common stock on the NASDAQ Global Market.

***Income Taxes*** Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement

## **Table of Contents**

carrying amounts of existing assets and liabilities and their respective tax bases. This method also requires the recognition of future tax benefits such as net operating loss and tax credit carry forwards, to the extent that realization of such benefits is more likely than not. A valuation allowance is recorded when the realization of future tax benefits is uncertain. We record a valuation allowance for the full amount of deferred tax assets, which would otherwise be recorded for tax benefits relating to the operating loss and tax credit carryforwards, as realization of such deferred tax assets cannot be determined to be more likely than not.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date.

We account for uncertain tax positions in accordance with the provisions of ASC 740, Income Taxes. When uncertain tax positions exist, we recognize the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2013 and 2012, we had no uncertain tax positions and no interest or penalties have been charged to us for the years ended December 31, 2013 and 2012. If incurred, we will classify any interest and penalties as a component of interest expense and operating expense, respectively. We are subject to routine audits by taxing jurisdictions; however, there are currently no audits for any tax periods in progress. The tax years 2004 through 2013 remain open to examination by the U.S. Internal Revenue Service.

## **Recently Issued Accounting Pronouncements**

In June 2014, the Financial Accounting Standards Board issued Accounting Standards Update No. 2014-10 ( ASU No. 2014-10 ), which eliminated the definition of a Development Stage Entity and the related reporting requirements. ASU No. 2014-10 is effective for annual reporting periods beginning after December 15, 2014, with early adoption allowed. We chose to adopt ASU No. 2014-10 early, effective in its financial statements for the nine months ended September 30, 2014.

## **JOBS Act**

In April 2012, the JOBS Act was enacted. Section 107(b) of the JOBS Act provides that an emerging growth company may take advantage of an extended transition period for complying with new or revised accounting standards. Thus, an emerging growth company can delay the adoption of certain accounting standards until those standards would otherwise apply to private companies. We have irrevocably elected not to avail ourselves of this extended transition period, and, as a result, we will adopt new or revised accounting standards on the relevant dates on which adoption of such standards is required for other public companies.

## **Quantitative and Qualitative Disclosures about Market Risk**

The primary objective of our investment activities is to preserve our capital to fund our operations. We also seek to maximize income from our investments without assuming significant risk. To achieve our objectives, we maintain a portfolio of cash equivalents and investments in a variety of securities of high credit quality. As of September 30, 2014, we had cash and cash equivalents of \$61.9 million consisting of cash and money market accounts in highly rated financial institutions in the United States.

A portion of our investments may be subject to interest rate risk and could fall in value if market interest rates increase. However, because our investments are primarily short-term in duration, we believe that our exposure to interest rate risk is not significant and a 1% movement in market interest rates would not have a significant impact on the total value of our portfolio. We actively monitor changes in interest rates.

#### **Changes in and Disagreements with Accountants on Accounting and Financial Disclosure**

In connection with this offering, Ernst & Young LLP became our independent registered public accounting firm effective as of August 25, 2014, and PMB Helin Donovan, LLP was dismissed as our independent registered public accounting firm effective as of July 31, 2014. The decision to appoint Ernst & Young LLP to re-audit our financial

**Table of Contents**

statements for the years ended December 31, 2013 and December 31, 2012, and dismiss PMB Helin Donovan, LLP was approved by our board of directors on July 31, 2014.

The report of PMB Helin Donovan, LLP on our financial statements for the years ended December 31, 2013 and December 31, 2012 did not contain an adverse opinion or a disclaimer of opinion and was not qualified or modified as to uncertainty, audit scope or accounting principles.

In connection with the audit of our financial statements for the years ended December 31, 2013 and December 31, 2012 and through PMB Helin Donovan, LLP's dismissal, there were no disagreements with PMB Helin Donovan, LLP on any matters of accounting principles or practices, financial statement disclosures or auditing scope or procedures, which if not resolved to PMB Helin Donovan, LLP's satisfaction would have caused PMB Helin Donovan, LLP to make reference to the matter in their report.

There have been no reportable events as set forth in Item 304(a)(1)(v) of Regulation S-K in connection with our audited financial statements for the years ended December 31, 2013 and December 31, 2012 and PMB Helin Donovan LLP's dismissal.

We requested that PMB Helin Donovan, LLP furnish us with a letter addressed to the SEC stating whether it agrees with the above statements. A copy of the letter, dated November 18, 2014, is filed as an exhibit to the registration statement of which this prospectus forms a part.



---

**Table of Contents****BUSINESS****Overview**

We are a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. Cellular immunotherapy has the potential to transform medicine by harnessing immune cells, principally T cells, to attack and eliminate harmful diseased cells in the body. Unlike traditional small molecule and biologic therapies which are predictably metabolized and eliminated from the body, cellular immunotherapies are unpredictable and uncontrollable. We are using our proprietary Chemical Induction of Dimerization, or CID, technology platform to engineer and then control components of the immune system in real time. By incorporating our CID platform, our product candidates may offer better safety and efficacy outcomes than are seen with current cellular immunotherapies.

We are developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including hematopoietic stem cell transplantation, or HSCT, CAR T cell therapy, and dendritic cell vaccines. HSCT, also known as bone marrow transplantation, has for decades been curative for many patients with hematological cancers or orphan inherited blood disorders. However, application of HSCT is limited by graft-versus-host-disease, or GvHD, a condition in which the transplanted immune cells recognize the host cells as foreign and attack them. Since the transplanted cells can persist indefinitely, GvHD does not resolve by itself and is a major cause of transplant-related morbidity and mortality. CAR T cell therapy is an innovative approach in which a patient's T cells are genetically modified to carry chimeric antigen receptors, or CARs, which redirect the T cells against cancer cells. While high objective response rates have been reported in some hematological malignancies, serious and sometimes fatal toxicities have arisen in patients treated with CAR T cell therapies. These toxicities include instances in which the CAR T cells have caused high levels of cytokines due to over-activation, referred to as cytokine release syndrome, frequent transient neurologic toxicities and cases in which they have attacked healthy organs instead of the targeted tumor, leading to death. In solid tumors, where the behavior of CAR T cells is particularly unpredictable and results have been inconsistent, researchers are developing enhanced CAR T cell approaches called armored CARs that raise even greater safety concerns. Lastly, despite the integral role that dendritic cells, specialized cells that are key regulators of the immune system that process and present antigens on the cell surface to T cells in order to activate the T cells, play in the immune system, they are difficult to activate appropriately and as a result their use has delivered only modest therapeutic benefit.

Our proprietary CID platform is designed to address these challenges. Events inside a cell are controlled by cascades of specialized signaling proteins. CID consists of molecular switches, modified forms of these signaling proteins, which are triggered inside the patient by infusion of a small molecule, rimiducid (AP1903), instead of by natural upstream signals. We include these molecular switches in the appropriate immune cells and deliver the cells to the patient in the manner of conventional cellular immunotherapy. We have developed two such switches: a safety switch, designed to initiate programmed cell death, or apoptosis, of the immunotherapy cells, and an activation switch, designed to stimulate activation and in some cases proliferation of the immunotherapy cells. Each of our technologies incorporates one of these switches, for enhanced, real time control of safety and efficacy:

- n ***CaspaCIDE*** is our safety switch, incorporated into our HSCT and T-cell receptor, or TCR, product candidates, where it is inactive unless the patient experiences a serious side effect. In that event, rimiducid is administered to fully or partially eliminate the cells, with the goal of terminating or attenuating the therapy and resolving the serious side effect.

- n ***CIDeCAR*** consists of CAR T cells modified to include our CaspaCIDE safety switch and in which the CAR incorporates the signaling domains of two proteins, MyD88 and CD40. Together, these form our proprietary dual co-stimulatory domain, MC, which is designed to activate T cells in the presence of cancer cells more potently than co-stimulatory molecules CD28 and 4-1BB, which are used in current CAR T cell therapy. Incorporation of CaspaCIDE in a CIDeCAR product candidate is intended to allow the enhanced potency of MC co-stimulation to be deployed safely in patients.
  
- n ***GoCAR-T*** consists of CAR T cells that are modified to include the proprietary dual co-stimulatory domain, MC. In contrast to CIDeCAR, MC is structured in GoCAR-T as a molecular switch, separate from the

**Table of Contents**

chimeric antigen receptor, which itself contains no co-stimulatory domains. GoCAR-T is designed to allow control of the activation and proliferation of the CAR T cells through the scheduled administration of a course of rimiducid infusions that may continue until the desired patient outcome is achieved. In the event of emergence of side effects, the level of activation of the GoCAR-T cells is designed to be attenuated by reducing the rimiducid administration schedule.

- n **DeCIDE** consists of dendritic cells that are modified to include the same MC switch used in GoCAR-T. Upon exposure to rimiducid, dendritic cells containing DeCIDE become highly activated in a process that is less susceptible to being turned off by the immune system's natural inhibitory processes. By administering rimiducid after the patient has been vaccinated and the dendritic cells have had time to migrate to the draining lymph nodes, our DeCIDE product candidates are designed to be activated in a potent and long-lasting manner.

By incorporating our novel switch technologies, we are developing product candidates with the potential to elicit positive clinical outcomes and ultimately change the treatment paradigm in various areas of cellular immunotherapy. Our clinical product candidates; each of which is a combination product of genetically modified immune cells and rimiducid, are described below.

- n **BPX-501.** We are developing a CaspaCIDE product candidate, BPX-501, as an adjunct T-cell therapy administered after allogeneic HSCT, using donor stem cells. In a typical allogeneic HSCT procedure, a patient receives a full complement of immune cells including both donor stem cells and donor T cells. T cells in the transplant often cause serious and potentially fatal side effects, such as GvHD. BPX-501 is designed to decrease the risk of including T cells with the transplant by enabling the elimination of donor T cells through the triggering of the CaspaCIDE safety switch upon emergence of GvHD. In a 10-patient Phase 1 clinical trial with CaspaCIDE modified T cells, conducted by an academic collaborator, four patients developed GvHD after donor T-cell infusion. A single dose of rimiducid rapidly eliminated over 90% of the modified T cells and resolved GvHD in all four patients without recurrence of GvHD. These findings have been replicated in preliminary data from three patients in a second clinical trial of CaspaCIDE-modified T cells. BPX-501 is currently being evaluated in multiple Phase 1/2 clinical trials in the United States and Europe, with the first top-line data expected in the second half of 2015.
- n **BPX-201.** We are developing a DeCIDE product candidate, BPX-201, as a dendritic cell cancer vaccine made from the patient's own white blood cells, designed to treat metastatic castrate-resistant prostate cancer or, mCRPC. It targets the prostate specific membrane antigen, or PSMA, and uses our DeCIDE activation switch technology. BPX-201 is currently being evaluated in an 18-patient Phase 1 clinical trial for mCRPC. We are evaluating opportunities for BPX-201 in combination with other cancer immunotherapies, such as checkpoint inhibitors, which are antibodies designed to block certain inhibitory receptors on the surface of T cells, and thus potentiate the T cells' ability to promote an immune response against cancer. We believe that the increased numbers of PSMA-specific T cells migrating to deposits of prostate cancer in the body that BPX-201 is designed to generate may serve as a substrate for checkpoint inhibitors, resulting in a synergistic, more potent anti-cancer immune response.

In addition, our preclinical product candidates are designed to overcome the current limitations of CAR-T and TCR therapies and include the following:

- n **BPX-401.** We are developing a CDeCAR product candidate, BPX-401, as a next-generation CAR T cell therapy for hematological cancers that express the CD19 antigen. CD19 is an antigen expressed in many hematological cancers, including acute lymphocytic leukemia, or ALL, chronic lymphocytic leukemia, or CLL, and certain non-Hodgkin's lymphomas. We believe that, while the activity of CAR T cell therapy has been demonstrated in early-stage clinical trials by third party researchers in these indications, safety issues, such as cytokine release syndrome, a systemic inflammatory response that is produced by elevated levels of cytokines that are associated with T-cell activation and proliferation, remain a major concern, which may be addressed by BPX-401.
  
- n **BPX-601.** We are developing a GoCAR-T product candidate, BPX-601, for solid tumors overexpressing the prostate stem cell antigen, or PSCA, such as some prostate, pancreatic, bladder, esophageal and gastric

## Table of Contents

cancers. We have obtained positive proof-of-principle data in an animal pancreatic tumor model, which we believe validate BPX-601's activity and rimiducid's ability to modulate therapeutic effect.

- n **BPX-701.** We are developing a CaspaCIDE TCR product candidate, BPX-701, in collaboration with Leiden University Medical Center, initially for the treatment of PRAME-expressing melanoma, sarcomas and neuroblastoma. Based on *in vitro* studies, BPX-701 has demonstrated strong affinity to panels of cancer cells presenting PRAME peptides and low affinity to non-tumor cells. In other *in vitro* studies, rimiducid administration has shown the ability to eliminate BPX-701 cells.

We expect to file investigational new drug applications, or INDs, for BPX-701 in the second half of 2015 and for BPX-401 and BPX-601 in 2016. Our IND-enabling activities for each of these preclinical product candidates, include manufacturing key components and developing a robust process to produce cell products that comply with regulations of the U.S. Food and Drug Administration, or FDA, and other regulatory agencies. We have developed an efficient and scalable process to manufacture genetically modified T cells of high quality and purity. This process is being implemented by our third-party contract manufacturers to produce BPX-501 for our clinical trials. We expect to leverage our resources, capabilities and expertise for the manufacture of our CAR-T and TCR product candidates.

## Strategy

Our goal is to become a leading innovator in the field of cellular immunotherapy by maximizing the inherent potential of this therapeutic modality and developing medicines with a differentiated combination of safety and efficacy. The key elements of our strategy to achieve this goal are as follows:

- n **Pursue a broad development strategy that will maximize the market potential of BPX-501.** We believe that BPX-501 will enable physicians to maximize the benefits of adjunct T-cell therapy for allogeneic HSCT, such as immune system recovery, prevention or treatment of relapse of underlying disease and improvement in stem cell engraftment, while mitigating safety issues associated with the therapy. Based on these attributes, BPX-501 may serve an integral role in the treatment paradigm for allogeneic HSCT in various diseases and increase the overall patient eligibility for the procedure. In order to make BPX-501 accessible to a broad group of patients and maximize the market potential of this product candidate, we are conducting multiple Phase 1/2 clinical trials that include U.S. and European protocols, adult and pediatric patients and different indications and usage of BPX-501. We expect to report data from these clinical trials and discuss registration trial design at an end-of-Phase 2 meeting with the FDA and European regulatory authorities in the first half of 2016.
- n **Focus on developing proprietary CAR-T and TCR product candidates with an improved safety and efficacy profile.** We intend to build a robust clinical pipeline of our own novel CAR-T and TCR product candidates, which incorporate our proprietary switch technologies, CIDE CAR, GoCAR-T and CaspaCIDE, and focus on indications in which current CAR-T and TCR therapies have significant shortcomings. To this end, we are developing BPX-401 for hematological cancers expressing the CD19 antigen, BPX-601 for solid tumors overexpressing PSCA and BPX-701 for solid tumors expressing PRAME. We believe that these product candidates may address serious safety concerns associated with conventional CAR-T and TCR therapies and achieve higher overall potency and efficacy, thereby widening the therapeutic window compared to other CAR-T and TCR product candidates. We intend to dedicate significant resources in the near term to advance BPX-401, BPX-601 and BPX-701 as well as our other product candidates toward human proof-of-concept

data.

- n ***Selectively pursue partnerships and collaborations.*** Although our priority is to develop internal product candidates, we may pursue opportunistic partnerships and collaborations for our technologies, including CaspaCIDE and DeCIDE. In indications outside of our interest or expertise, we may structure transactions in which our molecular switches are incorporated into our partners' CAR-T or TCR product candidates. We intend to build on our existing strong relationships with premier cancer research centers around the world to identify new opportunities and position our company at the forefront of innovations in the field of cellular immunotherapy.
  
- n ***Continue to innovate around our proprietary CID platform.*** We believe that our CID platform can be further leveraged to discover other novel technologies and therapeutic applications to capitalize on additional market opportunities. We intend to evaluate BPX-201 and other product candidates based on our DeCIDE

**Table of Contents**

technology in combination with other cancer immunotherapy such as checkpoint inhibitors. We are also developing new switches and two-switch systems to provide greater control over cellular immunotherapy.

- n ***Continue to strengthen our intellectual property profile.*** We believe that having a comprehensive patent estate that provides strong barriers to entry is critical to the success of our business. As such, our management team has made a concerted effort to develop and secure our intellectual property since inception. We currently own or have exclusive licenses to 74 issued patents and 45 pending patent applications. These patents and patent applications include composition and/or method of use claims in the United States, Europe and other jurisdictions. We intend to continue to strengthen our patent estate by developing and filing for patents on various aspects of our technologies and product candidates as well as through in-licensing activities with research institutions and other biopharmaceutical companies.
  
- n ***Become a fully integrated cellular immunotherapy company.*** Developing product candidates for cellular immunotherapy is complex and requires significant in-house capabilities in various areas of drug development. Over the years we have built a solid foundation from which to fulfill the highly demanding clinical and regulatory requirements of genetically modified cellular immunotherapy, with expertise in research and discovery, clinical trial management, data analysis, manufacturing, quality assurance and regulatory affairs. We intend to use a portion of the net proceeds from this offering to continue hiring staff with necessary expertise and investing in infrastructure to support the growth of our clinical development activities and to enable us to become the leading cellular immunotherapy company.

**Recent Developments**

To enable further development of our proprietary technology and product candidates, we completed a private placement of \$55 million of Series C convertible preferred stock and warrants to purchase Series C convertible preferred stock in August 2014. Investors in the transaction included, among others, Baker Brothers, RA Capital Management, LLC, Perceptive Advisors, LLC, Jennison Associates LLC (on behalf of certain clients), Sabby Capital, LLC, Ridgeback Capital Management, venBio Select, Redmile Group, LLC and AJU IB Investment, as well as our then current investors, including AVG Ventures and Remeditex Ventures.

Certain aspects of our platform technology are inlicensed from ARIAD Pharmaceuticals, Inc., or ARIAD. In October 2014, we amended our license agreement with ARIAD, pursuant to which we agreed to pay ARIAD \$50 million in three tranch ed payments, including an initial payment of \$15 million in connection with the execution of the amendment. In exchange, ARIAD gave us a fully paid-up license to its cell-signaling technology and agreed to return of all of the 677,463 shares of our common stock currently held by ARIAD at the time of the second tranche payment. The scope of the license and the field of use were also expanded as part of the amendment. The amended agreement gives us a worldwide exclusive license to ARIAD's cell-signaling technology for broad use in human cell therapies for all diseases on a royalty- and milestone-free basis.

**Cellular Immunotherapy**

Cellular immunotherapy harnesses a patient's own immune cells to attack and eliminate harmful disease cells in the body. The immune system is the body's defense network. It consists of a number of cells and organs that, working together, recognize and respond to threats in the form of pathogens. T cells are a type of white blood cell that recognize pathogens and can target and eliminate them upon full activation through the addition of appropriate co-stimulatory signals.

Dendritic cells, another component of the immune system, are antigen-presenting cells found in skin and other tissues like the lining of the gut that can sense and respond to the environment. Dendritic cells engulf and process potential threats they encounter, presenting them as antigens to T cells and B cells to allow the body to mount an immune response.

The following three therapeutic applications of cellular immunotherapy have been the primary areas of research and development by research institutes and biopharmaceutical companies, given their promise of effectively treating patients suffering from severe and life-threatening diseases.

**HSCT.** HSCT is the transplantation of stem cells and other immune cells derived from bone marrow, peripheral blood or umbilical cord blood. The transplantation may be autologous, using the patient's own cells, or allogeneic, using a



## **Table of Contents**

donor's cells. HSCT is often the only curative option for a wide range of treatment-refractory hematological cancers, such as ALL, acute myeloid leukemia, or AML, and chronic myeloid leukemia, or CML. HSCT is also used as a high-risk treatment for orphan inherited blood disorders, such as sickle cell disease, beta-thalassemia and certain immune disorders.

***Dendritic Cell Therapy.*** Whereas HSCT and CAR T cells involve direct administration of T cells to the patient, dendritic cell therapies are designed to indirectly stimulate T cells already present in the patient. Given the important role of dendritic cells in initiating an immune response in the body, substantial research has been conducted to leverage the attributes of dendritic cells to treat cancer. Cancer vaccines are the most common form of dendritic cell-based therapy. These vaccines entail collecting certain monocytes, a type of white blood cell, from the patient's body, maturing them into dendritic cells, loading them *ex vivo* with the patient's cancer antigens, and sometimes modifying them in other ways to improve their potency, and then re-infusing the modified dendritic cells in the patient.

***Genetically Modified T-cell Therapy (CAR-T and TCR).*** This approach entails collecting a patient's T cells, genetically modifying them *ex vivo*, or outside of the body, to incorporate specific receptors which target cancer cells and then re-infusing the modified T cells back into the patient. Two types of cancer-specific receptors are typically used, CARs that recognize whole antigens on the surface of cancer cells, and TCRs that bind to cancer-associated peptides, or fragments of proteins, from either inside or on the surface of the cancer cells. In early human clinical trials, CAR T cell therapy has demonstrated an unprecedented ability to achieve durable complete responses in some leukemias and lymphomas, even in patients who have suffered multiple relapses.

The following graphic shows how a standard CAR is constructed. A CAR includes:

n A single-chain Fv fragment, or scFv, the component of an antibody that recognizes a target antigen;

n Co-stimulatory domains of CD28 or 4-1BB, which are the signaling components of proteins expressed on T cells that provide co-stimulatory signals required for T-cell activation and survival; and

n CD3 chain, a component of the T-cell receptor, which provides the initial signal when the receptor engages with the antigen.

These components are normally found on three separate proteins but in a CAR they are fused together into a single receptor molecule. Introduction of a CAR creates a T cell that has been engineered to react in a potent way against a specified target cell surface antigen. Cancer cells have developed mechanisms to avoid activating

the immune system which may be circumvented by CAR T cells.

**Table of Contents**

***Limitations of Current Cellular Immunotherapy Approaches.***

Despite rapid advances in various approaches to cellular immunotherapy and the biopharmaceutical industry's considerable investment in research and development, certain challenges have prevented these therapies from realizing their maximum potential. Some of these obstacles and issues are highlighted below:

**Our Proprietary CID Technology Platform**

Our proprietary CID technology platform is designed to address the challenges of current cellular immunotherapies. Cellular activities and functions, such as growth, activation, proliferation and cell death, are controlled by cascades of specialized signaling proteins. Our CID platform consists of molecular switches, modified forms of these signaling proteins, which are triggered inside the patient by infusion of a small molecule, rimiducid, instead of by natural upstream signals. Our product candidates are based on either a safety switch, or an activation switch. After rimiducid is administered, the safety switch is designed to lead to programmed cell death, or apoptosis, and the activation switch is designed to lead to proliferation and/or activation of immune cells.

## Table of Contents

We incorporate the molecular switches in the appropriate immune cells and administer them to the patient. After the modified immune cells are inside the patient's body, specific functions of these cells may be controlled by administering rimiducid by intravenous, or IV, infusion. Rimiducid has been designed to bind to a specifically designed domain of CID switch proteins. Once introduced, rimiducid couples, or dimerizes, CID switch proteins together to create a cluster that triggers the signaling cascade. Aside from its impact on CID-modified immune cells bearing switch proteins, rimiducid has no other known effect on the body. To date, rimiducid has been used in more than 150 infusions in humans without any reported serious adverse events related to rimiducid.

Our proprietary CID-based product candidates depend on the following signaling molecules to trigger signaling cascades, resulting in different cell activities:

- n ***Caspase-9: Signaling Molecule for Apoptosis.*** Caspase-9 is the initiating enzyme in the apoptosis pathway. When activated, caspase-9 starts a signaling cascade, including the activation of caspase-3, which ultimately leads to apoptosis, a non-inflammatory process of cell elimination.
  
- n ***MyD88/CD40: Signaling Molecules for Activation and Proliferation.*** Myeloid differentiation primary response gene, or MyD88, is a protein that has functions in cellular responses to stimuli such as stress, cytokines and bacteria or viruses. CD40 is a co-stimulatory protein found on antigen-presenting cells, such as dendritic cells and B cells and is required for their activation. Although the effects of MyD88 and CD40 have been studied previously in dendritic cell therapies, our novel approach applies them to T cell based immunotherapies.

## **Table of Contents**

### **Our Proprietary Switch Technologies**

With the CID platform as the foundation, we have created different molecular switch technologies customized for specific cellular immunotherapy approaches and therapeutic indications. The table below summarizes our key switch technologies.

#### ***CaspaCIDE***

CaspaCIDE is our CID safety switch technology designed to eliminate cells in the event of toxicity. The CaspaCIDE switch consists of the CID-binding domain coupled to the signaling domain of caspase-9, an enzyme that is part of the apoptotic pathway. Infusion of rimiducid is designed to trigger activation of this domain of caspase-9 (iCasp9), which in turn leads to selective apoptosis of the CaspaCIDE-containing cells. Because CaspaCIDE is designed to be permanently incorporated into our cellular therapies, the safety switch has the potential to be available for use long after the initial therapy is delivered. This technology is applied to our lead clinical product candidate, BPX-501, an adjunct T-cell therapy after allogeneic HSCT, and to our TCR product candidate, BPX-701.

We believe that CaspaCIDE is the optimal cell therapy safety switch technology. The only other widely reported approach used in the clinic is based on the Herpes simplex virus thymidine kinase, or HSV-tk, a non-human and as such immunogenic protein which is activated to kill the cell by the widely-used anti-viral drug, ganciclovir. Comparative studies have demonstrated CaspaCIDE's superiority to HSV-tk, based on lack of immunogenicity, effectiveness in rescuing animals from toxicities that have progressed, lack of dependence on the cell cycle for cell elimination, and most importantly, speed of elimination. In human trials, CaspaCIDE has demonstrated clinical efficacy in human patients beginning as soon as 30 minutes after administration of the activating drug, rimiducid. Lastly, rimiducid is bio-inert in the absence of cells containing a CID switch, and has no other clinical use; ganciclovir has side effects, and physicians are reluctant to lose the ability to use it to treat viral infections in patients treated with cells containing HSV-tk.

**Table of Contents**

Other cell elimination approaches described in the literature include gene modification of cells to express truncated epidermal growth factor receptor, or EGFRt, or codon-optimized CD20. Administration of the monoclonal antibodies cetuximab or rituximab, respectively, is intended to trigger antibody-dependent cellular cytotoxicity, or ADCC, mediated cell elimination. While CaspaCIDE eliminates cells via the apoptotic pathway, the body's non-inflammatory mechanism for this important function, we believe an ADCC-mediated mechanism may add to complications in patients already in an inflammatory crisis, such as seen with serious cytokine release syndrome after CAR T cell therapy. Moreover, cetuximab and rituximab, both anti-cancer therapies that have potentially serious side effects, are unlikely to be usable in a titratable manner. Lastly, these approaches have yet to demonstrate efficacy in clinical trials.

The following diagram reflects the mechanism of action of our CaspaCIDE safety switch:

**Table of Contents**

CaspaCIDE has been evaluated in numerous preclinical studies and clinical trials. We observed the dose response to rimiducid by measuring the viability of BPX-501 cells in culture following the addition of increasing amounts of rimiducid to the culture medium as well as by measuring the survival of BPX-501 cells *in vivo* in immune-deficient mice following injection of increasing doses of rimiducid. In these preclinical studies, rimiducid rapidly and consistently reduced or eliminated CaspaCIDE-containing cells in a dose-dependent manner. In addition to using our CaspaCIDE technology for the substantial elimination of cellular therapy (like an off switch), we are studying partial elimination of a cellular therapy (like a dimmer switch) by delivering reduced doses of rimiducid.

In addition to our internal preclinical and clinical development activities, we are collaborating with renowned cancer research centers with expertise in cellular immunotherapy to apply our CaspaCIDE safety switch to the collaborators CAR-T product candidates. The National Cancer Institute, or NCI, has initiated a Phase 1/2 clinical trial for sarcoma and other solid tumors with a CAR construct targeting a solid tumor antigen combined with CaspaCIDE. Although we are not the sponsor of this clinical trial, we believe that it may extend clinical proof of principle for CaspaCIDE from the HSCT setting to the CAR T cell setting.

**Table of Contents**

***CIDeCAR***

CIDeCAR consists of a CAR T cell that incorporates MC, our proprietary novel dual co-stimulatory domain, for improved T-cell activation and proliferation, and the CaspaCIDE safety switch. CAR interaction with cancer cell antigens is designed to lead to MC signaling, which then leads to activation of T cells. In the event of serious toxicity, rimiducid activation of caspase-9 is designed to eliminate the CIDeCAR T cells.

The following diagrams reflect the mechanism of action of our CIDeCAR technology:

In order to improve the effectiveness of CAR T cells in settings other than blood cancers located principally in the bone marrow, such as leukemia, some researchers have been working to develop armored CARs in which supplemental co-stimulatory signals or pro-inflammatory cytokines are added to the CAR T cells. Like an armored CAR, we include MC in our CIDeCAR technology in order to increase the potency of the therapy in these indications. While promising, these approaches may exacerbate safety issues found in standard CARs, such as cytokine release syndrome. We incorporate CaspaCIDE into CIDeCAR to address these safety concerns.

In proof-of-principle preclinical studies of CIDeCAR technology, CIDeCAR candidate BPX-401 and CIDeCAR solid tumor CAR targeting Her2, both of which incorporate MC, in place of the standard co-stimulatory molecules CD28, 4-1BB, or both together, were evaluated *in vitro*. These preclinical studies show that CIDeCAR technology results in enhanced activation, proliferation and tumor cell killing compared to standard comparator CARs. In addition, these studies demonstrate elimination of these CIDeCAR T cells after exposure to rimiducid.



**Table of Contents**

**BPX-401 (CD19 CIDE CAR)**

**Solid Tumor CIDE CAR (e.g., Her2)**

**Figure 1A**

**Figure 1B**

**Figure 2A**

**Figure 2B**

**Figure 3A**

**Figure 3B**

**Figure 4A**

**Figure 4B**

---

**Table of Contents**

**BPX-401 (CD19.MC. ) and solid tumor CIDECAR candidate targeted Her 2 (Her2.MC. ) were compared to non-modified T cells, or NT, and other comparably-targeted CAR-T constructs, such as early generation CAR-T with CD3 alone (CD19. and Her2. ), current generation CAR-T with CD3 and either CD28 (CD19.28. or Her2.28. ), 4-1BB (CD19.BB. or Her2.BB. ), or both co-stimulatory molecules (CD19.28.BB. or Her2.28.BB. ). The cells were cultured together with their target-expressing cancer cells (Daudi and Raji cell lines for CD 19, SK-BR-3-GFP for Her2). The cultures were analyzed for the presence of cytokines e.g. IL-2, and both tumor cell and T cell number. In addition, we evaluated the ability of the CaspaCIDE safety switch to eliminate both CIDECARs by counting T cell number before, and after overnight exposure, to rimiducid.**

Preclinical animal studies have shown that BPX-401 cells exhibit both anti-tumor activity and partial or complete elimination of T cells after administration of rimiducid in an NSG mouse Raji tumor model.

***GoCAR-T***

Our GoCAR-T technology incorporates a switch that activates CAR T cells when triggered by both rimiducid and the targeted antigen expressed on the surface of the cancer cells. Current generation CAR T cell constructs consist of a CD3 domain and one or more co-stimulatory molecules that are both activated when a cancer antigen binds to the portion of the chimeric antigen receptor on the outside of the engineered T cell. This reliance on antigen for activation of the CAR T cell results in an unpredictable and inherently uncontrollable therapeutic effect. For example, CAR T cells that target the CD19 receptor have been shown to proliferate in excess of 100,000-fold in some patients, to comprise over 50% of circulating lymphocytes. Solid tumor CAR T cells, on the other hand, often fail to proliferate or persist at all for more than a few days or weeks and have been largely ineffective. In each situation, the physician has no effective way to intervene to achieve greater consistency, once the cells have been administered.

Our GoCAR-T technology is designed to change current paradigm by separating the CIDECAR dual co-stimulatory domain, MC, from the antigen recognition domain and moving it onto a separate molecular switch that can be controlled by rimiducid. GoCAR-T cells can only be fully activated when exposed to both the cancer cells and rimiducid (see figure below). This separation is designed to control the degree of activation of the CAR T cells through adjustments to the amount of rimiducid administered, but still in a tumor-dependent manner.

**Comparison of Conventional CAR-T Technology vs. GoCAR-T**

**Table of Contents**

In a proof-of-principle *in vitro* study of our GoCAR-T technology (shown below) GoCAR-T cells targeting the PSCA antigen can only be fully activated, as evidenced by production of IL-2 (left panel) and T-cell proliferation (right panel) when the GoCAR-T cells are exposed to both their target PSCA-expressing human pancreatic cancer cells and rimiducid.

In further *in vivo* studies of GoCAR-T technology, target antigen PSCA-expressing Capan-1 human pancreatic tumors were established in immune-deficient, or NSG, mice. After seven days, five mice received control T cells modified only with firefly luciferase, an imaging protein, and 10 mice received T cells modified with MC in the form of a molecular switch or iMC, plus a PSCA. CAR (together, BPX-601) and firefly luciferase. Five mice in this second group also received 5 mg/kg rimiducid weekly. T-cell imaging clearly demonstrated that GoCAR-T cells can be stimulated to proliferate *in vivo* when exposed to target antigen-expressing cancer cells by rimiducid administration.

## **Table of Contents**

We believe these studies together provide proof-of-principle that GoCAR-T technology may allow rimiducid to modulate the therapeutic effect from initiation of treatment, turning CAR T cell therapy from an uncontrollable, and largely unpredictable class into a more predictable therapy which can be adjusted, like a small molecule, to the patient's therapeutic window to the appropriate level.

### ***DeCIDE***

DeCIDE technology is used to control the activation of dendritic cells. Dendritic cells are an important part of the immune system, processing antigens for presentation to T cells. Optimal stimulation of dendritic cells requires the activation of both the CD40 and toll-like receptor, or TLR, pathways, which results in maturation and activation of the dendritic cells as well as production of key cytokines, such as IL-12. These processes lead to a therapeutic response to the antigen by the patient's immune system. The potency of an immune response is governed by the maturation of dendritic cells in the patient's lymph nodes as well as the duration of interaction between activated dendritic cells with circulating T cells.

Activation of CD40 receptors on dendritic cells normally occurs via engagement of the trimeric CD40 ligand expressed on the surface of activated T helper cells (figure below, panels 1 and 2). This potent activation signal for dendritic cells may be quickly turned off by the action of certain proteases (MMP for example), which can cut and remove the CD40 ligand-binding portion of CD40 receptors (panel 3).

To take control of the activation of the dendritic cells and the resulting immune response to cancer, we have taken the signaling domains of CD40 and MyD88, and coupled them to our CID binding domain, to create our inducible MC switch, which we then insert into dendritic cells along with the PSMA antigen. Upon exposure to rimiducid, DeCIDE-containing dendritic cells are designed to become highly activated in a process that is no longer susceptible to being turned off by MMP (second set of panels below). Our DeCIDE technology, thus, potentially enables us to activate dendritic cells with rimiducid after the patient has been vaccinated and the dendritic cells have migrated to the draining lymph nodes in a potent and long-lasting manner.

## **Table of Contents**

Fully activated dendritic cells exhibit a number of important traits, including increases to the levels of important cell surface markers, and production of important cytokines, such as IL-12. As demonstrated below, cultured BPX-201 cells, which are dendritic cells transduced with our DeCIDE switch technology, produce supra-normal levels of IL-12 in response to rimiducid. These data suggest that in addition to the temporal control of dendritic cell activation that DeCIDE technology affords, once exposed to rimiducid, DeCIDE-containing dendritic cells become highly activated, which may lead to more potent anti-cancer activity in patients.

Our DeCIDE technology is applied to our cancer vaccine product candidate BPX-201.

## **Our Product Candidates**

Based on our CID platform and associated technologies, we have built a robust pipeline of controllable immunotherapy product candidates with the potential for clear differentiation compared to other cellular immunotherapies. Our product candidate pipeline is set forth below:

## **Table of Contents**

### **BPX-501: CaspaCIDE Product Candidate for Hematological Diseases**

BPX-501 is an adjunct T-cell therapy administered after allogeneic HSCT that incorporates our CaspaCIDE technology. We are developing BPX-501 in the initial indications of hematological cancers and orphan inherited blood disorders. In the indication of hematological cancers, we are pursuing two regulatory pathways: (1) support of immune system recovery following allogeneic HSCT, and (2) the treatment of the relapse of underlying disease following allogeneic HSCT. In orphan inherited blood disorders, we are pursuing a parallel regulatory pathway for immune system recovery following allogeneic HSCT.

We are currently conducting two Phase 1/2 clinical trials of BPX-501 in the United States: BP-001, a clinical trial in adults in which BPX-501 is administered after initial allogeneic HSCT for hematological cancers, and BP-003, a clinical trial in children with orphan inherited blood disorders in which BPX-501 is administered after initial allogeneic HSCT. In November 2014, we initiated an additional Phase 1/2 clinical trial in children with hematological cancers or orphan inherited blood disorders, BP-004, which will be conducted in both the United States and Europe. In addition, we are planning to initiate additional Phase 1/2 clinical trials in the United States and Europe in 2015, as part of our strategy to pursue a global regulatory approval and expand the potential addressable patient population for BPX-501.

### ***HSCT Market Overview***

HSCT is used to treat a wide range of hematological cancers, such as ALL, AML and CML, as well as orphan inherited blood disorders, such as sickle-cell disease, beta-thalassemia and certain immune disorders, due to its ability to achieve long-term disease remission or functional cure. The majority of pediatric HSCT procedures are for inherited blood disorders while the majority of HSCT in adults are for hematological cancers. Autologous transplantations (using patient's stem cells) and allogeneic transplantations (using donor stem cells) comprise 55% and 45%, respectively, of approximately 50,000 HSCT procedures conducted annually worldwide. Within the allogeneic HSCT market, finding a compatible donor is a major limitation to successful treatment. A test comparing the human leukocyte antigen, or HLA, types, which are proteins found on most cells in the body, is used to measure compatibility of the donor and the patient. A sibling of the patient with an exactly matching HLA types is an ideal donor for the patient. However, only 30% of potential allogeneic HSCT patients have such a match, and this percentage is expected to decrease as the average family size among the population continues to decrease. A suitable match can be identified in databases of unrelated donors for approximately 60% of patients of European descent, but a suitable match of patients from other ethnicities is difficult to find and occurs in approximately 10% of patients. This leaves a large pool of patients in need of alternative treatment options.

Because of the challenges in identifying matched donors, there has been a growing interest in the use of hematopoietic stem cells from haplo-identical (half-matched) donors for HSCT, or haplo-HSCT. Identifying potential haplo-HSCT donors is much easier and faster because biological first degree relatives are all haplo-identical to the patient. Although haplo-HSCT techniques have improved patient eligibility for transplantation and increased the use of allogeneic HSCT, patients face substantial health risks, such as GvHD, low immune function and higher likelihood of relapse of underlying hematological disease.

### ***Limitations of Current Treatments***

In haplo-HSCT procedures, GvHD is the biggest health risk. GvHD develops when donor T cells attack the patient's cells, recognizing antigens on the patient's cells as foreign. The current standard of care for treating GvHD is high-dose steroids. However, in many cases, steroids' slow onset of action or lack of effect in certain patients has led to death. In addition, steroids may suppress all T-cell activity which may leave patients open to infection. According to a 2010

study published by the American Society for Blood and Marrow Transplantation, patients who fail to achieve a complete response to steroid therapy for severe GvHD within 14 days accounted for 83% of all deaths within six months of initiating GvHD treatment.

Various techniques have been developed to deplete allo-reactive (GvHD-causing) donor T cells in order to prevent GvHD. However, these approaches tend to eliminate the helpful T cells, as well as the harmful ones, leaving the patient immuno-compromised for a year or longer and prone to relapse of underlying disease. In addition, these procedures reduce the probability of stem cell engraftment and increase the risk of opportunistic infections in the patient.

**Table of Contents**

Adding back defined numbers of donor T cells after T-cell depletion can help restore some functions of the patient's immune system, but the risk of GvHD increases with increasing T cell doses. Unfortunately, the maximum dose of donor T cells that can be safely added back varies widely from patient to patient.

***Our Solution***

We are developing BPX-501 as an adjunct donor T-cell therapy designed to improve stem cell engraftment and accelerate immune system recovery, while providing for rapid and effective resolution of GvHD, should it occur, with the administration of rimiducid. As a result, BPX-501 is designed to enable a broader range of non-matched donors and make haplo-HSCT a viable treatment option for a broader patient population. We have identified two potential applications of BPX-501:

- n an add-back of donor T cells administered to accelerate immune system recovery after allogeneic haplo-HSCT in which the T cells in the transplanted stem cells were depleted; and
- n a donor T cell infusion administered to prevent or treat relapse of underlying disease after allogeneic HSCT independent of donor match.

For each of these indications, BPX-501 potentially allows the patient to receive the benefits of adjunct donor T-cell therapy, while mitigating associated health risks. If a patient develops GvHD after an infusion of BPX-501, the physician can administer rimiducid to eliminate the modified donor T cells inside the patient, in order to potentially resolve GvHD. In clinical trials to date, GvHD-causing cells have been eliminated as quickly as 30 minutes after administration of rimiducid, and GvHD symptoms have resolved within one to two days.

***CASPALLO: Phase 1 Clinical Trial***

As reported in the *New England Journal of Medicine* in 2011, the Texas Children's Hospital, in collaboration with Baylor College of Medicine, under an agreement with us, conducted a Phase 1 clinical trial of T cells genetically modified to include CaspaCIDE. The CaspaCIDE switch (iCasp9) used in these cells was identical to the one used in BPX-501. The only differences between these cells and BPX-501 were cell processing techniques used and method of treatment. In this clinical trial, the CaspaCIDE T cells had been depleted of most of the allo-reactive cells prior to administration. The clinical trial enrolled ten pediatric patients selected to undergo allogeneic haplo-HSCT with T-cell depletion as a treatment for high-risk hematological cancers. Each patient received CaspaCIDE T cells between 30 and 90 days post-transplant. There were no immediate toxicities reported to us for this treatment.

Of the ten patients in the clinical trial, five of six patients with an original diagnosis other than ALL achieved complete remission throughout the trial period. Of four ALL patients, who do not typically respond well to donor lymphocyte infusions, one achieved complete remission throughout the trial period and three achieved remission lasting between five and 19 months, but later died.

Four patients in the clinical trial developed acute GvHD from two to six weeks after infusion of CaspaCIDE T cells. They were given a single dose of 0.4 mg/kg rimiducid. This resulted in a rapid decline in the level of circulating CaspaCIDE T cells. Within 30 minutes, the level of these cells decreased by 90% and within 24 hours, 99% of the most activated cells were eliminated. The characteristic acute GvHD skin rash in the four patients resolved within 24 to 48 hours and required no further treatment. There were no reported adverse events associated with rimiducid administration.





**Table of Contents**

The following table reflects the results of treatment for each patient in the study:

## **Table of Contents**

The data highlighted below in one of the patients from the CASPALLO trial who developed GvHD illustrates that:

- n the CaspaCIDE T cells, including those responsible for causing GvHD, were eliminated in 30 minutes after rimiducid administration;
- n patient's skin rash and elevated bilirubin, indicating Grade 2 GvHD involving the liver, returned to normal within 24 hours in response to rimiducid; and
- n T cells re-expanded without recurrence of chronic or acute GvHD.

### **Case History of Three-year-old Haplo-HSCT Patient Who Developed Grade 2 Liver GvHD**

The published report describing long-term follow-up of patients in this trial demonstrated that infusion of CaspaCIDE T cells could promote the control of cytomegalovirus, or CMV, Epstein-barr virus, or EBV, adenovirus, or AdV, BK virus, or BKV, and (in one patient) *Aspergillus* infections after haplo-HSCT. All patients with these infections quickly reduced both their viral loads and clinical symptoms. The researchers noted that in patients with viral reactivation, the infusion of rimiducid did not permanently delete virus-specific CaspaCIDE T cells since these cells consistently recovered, contributed to the elimination of the infection and remained detectable for months.

### ***DOTTI: Ongoing Phase 1 Clinical Trial***

The Texas Children's Hospital and Baylor College of Medicine, under an agreement with us, are conducting another Phase 1 clinical trial of CaspaCIDE T cells in pediatric patients with similar parameters as the CASPALLO clinical trial, except that the CaspaCIDE T cells were not depleted of allo-reactive cells prior to administration. This clinical trial has enrolled 11 patients as of September 30, 2014 and is ongoing, with top-line data expected by the end of 2014. As of September 30, 2014, we have been informed that three patients who developed GvHD were treated with rimiducid and that each demonstrated rapid resolution of GvHD and control of viral infections.

The collaborators have shared data with us from one of these patients in the clinical trial that was treated with rimiducid. In this 14 year-old patient who developed severe GvHD, rimiducid induced the following effects:

- n severe skin rash was resolved within 25 minutes;
- n a 105° fever returned to normal within one hour; and
- n levels of various cytokines, including IL-6, returned to normal in 2.5 to 24 hours.

***University of Texas, MD Anderson 2012-0501: Ongoing BPX-501 Phase 1/2 Clinical Trial***

We are collaborating with the University of Texas, MD Anderson Cancer Center on an ongoing investigator-led open-label Phase 1/2 clinical trial of our product candidate, BPX-501. This clinical trial is expected to enroll 10 adults in the United States who have previously received allogeneic haplo-HSCT and is expected to have a duration of 18 months. BPX-501 is given as a prophylaxis treatment to prevent relapse of underlying disease. Three patients have been enrolled, and one patient has been treated with rimiducid for GvHD.

**Table of Contents**

In this patient, BPX-501 T cells demonstrated engraftment and expansion after administration, increasing to 38% of the total T-cell population by Week 4 after administration. Shortly thereafter, the patient developed stage 3 skin GvHD, comprised of a skin rash involving over 80% of the patient's body. The patient received a brief course of steroids and received a dose of rimiducid as part of the clinical trial protocol. The graph below shows greater than 87% reduction of BPX-501 T cells within three hours of rimiducid dosing (the first time point examined), followed by resolution of symptoms of GvHD within two days.

**First Dosing of Rimiducid to Treat Stage 3 Skin (Grade 2) GvHD in a Patient Treated with BPX-501**

**Table of Contents**

***BPX-501 Development Plan***

We are pursuing a development strategy that will make BPX-501 available to a broad patient population in need of a better HSCT treatment regimen. As a result, we have designed our existing Phase 1/2 clinical trial protocols to consist of U.S. and European trials, adult and pediatric patients and different indications and BPX-501 usage. We submitted INDs for BPX-501 in September 2012 and May 2013 for our ongoing clinical trials and MD Anderson Cancer Center submitted an IND in April 2013 for clinical trial protocol BP-006. The table below outlines our ongoing and planned Phase 1/2 clinical trials for BPX-501.

## **Table of Contents**

### ***BPX-501 On-going Phase 1/2 Clinical Trials***

#### ***BP-001***

BP-001 is a Phase 1/2 dose escalation clinical trial designed to evaluate the safety and feasibility of BPX-501 infused after partially mismatched, related, or haploidentical, T cell-depleted HSCT. The purpose of this clinical trial is to determine whether BPX-501 infusion can facilitate engraftment, enhance immune reconstitution and potentially improve the graft versus leukemia, or GVL, effect, with the potential for reducing the severity and duration of severe GvHD. The trial will evaluate the treatment of acute GvHD by the infusion of rimiducid in those patients who present with severe GvHD, Grades 3 and 4, as well as those patients with Grade 1 and 2 GvHD who progress on corticosteroid therapy or do not respond within three days. The trial is intended to enroll up to 36 adult patients with a duration of approximately three years. As of November 18, 2014, five patients have been enrolled in this clinical trial.

#### ***BP-003***

BP-003 is a single arm, dose finding clinical trial designed to evaluate the safety and efficacy of BPX-501 followed by rimiducid on day seven after a partially mismatched, related, T cell-depleted HSCT in patients with inherited blood disorders. The purpose of this clinical trial is to determine the dose of BPX-501 with subsequent planned infusion of rimiducid to facilitate engraftment and prevent the occurrence of GVHD. The clinical trial is intended to enroll up to 20 pediatric patients with a duration of approximately three years. As of November 18, 2014 no patients have been enrolled in this clinical trial.

#### ***BP-004***

BP-004 is a Phase 1/2 clinical trial designed to evaluate the safety and feasibility of BPX-501 after partially mismatched, related, T cell-depleted HSCT in pediatric patients with either hematological cancer or inherited blood disorders. The purpose of this clinical trial is to determine whether BPX-501 can enhance immune reconstitution and retain the GVL effect, with the potential for reducing the severity and duration of severe acute GVHD. This clinical trial will evaluate the treatment of GVHD by the infusion of rimiducid in patients who present with Grade 3 or 4 acute GVHD, as well as patients with Grade 2 liver GVHD or with 1/2 GVHD, skin only, who progress or do not respond within seven days to standard of care treatment. The clinical trial is intended to enroll up to 30 patients with a duration of approximately three years. As of November 18, 2014, three patients have been enrolled.

We intend to conduct end-of-Phase 2 meetings with the FDA and the European regulatory agencies in the first half of 2016 to determine the optimal path for BPX-501 approval and appropriate designs for registration trials.

### **BPX-201: DeCIDE Cancer Vaccine Product Candidate**

We are developing BPX-201 as a dendritic cell cancer vaccine designed to treat mCRPC. BPX-201 is an autologous therapy, in which the patient's own white blood cells are extracted and modified *ex vivo*. The cells are matured and then genetically engineered to express the DeCIDE switch domains and the PSMA antigen. Then, the modified cells are washed, apportioned into individual doses, and frozen for later administration to the patient.

By incorporating the DeCIDE switch that activates therapy only in the presence of rimiducid, physicians may be able to strategically time the immune system's attack on cancer cells. The rationale behind this approach is to allow BPX-201 cells to bypass critical immune checkpoints that can potentially reduce therapeutic effect and migrate to

nearby lymph nodes to initiate a potent and durable antigen-specific T-cell response.

We submitted an IND for BPX-201 in September 2012 and it is currently being evaluated in an 18-patient Phase 1 clinical trial for mCRPC. The clinical trial design consists of three cohorts of six patients each, who will receive escalating doses of BPX-201. The patients will be followed for two years after enrollment and the clinical trial is expected to have a duration of three years. We are also intending to conduct preclinical studies to evaluate opportunities for BPX-201 in other solid tumors and as combination therapy with a checkpoint inhibitor.

We are evaluating opportunities for BPX-201 in combination with other cancer immunotherapies, such as checkpoint inhibitors. Checkpoint inhibitors act by removing inhibitory signals on antigen- or tumor- specific T cells already present in the patient's body. In the data shown below, BPX-201 shows the potential to stimulate proliferation of PSMA-specific T cells thereby providing a mechanism to successfully combine BPX-201 with checkpoint inhibitors. There are multiple checkpoint inhibitors in clinical and preclinical development, allowing us to potentially create a competitive situation in partnering BPX-201 with one or more of them.



**Table of Contents*****BPX-201 Phase 1 Clinical Trial***

We are evaluating BPX-201 in a multi-site Phase 1 dose escalation clinical trial in mCRPC patients to study its safety and preliminary efficacy. We anticipate that a total of 18 patients will receive BPX-201 every two weeks for a total of six cycles. Each dose of BPX-201 will be followed by a single dose of rimiducid given the following day. Enrolled patients undergo leukapheresis (harvesting of certain white blood cells) after baseline scans confirm progressive mCRPC. After manufacture and release of BPX-201, patients receive intradermal vaccination of BPX-201 every two weeks, for a total of six doses. Twenty four hours after each vaccine administration, patients are administered a two-hour infusion of rimiducid to activate the BPX-201 cells after they have had time to migrate to the draining lymph nodes.

**BPX-201 Clinical Trial Timeline and Stimulation of Immune System**

**As demonstrated in the figure above, the data from a patient enrolled in the first cohort of the trial demonstrated an increasing immune response to PSMA with each successive dose of BPX-501 through the first 12 weeks of treatment. The magnitude of the immune response is indicated by the number of spots in the three petri dishes with exposure ( + ) compared to without exposure ( - ) to target antigen PSMA peptides.**

**BPX-401: CIDECAR Product Candidate for Hematological Cancers**

We are developing BPX-401 for the treatment of hematological cancers overexpressing the CD19 antigen, such as ALL, CLL and certain types of non-Hodgkin's lymphoma. We have generated preclinical proof-of-principle data *in vitro* showing that BPX-401 has significant CAR T cell activation and proliferation potential, and may be more effective in killing cancer cells compared to other CAR-T constructs. We intend to file an IND and initiate a Phase 1/2 clinical trial in the first half of 2016.

The current standard of care in these indications, chemotherapy combined with monoclonal antibody therapies, works to varying degrees with high disease relapse rates. CD19-targeted CAR-T therapies have elicited high objective response rates in some of these B cell cancers, but they have demonstrated major safety risks.

***BPX-401 Preclinical Data***

BPX-401 was compared *in vitro* to non-modified T cells and other CAR-T constructs targeting the CD19 antigen, such as early generation CAR-T with CD3 alone, and current generation CAR-T with CD3 and either CD28, 4-1BB, or both co-stimulatory molecules. We have conducted *in vitro* studies that indicate, in addition to the safety feature, BPX-401 compares favorably to other therapies under development by third parties as measured by cell proliferation, production of key cytokines and *in vitro* tumor cell killing. In addition, rimiducid demonstrated an ability to rapidly eliminate engineered T cells in *in vitro* studies. These attributes may translate to best-in-class anti-tumor activity *in vivo*. The data from these studies is described under Business CIDECAR.

Preclinical animal studies have shown the BPX-401 cells exhibit both anti-tumor activity and partial or complete elimination of T cells after administration of rimiducid in an NSG mouse Raji tumor model.

**BPX-601: GoCAR-T Product Candidate for Solid Tumors**

We are currently conducting preclinical studies of BPX-601 for the treatment of solid tumors overexpressing the PSCA antigen. PSCA is highly expressed in some pancreatic cancers, as well as in a portion other solid tumors,

---

**Table of Contents**

including bladder, esophageal and gastric cancers. Although many product candidates are in development for these cancers, there are currently no approved products targeting PSCA. We intend to initiate a Phase 1/2 clinical trial in the second half of 2016. In order to commercialize this product candidate, we may need to obtain an additional intellectual property license.

***BPX-601 Preclinical Data***

BPX-601 *in vitro* data included under Business GoCAR-T showed that BPX-601 cells targeting the PSCA antigen can only be fully activated, as evidenced by production of IL-2 and CAR T cell proliferation, when they are exposed to both their target PSCA-expressing cancer cells and rimiducid. In addition, *in vivo* T-cell imaging clearly demonstrated that BPX-601 cells can be stimulated to proliferate when exposed to target antigen-expressing cancer cells by the administration of rimiducid.

BPX-601 was evaluated for anti-tumor activity against PSCA-target antigen expressing human pancreatic cancer (Capan-1) in an immune-deficient mouse model. Three groups of five mice each were engrafted with Capan-1 tumor cells then treated with either non-modified T cells, T cells expressing a standard CD3 CAR-T construct targeting PSCA, or BPX-601 construct containing the PSCA CD3 CAR and inducible MC on Day 6 and Day 13. For this study, mice were given IL-2 support, a common practice for CAR-T treatment, until Day 21. All mice received 5 mg/kg of rimiducid twice a week. As expected, in the arm treated with unmodified T-cell control, average tumor size rapidly increased, and no mice survived beyond Day 51. In the arm treated with a comparative PSCA CD3 CAR construct, the treatment did suppress tumor size to an extent, but shortly after the second injection of T cells and removal of IL-2 support, tumor size began to increase. By Day 57 all the mice in this arm were dead. In the BPX-601 arm, average tumor size remained low long after the second injection of T cells and removal of IL-2 support. Four out of five mice were still alive after 75 days, at which time the experiment was ended.

***BPX-601: In Vivo Preclinical Data in Mice***

Because the data above suggested that BPX-601 cells were effective even after withdrawal of IL-2 support, and since fully activated GoCAR-T cells produce higher levels of the critical T-cell growth cytokine, IL-2, than standard CD28-containing CARs (see figure below, left panel), we evaluated whether IL-2 support was necessary for the proliferation and anti-tumor effects of BPX-601 cells against human pancreatic cancers. Therefore, in a similar experiment to the one above (see figure below, right panel), anti-tumor efficacy *in vivo* was assessed by treating immune-deficient mice engrafted with Capan-1 (human pancreatic) tumor cells with two doses of BPX-601 cells on Days 4 and 6, but without any IL-2 support. All groups received 5 mg/kg of rimiducid twice per week. Tumors were best controlled in mice treated with BPX-601 cells and rimiducid compared to control T cells or standard CD28-containing CAR T cells, which were largely ineffective and not significantly different from non-transduced control T cells.

**Table of Contents**

These data demonstrate that BPX-601 may be effective in treating PSCA-expressing solid tumors in patients.

**BPX-701: CaspaCIDE TCR Product Candidate for Solid Tumors**

We are developing BPX-701, a TCR-based therapy that incorporates our CaspaCIDE technology, in collaboration with Leiden University Medical Center, for the treatment of PRAME-expressing melanoma, sarcomas and neuroblastomas. We intend to file an IND and initiate a Phase 1/2 clinical trial in the second half of 2015. Clinical sites for this trial have been identified.

BPX-701 is designed to target preferentially-expressed antigen in melanoma, or PRAME, a gene that is predominantly expressed in human melanomas but not in normal tissues. As initially reported in Clinical Cancer Research 2011, PRAME-specific clones showed high reactivity against a panel of PRAME positive tumor cell lines, metastatic melanoma, sarcomas and neuroblastoma tissues, and no reactivity against normal cell types, with the exception of low reactivity against kidney epithelial cells and intermediate reactivity against mature dendritic cells, or DCs. In other TCR programs, despite careful evaluation of normal tissues to identify potential off target effects, unexpected cross-reactivities have been encountered in clinical trials, leading to serious adverse events including patient deaths. BPX-701 containing the CaspaCIDE safety switch, has demonstrated complete elimination in response to rimiducid. Therefore, we believe a PRAME-TCR with CaspaCIDE can provide safety in the clinical development of this TCR.

**Other Development Programs**

We believe that our CIDE CAR, GoCAR-T and CaspaCIDE TCR technologies have broad applicability against a range of cancer targets which form the basis for additional development programs, some of which are described below:

## **Table of Contents**

### ***CIDeCAR for Solid Tumors***

Beyond hematological cancers, we are studying the full potential of CIDeCAR to enable treatment of more challenging solid tumor cancers in which concerns regarding toxicity are paramount in the field of cell therapy. To this end, we are conducting preclinical studies of various CIDeCAR product candidates targeting solid tumor antigens.

### ***GoCAR-T in Bulky Hematological Cancers***

The cell therapy treatment of bulky hematological cancers presents many of the same efficacy and safety challenges as the treatment of solid tumors. As such, we believe the potential for GoCAR-T cells to proliferate may lead to consistent, predictable efficacy for the treatment of these cancers. We are currently conducting preclinical studies of product candidates for the treatment of bulky hematological cancers, such as certain non-Hodgkin's lymphomas, as well as CLL.

### ***CaspaCIDE TCR for Hematological Cancers***

We are working with our collaborator, Leiden University Medical Center, to evaluate an additional TCR with high affinity for certain peptides for the treatment of hematological cancers, including ALL, CLL, MCL and MM. The TCR construct incorporates the CaspaCIDE safety switch.

## **Manufacturing, Processing and Delivering to Patients**

Our product candidates require a combination of three critical components: (1) viral vectors with DNA content encoded for our proprietary switch proteins and co-stimulatory and other accessory molecules, (2) patient-specific donor T cells or dendritic cells that are genetically modified by our viral vectors, and (3) the synthetic small molecule rimiducid which activates the switch proteins. Each of these components requires a separate supply chain and shares the same regulatory requirements applicable for biological or chemical materials suitable for human use. Details on each of these components are described below:

- n ***Viral Vectors.*** We use a retrovirus to transduce our T cell based product candidates. We believe that the retrovirus is optimal for T cell transduction given that it is an integrating vector that induces long-term gene expression, exhibits high transduction efficiency, has large capacity for DNA content, and have been safely used in clinical trials. To transduce dendritic cells, we use a specific type of adenovirus, which has been shown to be efficient at transducing this cell type and is cost-effective to manufacture and scale. The vector production is performed at multiple third-party supplier facilities under GMP procedures and requirements. These suppliers have significant experience and expertise in vector manufacturing and have dedicated capacity to satisfy demand for large clinical trials and product commercialization.
  
- n ***Genetically Modified T Cells and Dendritic Cells.*** We have agreements with reputable contract manufacturing organizations, or CMOs, with facilities in both the United States and Europe for processing and manufacturing our genetically modified T cells and dendritic cells. We have designed and refined a proprietary process for cell engineering that has been improved from lab-based open procedures used in academic and research settings to a functionally closed system that is more appropriate for large-scale clinical trials and commercialization. Our system is compliant with current guidelines and regulations for cell-based manufacturing in the United States and Europe and has been successfully transferred and implemented by our

CMOs.

- n ***Rimiducid***. Rimiducid is a synthetic small molecule which has been rationally designed to trigger the proprietary switch proteins in our CID platform. We have separate third-party manufacturers for the active pharmaceutical ingredient, or API and the finished drug product. Manufacturers of both the API and finished drug product are licensed to manufacture a variety of marketed drugs worldwide and have been selected based on their ability to provide supplies for our clinical trials and future commercialization.

Given that our product candidates are for patients whose conditions can rapidly deteriorate, we are focused on continuously refining our overall cell therapy process (manufacturing, processing and delivery to patient) to be more efficient.

Our current process cycle from our product candidates, from collection of white blood cells to infusion of the final product, can be completed in as little as two weeks and are customized to be complementary to the treatment procedure of interest in order to prevent any delays or complications.

## **Table of Contents**

The key steps of processing our T-cell and dendritic cell product candidates are depicted below:

### **Intellectual Property**

We seek to protect proprietary technology, inventions, and improvements that are commercially important to our business by seeking, maintaining, and defending patent rights, whether developed internally or licensed from third parties. We also seek to rely on regulatory protection afforded through orphan drug designations, data exclusivity, market exclusivity and patent term extensions where available as well as contractual agreements with our academic and commercial partners.

To achieve this objective, a strategic focus for us has been to identify and license key patents and patent applications that serve to enhance our intellectual property and technology position. Our intellectual property estate includes: (1) claims directed to core CID technologies and components used in our products; (2) claims directed to methods of treatment for therapeutic indications; (3) claims directed to specific products; and (4) claims directed to innovative methods for generating new constructs for genetically engineering T cells and dendritic cells. We believe our patent estate, together with our efforts to develop and patent next generation technologies, provides us with a substantial intellectual property position.

However, the area of patent and other intellectual property rights in biotechnology is an evolving one with many risks and uncertainties. We are aware there are third party patents having claims that may be considered relevant to the BPX-201 technology for which we are seeking, regulatory approval, however, we believe these patents have a patent term that may expire prior to the time we expect to obtain regulatory approval for this technology. We believe that if claims in one or more of the patents referenced in the previous sentence are asserted against us, we may be able to assert a defense for a safe harbor to patent infringement under 35 U.S.C. 271(e)(1) if certain requirements are met. Please refer to the section entitled **Risk Factors** **Risks Related to Our Intellectual Property** herein for associated risks.

---

**Table of Contents**

We are aware of a third party patent having claims directed to chimeric DNA comprising DNA segments encoding (1) a single chain antibody domain and (2) transmembrane and cytoplasmic domains of an endogenous protein. We believe that our BPX-401 and BPX-601 technologies are not covered by claims of this patent. Please refer to the section entitled Risk Factors Risks Related to Our Intellectual Property herein for associated risks.

We are aware of third party patents having claims that may be considered as being directed to single-chain antibody fragments that bind to PSCA and these patents may be considered relevant to BPX-601 technologies we are developing. We currently are evaluating whether or not we need to obtain rights to these patents under a license, and if it is determined that we need to obtain such rights, whether these rights can be obtained. Please refer to the section entitled Risk Factors Risks Related to Our Intellectual Property herein for associated risks.

To our knowledge, our patent estate, on a worldwide basis, includes 74 issued patents (18 of which are in the United States) and 45 pending patent applications (20 of which are in the United States) which we own or for which we have an exclusive (either in its entirety or within our field of use) commercial license as of November 14, 2014. Of these:

- n We have internally developed technology disclosed in three pending provisional patent applications and two utility patent applications in the United States, and two pending international (PCT) patent applications which relate to our CIDECAR technology. If these provisional patent applications are converted to utility patent applications, and U.S. patents issue from these, the estimated expiration date of the last to expire patent is in 2034 or later. If patents are issued in foreign jurisdictions, the anticipated expiration dates will also be in 2034.
- n We have internally developed technology disclosed in three pending provisional patent applications and one utility patent application in the United States, and one pending international (PCT) patent application which relates to our GoCAR-T technology. If these provisional patent applications are converted to utility patent applications, and U.S. patents issue from these, the estimated expiration date of the last to expire patent is in 2034 or later. If patents are issued in foreign jurisdictions, the anticipated expiration dates will be in 2034 or later.
- n We have internally developed technology disclosed in a U.S. provisional patent application, which relates to a non-inducible CAR and non-inducible co-stimulatory polypeptide, which may also be used in combination with our CIDECAR technology. If this provisional patent application is converted to a utility patent application, and a U.S. patent issues from it, the estimated expiration date of the patent is 2034 or later. If patents are filed and issued in foreign jurisdictions, the anticipated expiration dates will be in 2034 or later.
- n Pursuant to our licenses from Baylor, we have exclusive commercial rights to three issued U.S. patents expiring in 2024 or later, eight pending U.S. utility patent applications, one issued patent in Australia expiring in 2027, and 21 pending patent applications in foreign jurisdictions that relate to our GoCAR-T, BPX-201 and certain of our other technologies. If U.S. patents issue from the currently pending U.S. patent applications, the estimated expiration date of the last to expire patent is 2031 or later. Patent applications have been filed in foreign jurisdictions, including Australia, Canada, Europe, Hong Kong and Japan. If patents from the currently pending patent applications are issued in foreign jurisdictions, the estimated expiration dates range from 2024 to 2029.



- n Pursuant to our license agreement with ARIAD, as amended, we have exclusive commercial rights within our field of use to 69 patents (14 in the United States and 55 in foreign jurisdictions, including Australia, Canada, China, Europe, Japan and Korea), which relate to dimerizer technology. The estimated expiration date of the last to expire U.S. patent is February 2016. The estimated expiration dates of the last to expire foreign patents are between 2014 and 2019. Also pursuant to this license agreement, we have exclusive commercial rights within our field of use to two pending applications (one in the United States and one in Australia) which relate to dimerizer technology. If a U.S. patent issues from the currently pending U.S. patent application, the estimated expiration date of the last to expire patent is 2032 or later. If a patent issues in Australia from the currently pending application, the estimated expiration date is 2031.

These provisional, pending, or issued patents include composition of matter and/or method of use claims.

## **Table of Contents**

As noted above, patent coverage on rimiducid, the dimerization molecule AP1903, will expire in the U.S. in 2016. However, we believe that additional barriers to entry exist for a competitor attempting to use rimiducid after patent expiration. This is significant because, if true, then potential competitors will not be able to use the abbreviated new drug application pathway for approval of rimiducid. With respect to our investigational products, the FDA has assigned combination product status to BPX-501, and we plan to submit a biological license application, or BLA, for the combination product. We believe that this will be the case for each future product candidate of ours that incorporates rimiducid. If our investigational products incorporating rimiducid receive FDA approval through BLAs, then the FDA would not approve any biosimilar of these combination products until at least 12 years from the date that we receive FDA approval. Additionally, although biosimilar provisions exist for products approved through BLAs, it is not clear if the FDA will permit the biosimilar route to be used for complex biological products such as our investigational products.

Rimiducid is a relatively complex drug substance to manufacture. We have substantial experience in manufacturing of rimiducid and in preparing it for patient infusion. Our manufacturing know-how is a valuable asset and we incorporate contractual confidentiality terms in all agreements with our third party manufacturers. We believe that a competitor will face substantial obstacles with respect to time and cost in order to derive a clinically acceptable manufacturing process.

Our strategy is also to develop and obtain additional intellectual property covering manufacturing processes and methods for genetically engineering T cells expressing new constructs. To support this effort, we have established expertise and development capabilities focused in the areas of preclinical research and development, manufacturing and manufacturing process scale-up, quality control, quality assurance, product delivery and storage, regulatory affairs and clinical trial design and implementation. As appropriate, we expect to file additional patent applications to expand this layer of our intellectual property estate.

The term of individual patents depends upon the legal term of the patents in the countries in which they are obtained. In most countries in which we file, the patent term is 20 years from the date of filing of the first non-provisional application to which priority is claimed. In the United States, a patent's term may be lengthened by patent term adjustment, which compensates a patentee for administrative delays by the U.S. Patent and Trademark Office in granting a patent, or may be shortened if a patent is terminally disclaimed over an earlier-filed patent. The term of a patent that covers an FDA-approved drug or biologic may also be eligible for a patent term restoration of up to five years under the Hatch-Waxman Act, which is designed to compensate for the patent term lost during the FDA regulatory review process. The length of the patent term restoration is calculated based on the length of time the drug or biologic is under regulatory review. A patent term restoration under the Hatch-Waxman Act cannot extend the remaining term of a patent beyond a total of 14 years from the date of product approval and only one patent applicable to an approved drug or biologic may be restored. Moreover, a patent can only be restored once, and thus, if a single patent is applicable to multiple products, it can only be extended based on one product. Similar provisions are available in Europe and certain other foreign jurisdictions to extend the term of a patent that covers an approved drug or biologic. When possible, depending upon the length of clinical trials and other factors involved in the filing of a BLA we expect to apply for patent term extensions for patents covering our product candidates and their methods of use.

We may rely, in some circumstances, on trade secrets to protect our technology. We seek to protect our proprietary technology and processes, in part, by entering into confidentiality agreements with our employees, consultants, scientific advisors and contractors. We also seek to preserve the integrity and confidentiality of our data and trade secrets by maintaining physical security of our premises and physical and electronic security of our information technology systems. While we have confidence in these individuals, organizations and systems, agreements or security measures may be breached, and we may not have adequate remedies for any breach. In addition, our trade

secrets may otherwise become known or be independently discovered by competitors. To the extent that our consultants, contractors or collaborators use intellectual property owned by others in their work for us, disputes may arise as to the rights in related or resulting know-how and inventions.

## Table of Contents

### **Our License Agreements**

#### *License Agreement with ARIAD Pharmaceuticals, Inc.*

##### *2011 License Agreement*

On March 7, 2011, we entered into an amended and restated exclusive license agreement, or restated ARIAD license, with ARIAD which restated a license agreement entered into in 2006. Under the restated ARIAD license, ARIAD granted to us an exclusive (even as to the ARIAD) license, with the right to grant sublicenses, under ARIAD's patent rights relating to dimerizers, genetic constructs coding for dimerizer binding domains, vectors containing said constructs, cells containing said constructs and methods of inducing biological processes in cells containing said constructs. These licensed patent rights were limited in the 2011 restated license to defined products in the fields of cell transplantation and certain types of cancer.

In connection with the initial license, in 2006, we issued 121,242 shares of our common stock to ARIAD which were subject to antidilution protection that ultimately resulted in additional issuances to ARIAD by us of 556,221 shares of our common stock, such that ARIAD received a total of 677,463 shares of our common stock under the license agreement. In addition, we paid ARIAD a license fee of \$250,000 in connection with the restatement in 2011. The restated ARIAD license also provided for certain royalty and milestone payments, which were subsequently terminated pursuant to an omnibus amendment agreement with ARIAD (see below).

Under the restated ARIAD license, we are required to diligently proceed with the development, manufacture and sale of licensed products. The restated ARIAD license is subject at all times to restrictions and obligations under a license agreement by and between ARIAD Gene Therapeutics, Inc. (one of ARIAD's affiliates which merged into ARIAD) and the academic institution from which ARIAD obtained its license to the underlying technology. While we are not required to pay royalties or fees to such academic institution, no sublicensee of ours may enter into a sublicense with respect to any intellectual property owned by the academic institution without its consent, which terms must be consistent with those included in the agreement between ARIAD and such academic institution.

The restated ARIAD license will expire upon expiration of the last license term of a licensed product covered by the agreement, which is the later of (1) 12 years from the date of the first commercial sale of the licensed product, or (2) the expiration of the last to expire valid patent claim on the licensed product. Either party to the license may terminate or modify the restated ARIAD license upon a material breach by the other party that remains uncured following the date that is 30 days after written notice of a payment breach and 90 days for any other breach, and effective immediately upon bankruptcy of the other party. We may terminate the restated ARIAD license in our sole discretion at any time if we determine not to develop or commercialize any licensed product. In addition, upon termination of the restated ARIAD license prior to expiration, we must transfer any ownership and any beneficial ownership in any orphan drug designation or any similar designation in any jurisdiction of orphan drug status of the ARIAD dimerizer to ARIAD.

##### *2014 Amendment*

In September 2014, we entered into an omnibus amendment agreement with ARIAD, which in part amended the restated ARIAD license to expand the license to cover a broader scope of dimerizers and licensed products for use and exploitation in any human therapeutic field of use other than *in vivo* administration of genetic material directly into a human being using viral vectors for the purpose of producing proteins or other macromolecules that are expressed or secreted for therapeutic or prophylactic purposes.

In connection with the amendment, we made an initial payment of \$15,000,000 and we issued a promissory note to ARIAD for a principal amount of \$35,000,000 in return for the broader scope of the license and the termination of all obligations to make milestone and royalty payments to ARIAD in the future. The principal does not accrue interest unless we are in default, in which case it accrues at a rate of 10% per annum. We are required to pay \$20,000,000 in a lump sum installment on or before June 30, 2015 and to pay \$15,000,000 in a second lump sum installment on the date that is nine months following the closing of this offering. If we undergo a change of control while the note is outstanding, all remaining principal becomes due and payable upon the closing of such change of control. The two installment payments are also accelerated in the event that we raise predetermined amounts through public offerings of our securities. When we make the first installment payment, provided it is not later than December 31, 2015, ARIAD must return to us all of its 677,463 shares of our common stock and all of the agreements related to ARIAD's rights as a stockholder of us will terminate. If we fail to make either of the installment payments on the applicable due date, 50% of any funds we raise in a debt or equity financing will be applied against such past due installment payments.

## **Table of Contents**

ARIAD may terminate the restated ARIAD license, upon notice to us, if we do not make the first installment payment referred to above on or before June 30, 2016 or both of the installment payments referred to above on or prior to June 30, 2017.

### ***License Agreements with Baylor College of Medicine***

#### ***2008 Baylor License Agreement***

Pursuant to an Exclusive License Agreement with Baylor College of Medicine, or Baylor, dated March 20, 2008, or the 2008 Baylor license agreement, we obtained an exclusive, worldwide and fully paid up license to certain intellectual property, including intellectual property related to methods for activating antigen presenting cells and to genetic constructs coding for membrane bound inducible cytoplasmic CD40.

As consideration for the 2008 Baylor license agreement, we issued to Baylor 23,529 shares of our common stock and assumed responsibility for all legal fees and expenses, filing or maintenance fees, assessments and all other costs and expenses related to prosecuting, obtaining and maintaining patent protection on the patents subject to the 2008 Baylor license agreement.

The 2008 Baylor license agreement is subject to certain restrictions and is nonexclusive with respect to (1) the making or use of the licensed intellectual property for use in non-commercial research, patient care, teaching, and other educational purposes; (2) any non-exclusive license covering the licensed intellectual property that Baylor grants to other academic or research institutions for noncommercial research purposes; (3) any non exclusive licenses that Baylor is required to grant to the U.S. or foreign state pursuant to an existing or future treaty with the U.S.; and (4) a non-exclusive license granted to ARIAD Pharmaceuticals, Inc. under the terms of a materials transfer agreement between Baylor and ARIAD.

Baylor may terminate or modify the 2008 Baylor license agreement in the event of a material breach that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. We may terminate the 2008 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 30 days written notice to Baylor. Upon termination of the 2008 Baylor license agreement, all rights to the intellectual property immediately revert to Baylor.

#### ***2010 Baylor License Agreement***

Pursuant to an Exclusive License Agreement with Baylor, dated June 27, 2010, or the 2010 Baylor license agreement, we obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for treating prostate cancer, methods of administering T cells to a patient, and methods of activating antigen presenting cells with constructs comprising MyD88 and CD40.

Pursuant to the terms of the 2010 Baylor license agreement, we paid Baylor a license execution fee of \$30,000. In addition, we are required to pay a low annual maintenance fee on each anniversary of the agreement date.

The terms of the 2010 Baylor license agreement also require us to make royalty payments of less than one percent, subject to certain annual minimums, on net sales of products covered by the license. In addition, to the extent we enter into a sublicensing agreement relating to a licensed product, we are required to pay Baylor a percentage in the mid-single digits on all non-royalty income received from sublicensing revenue. We are required to make milestone payments, of up to \$735,000 in aggregate, upon successful completion of clinical and regulatory milestones regarding

the first two products covered by this license.

The 2010 Baylor license agreement will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which we will have a perpetual, paid-in-full license in such country. Baylor may terminate or modify the 2010 Baylor license agreement in the event of a material breach by us that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. We may terminate the 2010 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 60 days written notice to Baylor. Upon termination of the 2010 Baylor license agreement for any reason prior to expiration, we must assign to Baylor each authorized sublicense agreement that is currently in effect on the date of termination.

## **Table of Contents**

### ***2014 Baylor License Agreement***

Pursuant to an Exclusive License Agreement with Baylor, effective November 1, 2014, or the 2014 Baylor license agreement, we obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for inducing selective apoptosis.

Pursuant to the terms of the 2014 Baylor license agreement, we paid Baylor a license execution fee of \$25,000. In addition, we are required to pay Baylor a low annual maintenance fee on each anniversary of the agreement date. The terms of the 2014 Baylor license agreement also require us to make royalty payments in the low single digits, subject to certain annual minimums, on net sales of products covered by the license. To the extent we enter into a sublicensing agreement relating to a licensed product, we are also required to pay Baylor a percentage in the low double-digits on all non-royalty income received from sublicensing revenue. We are required to make milestone payments, of up to \$275,000 in aggregate, upon successful completion of clinical and regulatory milestones regarding the first product covered by this license. The 2014 Baylor license agreement will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which we will have a perpetual, paid-in-full license in each such country.

Baylor may terminate or modify the 2014 Baylor license agreement in the event of a material breach by us that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. We may terminate the 2014 Baylor license agreement, or any portion thereof, at our sole discretion at any time upon 60 days' written notice to Baylor.

### **Grant Agreement**

#### ***Grant Agreement with Cancer Prevention and Research Institute of Texas***

On July 27, 2011, we entered into a Cancer Research Grant Contract, or the Grant Contract, with the Cancer Prevention and Research Institute of Texas, or CPRIT, under which CPRIT awarded a grant not to exceed approximately \$5.7 million to be used by us for the execution of defined clinical development of BPX-501. In addition, CPRIT may award supplemental funding not to exceed ten percent of the total grant amount based upon our progress. To date, we have received approximately \$4.0 million under the grant. The Grant Contract terminated on June 30, 2014, but obligations exist as to licensing, royalty payments, and indemnification provisions.

Pursuant to the Grant Contract, we granted the CPRIT with a non-exclusive, irrevocable, royalty-free, perpetual, worldwide license to the intellectual property facilitated by the Grant Contract for and on behalf of CPRIT and other governmental entities and agencies of the State of Texas for education, research and other non-commercial purposes only.

The terms of the Grant Contract require that we pay tiered royalties in the low- to mid-single digit percentages on revenues from sales and licenses of intellectual property facilitated by the Grant Contract. If a third party acquires substantially all of our assets, we have the option to buyout from the royalty obligations by paying a buyout amount that is equal to a percentage of the net grant award proceeds received by us under the Grant Contract, less the aggregate amount of all royalties paid at the time of the buyout. The applicable percentage depends on the timing of the buyout and ranges from 125% to 200%.

We are required to use diligent and commercially reasonable efforts to commercialize or otherwise bring to practical application the results of the funded clinical trial. If CPRIT notifies us of our failure to (1) make the required effort to



commercialize any product covered by this agreement or (2) perform our obligations with respect to protection of intellectual property, the rights to any intellectual property and proprietary and confidential information may, at CPRIT's option, revert to CPRIT and CPRIT, at its own cost, can take over the prosecution and maintenance of any impacted patents and commercialize such product candidate. CPRIT's option is subject to our ability to cure any failures identified by CPRIT within 30 days.

### **Competition**

The biopharmaceutical industry is characterized by intense and dynamic competition to develop new technologies and proprietary therapies. Any product candidates that we successfully develop and commercialize will have to

## **Table of Contents**

compete with existing therapies and new therapies that may become available in the future. While we believe that our proprietary CID platform, differentiated product candidates and scientific expertise in the field of cellular immunotherapy provide us with competitive advantages, we face potential competition from various sources, including larger and better-funded pharmaceutical, specialty pharmaceutical and biotechnology companies, as well as from academic institutions, governmental agencies and public and private research institutions.

Our lead product candidate, BPX-501, is an adjunct therapy for HSCT with alternative donors that potentially improves stem cell engraftment, accelerates host immune system recovery and treats GvHD. The current standard-of-care that addresses some of the safety challenges associated with HSCT, primarily GvHD, is high-dose steroids. We are aware of other companies that are developing product candidates to improve the outcome of HSCT, including Kiadis Pharma Netherlands B.V. and Molecular Medicine S.p.A.

T-cell based treatments for cancer, such as CAR-T and TCR therapies, have recently been an area of significant research and development by academic institutions and biopharmaceutical companies. BPX-401, BPX-601 and BPX-701 based on our CIDeCAR and Go-CART technologies will compete with product candidates from a number of companies that are currently focused on this therapeutic modality, including Adaptimmune Limited, bluebird bio, Inc., Celgene Corporation, Cellectis SA, GlaxoSmithKline plc, Intrexon Corporation, Juno Therapeutics, Inc., Kite Pharma, Inc., Novartis AG and Pfizer Inc.

BPX-201 based on our DeCIDE technology is a dendritic cell-based cancer vaccine for the treatment of metastatic prostate cancer and other solid tumors. PROVENGE<sup>®</sup>, marketed by Dendreon Corporation, is the first approved cancer vaccine for the treatment of mCRPC. We are aware of other companies focused on developing cancer vaccines, including Advaxis, Inc., Argos Therapeutics, Inc., Biovest International, Inc., ImmunoCellular Therapeutics, Ltd., Immune Design, Inc., Inovio Pharmaceuticals, Inc., Intrexon Corporation and Northwest Biotherapeutics, Inc.

Many of our competitors, either alone or with their strategic partners, have substantially greater financial, technical and human resources than we do and significantly greater experience in the discovery and development of product candidates, obtaining FDA and other regulatory approvals of treatments and commercializing those treatments. Accordingly, our competitors may be more successful than us in obtaining approval for treatments and achieving widespread market acceptance. Our competitors' treatments may be more effective, or more effectively marketed and sold, than any treatment we may commercialize and may render our treatments obsolete or non-competitive before we can recover the expenses of developing and commercializing any of our treatments.

Mergers and acquisitions in the biotechnology and pharmaceutical industries may result in even more resources being concentrated among a smaller number of our competitors. These competitors also compete with us in recruiting and retaining qualified scientific and management personnel and establishing clinical study sites and subject registration for clinical studies, as well as in acquiring technologies complementary to, or necessary for, our programs. Smaller or early-stage companies may also prove to be significant competitors, particularly through collaborative arrangements with large and established companies.

We anticipate that we will face intense and increasing competition as new drugs enter the market and advanced technologies become available. We expect any treatments that we develop and commercialize to compete on the basis of, among other things, efficacy, safety, convenience of administration and delivery, price, the level of generic competition and the availability of reimbursement from government and other third-party payers. For example, if a third party is able to obtain a stand-alone new drug application for rimiducid, then potential generic manufacturers may be able to file abbreviated new drug applications for such product.

Our commercial opportunity could be reduced or eliminated if our competitors develop and commercialize products that are safer, more effective, have fewer or less severe side effects, are more convenient or are less expensive than any products that we may develop. Our competitors also may obtain FDA or other regulatory approval for their products more rapidly than we may obtain approval for ours, which could result in our competitors establishing a strong market position before we are able to enter the market. In addition, we expect that our therapeutic products, if approved, will be priced at a significant premium over competitive generic products and our ability to compete may be affected in many cases by insurers or other third-party payers seeking to encourage the use of generic products.

## **Table of Contents**

### **Government Regulation and Product Approval**

As a biopharmaceutical company that operates in the United States, we are subject to extensive regulation. Our cell products will be regulated as biologics. With this classification, commercial production of our products will need to occur in registered and licensed facilities in compliance with cGMP for biologics.

The FDA regulates human cells, tissues, and cellular and tissue-based products, or HCT/Ps, under a two-tiered framework, based on risk categorization. Higher risk HCT/Ps are regulated as biologics. Manufacturers of biologics are subject to extensive government regulation. For example, such products must complete extensive clinical trials, which must be conducted pursuant to an effective investigational new drug application, or IND. The FDA must review and approve a BLA before a new biologic may be marketed.

FDA considers our investigational products to be combination products because our products involve a biologic (the engineered cells) that is intended to be used with a small molecule chemical drug (AP1903, licensed from ARIAD). In general, biologics such as our engineered cells are regulated through FDA's Center for Biologics Evaluation and Research, or CBER, while synthetic drugs are regulated through FDA's Center for Drug Evaluation and Research, or CDER. When FDA encounters a combination product such as our products, the agency determines which of the two centers will have primary responsibility for regulating the product by determining the primary mode of action for the product. In this case, we believe that the cellular component of the combination contributes the primary mode of action and, as a result, that FDA will regulate our investigational products as biologics, through CBER.

Government authorities in the United States (at the federal, state and local level) and in other countries extensively regulate, among other things, the research, development, testing, manufacturing, quality control, approval, labeling, packaging, storage, record-keeping, promotion, advertising, distribution, post-approval monitoring and reporting, marketing and export and import of biopharmaceutical products such as those we are developing. Our product candidates must be approved by the FDA before they may be legally marketed in the United States and by the appropriate foreign regulatory agency before they may be legally marketed in foreign countries. Generally, our activities in other countries will be subject to regulation that is similar in nature and scope as that imposed in the United States, although there can be important differences. Additionally, some significant aspects of regulation in Europe are addressed in a centralized way, but country-specific regulation remains essential in many respects. The process for obtaining regulatory marketing approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources.

#### *U.S. Product Development Process*

In the United States, the FDA regulates new drugs and biological products under the Federal Food, Drug and Cosmetic Act, or FDCA; the Public Health Service Act, or PHSA; and implementing regulations. Products are also subject to other federal, state and local statutes and regulations. The process of obtaining regulatory approvals and the subsequent compliance with appropriate federal, state, local and foreign statutes and regulations require the expenditure of substantial time and financial resources. Failure to comply with the applicable U.S. requirements at any time during the product development process, approval process or after approval, may subject an applicant to administrative, criminal, or civil sanctions. FDA sanctions could include, among other actions, refusal to approve pending applications, withdrawal of an approval, a clinical hold, warning letters, product recalls or withdrawals from the market, product seizures, total or partial suspension of production or distribution injunctions, fines, refusals of government contracts, restitution, disgorgement or civil or criminal penalties. Any administrative, criminal, or civil enforcement action could have a material adverse effect on us. The FDA has limited experience with commercial development of T cell therapies for cancer. The process required by the FDA before a biological product may be marketed in the United States generally involves the following:

- n completion of nonclinical laboratory tests and animal studies according to good laboratory practices, or GLPs, and applicable requirements for the humane use of laboratory animals or other applicable regulations;
- n submission to the FDA of an IND, which must become effective before human clinical trials may begin;
- n performance of adequate and well-controlled human clinical trials according to the FDA's regulations commonly referred to as good clinical practices, or GCPs, and any additional requirements for the protection

---

**Table of Contents**

of human research patients and their health information, to establish the safety and efficacy of the proposed biological product for its intended use;

- n submission to the FDA of a BLA for marketing approval that includes substantial evidence of safety, purity, and potency from results of nonclinical testing and clinical trials;
- n satisfactory completion of an FDA inspection of the manufacturing facility or facilities where the biological product is produced to assess compliance with cGMP, to assure that the facilities, methods and controls are adequate to preserve the biological product's identity, strength, quality and purity and, if applicable, the FDA's current good tissue practices, or GTPs, for the use of HCT/PS;
- n potential FDA audit of the nonclinical study and clinical trial sites that generated the data in support of the BLA; and
- n FDA review and approval, or licensure, of the BLA.

Before testing any biological product candidate, including our product candidates, in humans, the product candidate enters the preclinical testing stage. Preclinical tests, also referred to as nonclinical studies, include laboratory evaluations of product chemistry, toxicity and formulation, as well as animal studies to assess the potential safety and activity of the product candidate. The conduct of the preclinical tests must comply with federal regulations and requirements including GLPs. The clinical trial sponsor must submit the results of the preclinical tests, together with manufacturing information, analytical data, any available clinical data or literature and a proposed clinical protocol, to the FDA as part of the IND. Some preclinical testing may continue even after the IND is submitted. The IND automatically becomes effective 30 days after receipt by the FDA, unless the FDA raises concerns or questions regarding the proposed clinical trials and places the trial on a clinical hold within that 30-day time period. In such a case, the IND sponsor must resolve FDA's outstanding concerns before the clinical trial can begin. The FDA may also impose clinical holds on a biological product candidate at any time before or during clinical trials due to safety concerns or non-compliance. If the FDA imposes a clinical hold, trials may not recommence without FDA authorization and then only under terms authorized by the FDA. Accordingly, we cannot be sure that submission of an IND will result in the FDA allowing clinical trials to begin, or that, once begun, issues will not arise that suspend or terminate such clinical trials.

Clinical trials involve the administration of the biological product candidate to healthy volunteers or patients under the supervision of qualified investigators, generally physicians not employed by or under the trial sponsor's control. Clinical trials are conducted under protocols detailing, among other things, the objectives of the clinical trial, dosing procedures, subject selection and exclusion criteria, and the parameters to be used to monitor subject safety, including stopping rules that assure a clinical trial will be stopped if certain adverse events should occur. Each protocol and any amendments to the protocol must be submitted to the FDA as part of the IND. Clinical trials must be conducted and monitored in accordance with the FDA's regulations comprising the GCP requirements, including the requirement that all research patients provide informed consent. Further, each clinical trial must be reviewed and approved by an institutional review board, or IRB, at or servicing each institution at which the clinical trial will be conducted. An IRB is independent from the trial sponsor and is charged with protecting the welfare and rights of clinical trial participants and considers such items as whether the risks to individuals participating in the clinical trials are minimized and are reasonable in relation to anticipated benefits. The IRB also approves the form and content of the informed consent that must be signed by each clinical trial subject or his or her legal representative and must monitor the clinical trial until

completed. Clinical trials also must be reviewed by an institutional biosafety committee, or IBC, a local institutional committee that reviews and oversees basic and clinical research conducted at that institution. The IBC assesses the safety of the research and identifies any potential risk to public health or the environment.

Human clinical trials for biologic products are typically conducted in three sequential phases that may overlap or be combined:

- n *Phase 1.* The biological product is initially introduced into healthy human subjects and tested for safety. In the case of some products for severe or life-threatening diseases, especially when the product may be too inherently toxic to ethically administer to healthy volunteers, the initial human testing is often conducted in patients.

**Table of Contents**

n *Phase 2.* The biological product is evaluated in a limited patient population to identify possible adverse effects and safety risks, to preliminarily evaluate the efficacy of the product for specific targeted diseases and to determine dosage tolerance, optimal dosage and dosing schedule.

n *Phase 3.* Clinical trials are undertaken to further evaluate dosage, clinical efficacy, potency, and safety in an expanded patient population at geographically dispersed clinical trial sites. These clinical trials are intended to establish the overall risk to benefit ratio of the product and provide an adequate basis for product labeling.

Post-approval clinical trials, sometimes referred to as Phase 4 clinical trials, may be conducted after initial marketing approval. These clinical trials are used to gain additional experience from the treatment of patients in the intended therapeutic indication, particularly for long-term safety follow-up.

During all phases of clinical development, regulatory agencies require extensive monitoring and auditing of all clinical activities, clinical data, and clinical trial investigators. Annual progress reports detailing the progress of the clinical trials must be submitted to the FDA. Written IND safety reports must be promptly submitted to the FDA, the NIH and the investigators for serious and unexpected adverse events, any findings from other studies, tests in laboratory animals or *in vitro* testing that suggest a significant risk for human patients, or any clinically important increase in the rate of a serious suspected adverse reaction over that listed in the protocol or investigator brochure. The sponsor must submit an IND safety report within 15 calendar days after the sponsor determines that the information qualifies for reporting. The sponsor also must notify the FDA of any unexpected fatal or life-threatening suspected adverse reaction within seven calendar days after the sponsor's initial receipt of the information. Phase 1, Phase 2 and Phase 3 clinical trials may not be completed successfully within any specified period, if at all. The FDA or the sponsor or its data safety monitoring board may suspend or terminate a clinical trial at any time on various grounds, including a finding that the research patients are being exposed to an unacceptable health risk, including risks inferred from other unrelated immunotherapy trials. Similarly, an IRB can suspend or terminate approval of a clinical trial at its institution if the clinical trial is not being conducted in accordance with the IRB's requirements or if the biological product has been associated with unexpected serious harm to patients.

Human immunotherapy products are a new category of therapeutics. Because this is a relatively new and expanding area of novel therapeutic interventions, there can be no assurance as to the length of the trial period, the number of patients the FDA will require to be enrolled in the clinical trials in order to establish the safety, efficacy, purity and potency of immunotherapy products, or that the data generated in these clinical trials will be acceptable to the FDA to support marketing approval.

Concurrently with clinical trials, companies usually complete additional studies and must also develop additional information about the physical characteristics of the biological product as well as finalize a process for manufacturing the product in commercial quantities in accordance with cGMP requirements. To help reduce the risk of the introduction of adventitious agents with use of biological products, the PHSA emphasizes the importance of manufacturing control for products whose attributes cannot be precisely defined. The manufacturing process must be capable of consistently producing quality batches of the product candidate and, among other things, the sponsor must develop methods for testing the identity, strength, quality, potency and purity of the final biological product. Additionally, appropriate packaging must be selected and tested and stability studies must be conducted to demonstrate that the biological product candidate does not undergo unacceptable deterioration over its shelf life.

Federal law requires that we register all of our clinical trials on a publicly accessible website. We must also provide results information for most of our clinical trials, other than Phase 1 clinical trials.

*U.S. Review and Approval Processes*



After the completion of clinical trials of a biological product, FDA approval of a BLA must be obtained before commercial marketing of the biological product. The BLA must include results of product development, laboratory and animal studies, human trials, information on the manufacture and composition of the product, proposed labeling and other relevant information. The FDA may grant deferrals for submission of certain data or full or partial waivers. The testing and approval processes require substantial time and effort and there can be no assurance that the FDA will accept the BLA for filing and, even if filed, that any approval will be granted on a timely basis, if at all.

## **Table of Contents**

Under the Prescription Drug User Fee Act, or PDUFA, as amended, each BLA must be accompanied by a significant user fee. The FDA adjusts the PDUFA user fees on an annual basis. PDUFA also imposes an annual product fee for biological products and an annual establishment fee on facilities used to manufacture prescription biological products. Fee waivers or reductions are available in certain circumstances, including a waiver of the application fee for the first application filed by a small business. Additionally, no user fees are assessed on BLAs for products designated as orphan drugs, unless the application also includes a non-orphan indication.

Within 60 days following submission of the application, the FDA reviews a BLA submitted to determine if it is substantially complete before the agency accepts it for filing. The FDA may refuse to file any BLA that it deems incomplete or not properly reviewable at the time of submission and may request additional information. In this event, the BLA must be resubmitted with the additional information. The resubmitted application also is subject to review before the FDA accepts it for filing. Once the submission is accepted for filing, the FDA begins an in-depth substantive review of the BLA. The FDA reviews the BLA to determine, among other things, whether the proposed product is safe, potent, and/or effective for its intended use, and has an acceptable purity profile, and whether the product is being manufactured in accordance with cGMP to assure and preserve the product's identity, safety, strength, quality, potency and purity. The FDA may refer applications for novel biological products or biological products that present difficult questions of safety or efficacy to an advisory committee, typically a panel that includes clinicians and other experts, for review, evaluation and a recommendation as to whether the application should be approved and under what conditions. The FDA is not bound by the recommendations of an advisory committee, but it considers such recommendations carefully when making decisions. During the biological product approval process, the FDA also will determine whether a Risk Evaluation and Mitigation Strategy, or REMS, is necessary to assure the safe use of the biological product. If the FDA concludes a REMS is needed, the sponsor of the BLA must submit a proposed REMS. The FDA will not approve a BLA without a REMS, if required.

Before approving a BLA, the FDA will inspect the facilities at which the product is manufactured. The FDA will not approve the product unless it determines that the manufacturing processes and facilities are in compliance with cGMP requirements and adequate to assure consistent production of the product within required specifications. For immunotherapy products, the FDA also will not approve the product if the manufacturer is not in compliance with the GTPs, to the extent applicable. These are FDA regulations and guidance documents that govern the methods used in, and the facilities and controls used for, the manufacture of HCT/Ps. The primary intent of the GTP requirements is to ensure that cell and tissue based products are manufactured in a manner designed to prevent the introduction, transmission and spread of communicable disease. FDA regulations also require HCT/P establishments to register and list their HCT/Ps with the FDA and, when applicable, to evaluate donors through screening and testing. Additionally, before approving a BLA, the FDA will typically inspect one or more clinical sites to assure that the clinical trials were conducted in compliance with IND trial requirements and GCP requirements. To maintain compliance with cGMPs, GTPs, and GCPs, an applicant must incur significant expenditure of time, money and effort in the areas of training, record keeping, production, and quality control.

Notwithstanding the submission of relevant data and information, the FDA may ultimately decide that the BLA does not satisfy its regulatory criteria for approval and deny approval. Data obtained from clinical trials are not always conclusive and the FDA may interpret data differently than we interpret the same data. If the agency decides not to approve the BLA in its present form, the FDA will issue a complete response letter that describes all of the specific deficiencies in the BLA identified by the FDA. The deficiencies identified may be minor, for example, requiring labeling changes, or major, for example, requiring additional clinical trials. Additionally, the complete response letter may include recommended actions that the applicant might take to place the application in a condition for approval. If a complete response letter is issued, the applicant may either resubmit the BLA, addressing all of the deficiencies identified in the letter, or withdraw the application.

If a product receives regulatory approval, the approval may be significantly limited to specific diseases and dosages or the indications for use may otherwise be limited, which could restrict the commercial value of the product.

Further, the FDA may require that certain contraindications, warnings or precautions be included in the product labeling. The FDA may impose restrictions and conditions on product distribution, prescribing, or dispensing in the form of a REMS or other risk management plan, or otherwise limit the scope of any approval. In addition, the FDA

## **Table of Contents**

may require post marketing clinical trials, sometimes referred to as Phase 4 clinical trials, designed to further assess a biological product's safety and effectiveness, and testing and surveillance programs to monitor the safety of approved products that have been commercialized.

In addition, under the Pediatric Research Equity Act, or PREA, a BLA or supplement to a BLA must contain data to assess the safety and effectiveness of the product for the claimed indications in all relevant pediatric subpopulations and to support dosing and administration for each pediatric subpopulation for which the product is safe and effective. The FDA may grant deferrals for submission of data or full or partial waivers. Unless otherwise required by regulation, PREA does not apply to any product for an indication for which orphan designation has been granted. However, if only one indication for a product has orphan designation, a pediatric assessment may still be required for any applications to market that same product for the non-orphan indication(s). Sponsors in satisfaction of this obligation may receive an additional six months of marketing exclusivity for all dosage forms and all indications with the same active moiety as the drug studied.

### *Orphan Drug Designation*

Under the Orphan Drug Act, the FDA may grant orphan designation to a drug or biologic intended to treat a rare disease or condition, which is generally a disease or condition that affects fewer than 200,000 individuals in the United States, or more than 200,000 individuals in the United States and for which there is no reasonable expectation that the cost of developing and making available in the United States a drug or biologic for this type of disease or condition will be recovered from sales in the United States for that drug or biologic. Orphan drug designation must be requested before submitting a BLA. After the FDA grants orphan drug designation, the generic identity of the therapeutic agent and its potential orphan use are disclosed publicly by the FDA. The orphan drug designation does not shorten the duration of the regulatory review or approval process, but does provide certain advantages, such as a waiver of PDUFA fees, enhanced access to FDA staff, and potential waiver of PREA requirements discussed above.

If a product that has orphan drug designation subsequently receives the first FDA approval for the disease for which it has such designation, the product is entitled to orphan product exclusivity, which means that the FDA may not approve any other applications, including a full BLA, to market the same biologic for the same indication for seven years, except in limited circumstances, such as a showing of clinical superiority to the product with orphan drug exclusivity. Orphan drug exclusivity does not prevent FDA from approving a different drug or biologic for the same disease or condition, or the same drug or biologic for a different disease or condition. Among the other benefits of orphan drug designation are tax credits for certain research and a waiver of the BLA application user fee.

A designated orphan drug may not receive orphan drug exclusivity if it is approved for a use that is broader than the indication for which it received orphan designation. In addition, exclusive marketing rights in the United States may be lost if the FDA later determines that the request for designation was materially defective or if the manufacturer is unable to assure sufficient quantities of the product to meet the needs of patients with the rare disease or condition.

We are currently in discussions with FDA regarding orphan drug designation for our investigational products.

### *Expedited Development and Review Programs*

The FDA has a Fast Track program that is intended to expedite or facilitate the process for reviewing new products that meet certain criteria. Specifically, new products are eligible for Fast Track designation if they are intended to treat a serious or life-threatening disease or condition and demonstrate the potential to address unmet medical needs, or if the drug has been designated as a qualified infectious disease product. Fast Track designation applies to the combination of the product and the specific indication for which it is being studied. Under Fast Track, the FDA may

consider for review sections of the BLA on a rolling basis before the complete application is submitted, if the sponsor provides a schedule for the submission of the sections of the BLA, the FDA agrees to accept sections of the BLA and determines that the schedule is acceptable, and the sponsor pays any required user fees upon submission of the first section of the BLA. Even if Fast Track designation is granted, it may be rescinded if the product no longer meets the qualifying criteria.

Any product, submitted to the FDA for approval, including a product with a Fast Track designation, may also be eligible for other types of FDA programs intended to expedite development and review, such as priority review and accelerated approval. A product is eligible for priority review if it treats a serious condition and, if approved, would

## **Table of Contents**

provide a significant improvement in safety and effective. The FDA will attempt to direct additional resources to the evaluation of an application for a new product designated for priority review in an effort to facilitate the review. Additionally, a product may be eligible for accelerated approval. Products studied for their safety and effectiveness in treating serious or life-threatening diseases or conditions may receive accelerated approval upon a determination that the product treats a serious condition, provides a meaningful advantage over available therapies, and demonstrates an effect on a surrogate endpoint that is reasonably likely to predict clinical benefit, or on a clinical endpoint that can be measured earlier than irreversible morbidity or mortality, that is reasonably likely to predict an effect on irreversible morbidity or mortality or other clinical benefit, taking into account the severity, rarity, or prevalence of the condition and the availability or lack of alternative treatments. As a condition of approval, the FDA may require that a sponsor of a drug or biological product receiving accelerated approval perform appropriate post-marketing clinical studies to verify and describe the predicted effect on irreversible morbidity or mortality or other clinical benefit. In addition, the FDA currently requires as a condition for accelerated approval pre-approval of promotional materials, which could adversely impact the timing of the commercial launch of the product. The FDCA also provides expedited procedures for FDA withdrawal of approval of a product approved through accelerated approval. Fast Track designation, priority review and accelerated approval do not change the standards for approval but may expedite the development or approval process.

In 2012 the FDA established a Breakthrough Therapy Designation which is intended to expedite the development and review of products that treat serious or life-threatening conditions. The designation requires preliminary clinical evidence that may demonstrate substantial improvement on a clinically significant endpoint over available therapies. The designation includes all of the Fast Track program features, as well as more intensive FDA interaction and guidance, organizational commitment, and other potential actions to expedite review. The Breakthrough Therapy Designation is a distinct status from both accelerated approval and priority review, which can also be granted to the same product if relevant criteria are met. If a product is designated as breakthrough therapy, FDA will expedite the development and review of such product. Even if a Breakthrough Therapy Designation is granted, it may be rescinded if the product no longer meets the qualifying criteria.

Where applicable, we plan to request Fast Track and Breakthrough Therapy Designation for our product candidates, including BPX-051, BPX-401 and BPX-601. Even if we receive one or both of these designations for our product candidates, the FDA may later decide that our product candidates no longer meets the conditions for qualification. In addition, these designations may not provide us with a material commercial advantage.

### *Post-Approval Requirements*

Any product for which we receive FDA approval is subject to continuing regulation by the FDA, including, among other things, record-keeping requirements, reporting of adverse experiences with the product, providing the FDA with updated safety and efficacy information, product sampling and distribution requirements, and complying with FDA promotion and advertising requirements, which include, among others, standards for direct-to-consumer advertising, restrictions on promoting products for uses or in patient populations that are not described in the product's approved uses (known as off-label use), limitations on industry-sponsored scientific and educational activities, and requirements for promotional activities involving the internet. Although physicians may prescribe legally available products for off-label uses, if the physicians deem it to be appropriate in their professional medical judgment, manufacturers may not market or promote such off-label uses.

In addition, quality control and manufacturing procedures must continue to conform to applicable manufacturing requirements after approval to ensure the long-term stability of the product. We rely, and expect to continue to rely, on third parties for the production of clinical and commercial quantities of our products in accordance with cGMP regulations. cGMP regulations require among other things, quality control and quality assurance as well as the

corresponding maintenance of records and documentation and the obligation to investigate and correct any deviations from cGMP. Manufacturers and other entities involved in the manufacture and distribution of approved products are required to register their establishments with the FDA and certain state agencies, and are subject to periodic unannounced inspections by the FDA and certain state agencies for compliance with cGMP and other laws. Accordingly, manufacturers must continue to expend time, money, and effort in the area of production and quality control to maintain cGMP compliance. Discovery of problems with a product after approval may result in restrictions on a product, manufacturer, or holder of an approved BLA, including, among other things, recall or withdrawal of the product from the market, seizure of product manufactured not in accordance with GMPs, suspension or termination

---

**Table of Contents**

of manufacturing activities at one or more facilities, or other civil or criminal sanctions. In addition, changes to the manufacturing process are strictly regulated, and depending on the significance of the change, may require prior FDA approval before being implemented. Other types of changes to the approved product, such as adding new indications and claims, are also subject to further FDA review and approval.

The FDA also may require post-marketing testing, known as Phase 4 testing, and surveillance to monitor the effects of an approved product. Discovery of previously unknown problems with a product or the failure to comply with applicable FDA requirements can have negative consequences, including adverse publicity, judicial or administrative enforcement, warning letters from the FDA, mandated corrective advertising or communications with doctors, and civil or criminal penalties, among others. Newly discovered or developed safety or effectiveness data may require changes to a product's approved labeling, including the addition of new warnings and contraindications, and also may require the implementation of a REMS or other risk management measures. Also, new government requirements, including those resulting from new legislation, may be established, or the FDA's policies may change, which could delay or prevent regulatory approval of our products under development.

*U.S. Patent Term Restoration and Marketing Exclusivity*

The Biologics Price Competition and Innovation Act, or BPCIA, amended the PHS Act to authorize the FDA to approve similar versions of innovative biologics, commonly known as biosimilars. Among other requirements, a competitor seeking approval of a biosimilar must file an application to establish its molecule as highly similar to an approved innovator biologic, without any clinically meaningful differences in terms of safety, purity, and potency. The BPCIA, however, bars the FDA from approving biosimilar applications for 12 years after an innovator biological product receives initial marketing approval. This 12-year period of data exclusivity may be extended by six months, for a total of 12.5 years, if the FDA requests that the innovator company conduct pediatric clinical investigations of the product. Although a statutory provision exists for FDA approval of biosimilars, FDA has yet to provide clarity on many aspects of the regulatory pathway for such products. Furthermore, the first biosimilar applications have only recently been submitted to FDA, and it remains to be seen how FDA will apply the statutory biosimilar provisions to biological products such as ours.

Depending upon the timing, duration and specifics of the FDA approval of the use of our product candidates, some of our U.S. patents, if granted, may be eligible for limited patent term extension under the Drug Price Competition and Patent Term Restoration Act of 1984, commonly referred to as the Hatch-Waxman Act. The Hatch-Waxman Act permits a patent restoration term of up to five years, as compensation for patent term lost during product development and the FDA regulatory review process. However, patent term restoration cannot extend the remaining term of a patent beyond a total of 14 years from the product's approval date. The patent term restoration period is generally one-half the time between the effective date of an IND and the submission date of a BLA plus the time between the submission date of a BLA and the approval of that application. Only one patent applicable to an approved product is eligible for the extension and the application for the extension must be submitted prior to the expiration of the patent. The U.S. Patent and Trademark Office, in consultation with the FDA, reviews and approves the application for any patent term extension or restoration. In the future, we may intend to apply for restoration of patent term for one of our currently owned or licensed patents to add patent life beyond its current expiration date, depending on the expected length of the clinical trials and other factors involved in the filing of the relevant BLA.

*Other U.S. Healthcare Laws and Compliance Requirements*

In the United States, our activities are potentially subject to regulation by various federal, state and local authorities in addition to the FDA, including but not limited to, the Centers for Medicare and Medicaid Services, or CMS, other divisions of the U.S. Department of Health and Human Services (e.g., the Office of Inspector General), the U.S.



Department of Justice, or DOJ, and individual U.S. Attorney offices within the DOJ, and state and local governments. For example, sales, marketing and scientific/educational grant programs must comply with the anti-fraud and abuse provisions of the Social Security Act, the false claims laws, the privacy provisions of the Health Insurance Portability and Accountability Act, or HIPAA, the sunshine provisions of the Affordable Care Act, and similar state laws, each as amended.

The federal Anti-Kickback Statute prohibits, among other things, any person or entity, from knowingly and willfully offering, paying, soliciting or receiving any remuneration, directly or indirectly, overtly or covertly, in cash or in kind, to induce or in return for purchasing, leasing, ordering or arranging for the purchase, lease or order of any good,

## **Table of Contents**

facility, item or service reimbursable, in whole or in part, under Medicare, Medicaid or other federal healthcare programs. The term remuneration has been interpreted broadly to include anything of value. The Anti-Kickback Statute has been interpreted to apply to arrangements between biologic manufacturers on one hand and prescribers, purchasers, and formulary managers on the other. There are a number of statutory exceptions and regulatory safe harbors protecting some common activities from prosecution. The exceptions and safe harbors are drawn narrowly and practices that involve remuneration that may be alleged to be intended to induce prescribing, purchasing or recommending may be subject to scrutiny if they do not qualify for an exception or safe harbor. Failure to meet all of the requirements of a particular applicable statutory exception or regulatory safe harbor does not make the conduct per se illegal under the Anti-Kickback Statute. Instead, the legality of the arrangement will be evaluated on a case-by-case basis based on a cumulative review of all of its facts and circumstances.

Additionally, the intent standard under the Anti-Kickback Statute was amended by the Affordable Care Act to a stricter standard such that a person or entity no longer needs to have actual knowledge of the statute or specific intent to violate it in order to have committed a violation. In addition, the Affordable Care Act codified case law that a claim including items or services resulting from a violation of the federal Anti-Kickback Statute constitutes a false or fraudulent claim for purposes of the federal civil False Claims Act.

The civil monetary penalties statute imposes penalties against any person or entity who, among other things, is determined to have presented or caused to be presented a claim to a federal health program that the person knows or should know is for an item or service that was not provided as claimed or is false or fraudulent.

The federal false claims laws, including but not limited to the federal civil False Claims Act, prohibit, among other things, any person or entity from knowingly presenting, or causing to be presented, a false claim for payment to, or approval by, the federal government. Pharmaceutical and other healthcare companies have been prosecuted under these laws for allegedly providing free product to customers with the expectation that the customers would bill federal programs for the product. Other companies have been prosecuted for causing false claims to be submitted because of the companies' marketing of the product for unapproved (i.e., off-label), and thus non-reimbursable, uses.

HIPAA created new federal criminal statutes that prohibit knowingly and willfully executing, or attempting to execute, a scheme to defraud or to obtain, by means of false or fraudulent pretenses, representations or promises, any money or property owned by, or under the control or custody of, any healthcare benefit program, including private third-party payors and knowingly and willfully falsifying, concealing or covering up by trick, scheme or device, a material fact or making any materially false, fictitious or fraudulent statement in connection with the delivery of or payment for healthcare benefits, items or services.

Also, many states have similar fraud and abuse statutes or regulations that apply to items and services reimbursed under Medicaid and other state programs, or, in several states, apply regardless of the payor.

We may be subject to data privacy and security regulations by both the federal government and the states in which we conduct our business. HIPAA, as amended by the Health Information Technology for Economic and Clinical Health Act, or HITECH, and its implementing regulations, imposes requirements relating to the privacy, security and transmission of individually identifiable health information. Among other things, HITECH makes HIPAA's security standards directly applicable to business associates independent contractors or agents of covered entities that receive or obtain protected health information in connection with providing a service on behalf of a covered entity. HITECH also created four new tiers of civil monetary penalties, amended HIPAA to make civil and criminal penalties directly applicable to business associates, and gave state attorneys general new authority to file civil actions for damages or injunctions in federal courts to enforce the federal HIPAA laws and seek attorneys' fees and costs associated with pursuing federal civil actions. In addition, state laws govern the privacy and security of health information in specified

circumstances, many of which differ from each other in significant ways and may not have the same effect, thus complicating compliance efforts.

Additionally, the federal Physician Payments Sunshine Act within the Affordable Care Act, and its implementing regulations, require that certain manufacturers of drugs, devices, biological and medical supplies for which payment is available under Medicare, Medicaid or the Children's Health Insurance Program (with certain exceptions) to report

## **Table of Contents**

annually information related to certain payments or other transfers of value made or distributed to physicians and teaching hospitals, or to entities or individuals at the request of, or designated on behalf of, the physicians and teaching hospitals and to report annually certain ownership and investment interests held by physicians and their immediate family members. CMS published certain data reported by covered manufacturers for the first reporting period on September 30, 2014.

We will also be required to begin satisfying the product tracing, verification, and reporting requirements set out in the newly enacted Drug Quality and Security Act.

In order to distribute products commercially, we must also comply with state laws that require the registration of manufacturers and wholesale distributors of drug and biological products in a state, including, in certain states, manufacturers and distributors who ship products into the state even if such manufacturers or distributors have no place of business within the state.

Several states have enacted legislation requiring pharmaceutical and biotechnology companies to, among other things, establish marketing compliance programs, file periodic reports with the state, make periodic public disclosures on sales, marketing, pricing, clinical trials and other activities, and/or register their sales representatives, as well as to prohibit pharmacies and other healthcare entities from providing certain physician prescribing data to pharmaceutical and biotechnology companies for use in sales and marketing, and to prohibit certain other sales and marketing practices. All of our activities are potentially subject to federal and state consumer protection and unfair competition laws.

If our operations are found to be in violation of any of the federal and state healthcare laws described above or any other governmental regulations that apply to us, we may be subject to penalties, including without limitation, civil, criminal and/or administrative penalties, damages, fines, disgorgement, exclusion from participation in government programs, such as Medicare and Medicaid, injunctions, private *qui tam* actions brought by individual whistleblowers in the name of the government, or refusal to allow us to enter into government contracts, contractual damages, reputational harm, administrative burdens, diminished profits and future earnings, and the curtailment or restructuring of our operations, any of which could adversely affect our ability to operate our business and our results of operations.

### *Coverage, Pricing and Reimbursement*

Significant uncertainty exists as to the coverage and reimbursement status of any product candidates for which we obtain regulatory approval. In the United States and markets in other countries, sales of any products for which we receive regulatory approval for commercial sale will depend, in part, on the extent to which third-party payors provide coverage and establish adequate reimbursement levels for such products. In the United States, third-party payors include federal and state healthcare programs, private managed care providers, health insurers and other organizations. The process for determining whether a third-party payor will provide coverage for a product may be separate from the process for setting the price of a product or for establishing the reimbursement rate that such a payor will pay for the product. Third-party payors may limit coverage to specific products on an approved list, also known as a formulary, which might not include all of the FDA-approved products for a particular indication. Third-party payors are increasingly challenging the price, examining the medical necessity and reviewing the cost-effectiveness of medical products, therapies and services, in addition to questioning their safety and efficacy. We may need to conduct expensive pharmaco-economic studies in order to demonstrate the medical necessity and cost-effectiveness of our products, in addition to the costs required to obtain the FDA approvals. Our product candidates may not be considered medically necessary or cost-effective. A payor's decision to provide coverage for a product does not imply that an adequate reimbursement rate will be approved. Further, one payor's determination to provide coverage for a product does not assure that other payors will also provide coverage for the product. Adequate third-party reimbursement may

not be available to enable us to maintain price levels sufficient to realize an appropriate return on our investment in product development.

Different pricing and reimbursement schemes exist in other countries. In the EU, governments influence the price of pharmaceutical products through their pricing and reimbursement rules and control of national health care systems that fund a large part of the cost of those products to consumers. Some jurisdictions operate positive and negative list systems under which products may only be marketed once a reimbursement price has been agreed. To obtain

**Table of Contents**

reimbursement or pricing approval, some of these countries may require the completion of clinical trials that compare the cost-effectiveness of a particular product candidate to currently available therapies. Other member states allow companies to fix their own prices for medicines, but monitor and control company profits. The downward pressure on health care costs has become very intense. As a result, increasingly high barriers are being erected to the entry of new products. In addition, in some countries, cross-border imports from low-priced markets exert a commercial pressure on pricing within a country.

The marketability of any product candidates for which we receive regulatory approval for commercial sale may suffer if the government and third-party payors fail to provide adequate coverage and reimbursement. In addition, emphasis on managed care in the United States has increased and we expect will continue to increase the pressure on healthcare pricing. Coverage policies and third-party reimbursement rates may change at any time. Even if favorable coverage and reimbursement status is attained for one or more products for which we receive regulatory approval, less favorable coverage policies and reimbursement rates may be implemented in the future.

*Healthcare Reform*

In March 2010, President Obama enacted the Affordable Care Act, which has the potential to substantially change healthcare financing and delivery by both governmental and private insurers, and significantly impact the pharmaceutical and biotechnology industry. The Affordable Care Act will impact existing government healthcare programs and will result in the development of new programs.

Among the Affordable Care Act's provisions of importance to the pharmaceutical and biotechnology industries, in addition to those otherwise described above, are the following:

- n an annual, nondeductible fee on any entity that manufactures or imports certain specified branded prescription drugs and biologic agents apportioned among these entities according to their market share in some government healthcare programs, that began in 2011;
- n an increase in the statutory minimum rebates a manufacturer must pay under the Medicaid Drug Rebate Program, retroactive to January 1, 2010, to 23.1% and 13% of the average manufacturer price for most branded and generic drugs, respectively and capped the total rebate amount for innovator drugs at 100% of the Average Manufacturer Price, or AMP;
- n a new Medicare Part D coverage gap discount program, in which manufacturers must agree to offer 50% point-of-sale discounts off negotiated prices of applicable brand drugs to eligible beneficiaries during their coverage gap period, as a condition for the manufacturers' outpatient drugs to be covered under Medicare Part D;
- n extension of manufacturers' Medicaid rebate liability to covered drugs dispensed to individuals who are enrolled in Medicaid managed care organizations;

n

expansion of eligibility criteria for Medicaid programs by, among other things, allowing states to offer Medicaid coverage to additional individuals and by adding new mandatory eligibility categories for individuals with income at or below 133% of the federal poverty level, thereby potentially increasing manufacturers' Medicaid rebate liability;

n expansion of the entities eligible for discounts under the Public Health Service pharmaceutical pricing program; and

n a new Patient-Centered Outcomes Research Institute to oversee, identify priorities in, and conduct comparative clinical effectiveness research, along with funding for such research.

We anticipate that the Affordable Care Act will result in additional downward pressure on coverage and the price that we receive for any approved product, and could seriously harm our business. Any reduction in reimbursement from Medicare and other government programs may result in a similar reduction in payments from private payors. The implementation of cost containment measures or other healthcare reforms may prevent us from being able to generate revenue, attain profitability, or commercialize our products.

There have also been changes to the reimbursement landscape in the U.S. since the passage of the Affordable Care Act. On August 2, 2011, the President signed into law the Budget Control Act of 2011, which, among other things, created the Joint Select Committee on Deficit Reduction to recommend to Congress proposals in spending

## **Table of Contents**

reductions. The Joint Select Committee did not achieve a targeted deficit reduction of at least \$1.2 trillion for the years 2013 through 2021, triggering the legislation's automatic reduction to several government programs. This includes reductions to Medicare payments to providers of 2% per fiscal year, which went into effect on April 1, 2013. On January 2, 2013, President Obama signed into law the American Taxpayer Relief Act of 2012, which, among other things, reduced Medicare payments to several providers, including hospitals, imaging centers and cancer treatment centers and increased the statute of limitations period for the government to recover overpayments to providers from three to five years. We expect that additional state and federal healthcare reform measures will be adopted in the future, any of which could limit the amounts that federal and state governments will pay for healthcare products and services, which could result in reduced demand for our products and/or additional pricing pressure. In addition, it is possible that there will be further legislation or regulation that could harm our business, financial condition, and results of operations.

### *The Foreign Corrupt Practices Act*

The Foreign Corrupt Practices Act, or FCPA, prohibits any U.S. individual or business from paying, offering, or authorizing payment or offering of anything of value, directly or indirectly, to any foreign official, political party or candidate for the purpose of influencing any act or decision of the foreign entity in order to assist the individual or business in obtaining or retaining business. The FCPA also obligates companies whose securities are listed in the United States to comply with accounting provisions requiring the company to maintain books and records that accurately and fairly reflect all transactions of the corporation, including international subsidiaries, and to devise and maintain an adequate system of internal accounting controls for international operations.

### *Additional Regulation*

In addition to the foregoing, state and federal laws regarding environmental protection and hazardous substances, including the Occupational Safety and Health Act, the Resource Conservancy and Recovery Act and the Toxic Substances Control Act, affect our business. These and other laws govern our use, handling and disposal of various biological, chemical and radioactive substances used in, and wastes generated by, our operations. If our operations result in contamination of the environment or expose individuals to hazardous substances, we could be liable for damages and governmental fines. We believe that we are in material compliance with applicable environmental laws and that continued compliance therewith will not have a material adverse effect on our business. We cannot predict, however, how changes in these laws may affect our future operations.

### *Europe / Rest of World Government Regulation*

In addition to regulations in the United States, we will be subject to a variety of regulations in other jurisdictions governing, among other things, clinical trials and any commercial sales and distribution of our products. Whether or not we obtain FDA approval of a product, we must obtain the requisite approvals from regulatory authorities in foreign countries prior to the commencement of clinical trials or marketing of the product in those countries. Certain countries outside of the United States have a similar process that requires the submission of a clinical trial application much like the IND prior to the commencement of human clinical trials. In the EU, for example, a clinical trial application must be submitted to each country's national health authority and an independent ethics committee, much like the FDA and IRB, respectively. Once the clinical trial application is approved in accordance with a country's requirements, clinical trial development may proceed. Because biologically sourced raw materials are subject to unique contamination risks, their use may be restricted in some countries.

The requirements and process governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, the clinical trials are conducted in accordance with GCP and the applicable



regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

To obtain regulatory approval of an investigational drug or biological product under EU regulatory systems, we must submit a marketing authorization application. The application used to file the BLA in the United States is similar to that required in the EU, with the exception of, among other things, country-specific document requirements.

For other countries outside of the EU, such as countries in Eastern Europe, Latin America or Asia, the requirements governing the conduct of clinical trials, product licensing, pricing and reimbursement vary from country to country. In all cases, again, the clinical trials are conducted in accordance with GCP and the applicable regulatory requirements and the ethical principles that have their origin in the Declaration of Helsinki.

**Table of Contents**

**Employees**

As of September 30, 2014, we had 30 employees, all of whom were full-time, 24 of whom were engaged in research and development activities and six of whom were engaged in business development, finance, information systems, facilities, human resources or administrative support. None of our employees is subject to a collective bargaining agreement. We consider our relationship with our employees to be good.

**Facilities**

We lease a 23,325 square foot facility in Houston, Texas for administrative and research and development activities that expires on January 31, 2020, subject to five one-year renewal options. We believe that our existing facilities are adequate to meet our current needs, and that suitable additional alternative spaces will be available in the future on commercially reasonable terms.

**Legal Proceedings**

From time to time, we may be involved in various claims and legal proceedings relating to claims arising out of our operations. We are not currently a party to any material legal proceedings.

**Table of Contents****MANAGEMENT****Executive Officers and Directors**

The following table sets forth certain information regarding our current executive officers and directors:

<b>NAME</b>	<b>AGE</b>	<b>POSITION(S)</b>
<b>Executive Officers</b>		
Thomas J. Farrell	51	President and Chief Executive Officer and Director
Alan A. Musso, C.P.A., C.M.A.	52	Chief Financial Officer and Treasurer
Kevin M. Slawin, M.D.	53	Chief Medical Officer and Chief Technology Officer and Director
David M. Spencer, Ph.D.	52	Chief Scientific Officer
Annemarie Moseley, Ph.D., M.D. <sup>(4)</sup>	59	Chief Operating Officer and Senior Vice President of Clinical Development and Regulatory Affairs
Peter L. Hoang	42	Senior Vice President of Business Development and Strategy
Ken Moseley, J.D. <sup>(4)</sup>	58	Vice President of Intellectual Property and Legal Affairs
Joseph Senesac	44	Vice President of Manufacturing
<b>Non-Employee Directors</b>		
James Brown <sup>(1)(2)(3)</sup>	50	Director and Chairman of the Board
Reid M. Huber, Ph.D. <sup>(1)(3)</sup>	42	Director
Frank B. McGuyer <sup>(1)(2)</sup>	63	Director
Jon P. Stonehouse <sup>(1)(2)</sup>	53	Director

(1) Member of the compensation committee.

(2) Member of the audit committee.

(3) Member of the nominating and corporate governance committee.

(4) Annemarie Moseley and Ken Moseley are married.

**Executive Officers**

**Thomas J. Farrell** has served as our Chief Executive Officer since February 2006 and as a member of our board of directors since April 2007. Prior to joining us, he was the founding president and chief executive officer of San Diego-based Cylene Pharmaceuticals, Inc. a private pharmaceutical development company. Mr. Farrell received his B.A. in Engineering from the University of Cambridge and M.B.A. from Stanford Graduate School of Business, where he was an Arjay Miller Scholar. Our board of directors believes that Mr. Farrell's experience in the pharmaceutical industry and his long-standing services as our Chief Executive Officer qualify him to serve on our Board of Directors.

**Alan A. Musso, C.P.A., C.M.A.** has served as our Chief Financial Officer and Treasurer since November 2014. From February 2002 to November 2014, Mr. Musso served in various positions at Targacept, Inc., a public biopharmaceutical company, including as Senior Vice President of Finance and Administration from March 2010 to November 2014, Chief Financial Officer and Treasurer from February 2002 to November 2014, and Assistant Secretary from June 2007 to November 2014. Mr. Musso has over 25 years of biotech and pharmaceutical industry experience in both large and emerging growth companies. Mr. Musso received his B.S. degree from Saint Mary's College of California and his graduate degree from the American Graduate School of International Management in Glendale, Arizona.

**Kevin M. Slawin, M.D.** founded Bellicum with David Spencer, Ph.D., in July 2004 and has served as a member of our board of directors since its founding. Since April 2014, Dr. Slawin has served as our Chief Medical Officer and Chief Technology Officer. From February 2006 to April 2014, he served as our Executive Chairman and Chief Medical Officer. Dr. Slawin was the chairman of the board and our Chief Executive Officer, President and Secretary from September 2004 until February 2006. Previously, Dr. Slawin had a long tenure in academic medicine at Baylor College of Medicine, where he most recently was the Dan Duncan Professor in Prostate Cancer and Prostatic Diseases, and Director, The Baylor Prostate Center, until 2007. He received his B.A. and M.D. from Columbia University, where he was inducted into Phi Beta Kappa and Alpha Omega Alpha, and completed an American Foundation of Urologic Diseases Scholar Fellowship in Urologic Oncology at Baylor College of Medicine. Our board of directors believes that Dr. Slawin's educational and professional experiences as well as his experience as one of our founders qualifies him to serve on our Board of Directors.

---

**Table of Contents**

**David M. Spencer, Ph.D.** founded Bellicum with Kevin M. Slawin, M.D. in July 2004 and served as a member of our board of directors until September 2004. He has served as our scientific advisor to the Company since our inception and has served as our Chief Scientific Officer since November 2011, a position that he also held part-time as a consultant since September 2004. From January 1996 to November 2011 he served as professor in the department of Pathology and Immunology at Baylor College of Medicine and as vice chairman of the department from January 2010 to November 2011. Dr. Spencer is the original inventor of our CID technology, and together with Dr. Slawin, developed the first clinical applications of the technology, DeCIDE and CaspaCIDE. He received his B.A. degree in Chemistry from the University of California, San Diego and his Ph.D. in Biology at Massachusetts Institute of Technology and was a postdoctoral fellow at Stanford University.

**Annemarie Moseley, Ph.D., M.D.** has served as our Chief Operating Officer since November 2012 and has served as our Senior Vice President of Clinical Development and Regulatory Affairs since October 2011. From July 2005 to September 2011 Dr. Moseley served as Chief Executive Officer and Chief Medical Officer at REPAIR Technologies, Inc., a private biotechnology company. Dr. Moseley has over 20 years of industry experience in translational medicine and clinical development of stem cell therapies, immunotherapies, biological devices and combination products, including overseeing the first late-stage Graft versus Host Disease study in patients who underwent hematopoietic stem cell transplant. She received her B.S. and M.S. from the University of Texas at Arlington, and received her Ph.D. in Physiology and Biochemistry from Utah State. She received her M.D. from Baylor College of Medicine where she completed an internal medicine residency and a genetics fellowship.

**Peter L. Hoang** has served as our Senior Vice President of Business Development and Strategy since November 2014. From September 2012 to November 2014, he served as the Managing Director of Innovations in the Strategic Industry Ventures department at the University of Texas MD Anderson Cancer Center, a nonprofit cancer treatment and research center. From November 2010 to March 2012, Mr. Hoang served as a Managing Director of Mergers & Acquisitions in the healthcare investment banking practice at CIT Group Inc., a public investment banking firm. Prior to CIT Group, from May 2005 to November 2010, he served as an Executive Director of Global Mergers & Acquisitions in the Investment Banking department of Oppenheimer & Co. Inc., an investment banking and financial advisory firm. Mr. Hoang received his B.A. degree from Yale University and his M.B.A. from the Anderson School of Management at the University of California, Los Angeles, with Business Honors Society distinction.

**Ken Moseley, J.D.** has served as our Vice President of Intellectual Property and Legal Affairs since December 2011 and as our Corporate Secretary since February 2012. From March 2009 to September 2011, he served as General Counsel at REPAIR Technologies, Inc., a private biotechnology company. From February 2002 to March 2009 he served as General Counsel at Cognate Bioservices, a private biotechnology company. He received his B.S. degree from the University of Houston and his B.A. degree from Rice University. He received his J.D. from the University of Houston Law Center. He is a registered U.S. patent attorney and is a member of the State Bars of Texas and California.

**Joseph Senesac** has served as our Vice President of Manufacturing since September 2011. From February 2009 to August 2011, he served as Senior Director of Biologics Manufacturing and Development in the Human Therapeutics Division at Intrexon Corporation, a public biotechnology company. Prior to Intrexon, from May 1996 to February 2009, Mr. Senesac served in various positions at Introgen Therapeutics, which at that time was a public gene therapy company, with his final role being Director of Process Sciences and Development. Mr. Senesac received a B.A. in Chemistry and Economics from Knox College, and an M.B.A. from the University of Colorado at Colorado Springs.

**Non-Employee Directors**

**James Brown** has served as a member of our board of directors since November 2011. Since July 2009 he has served as managing director of AVG Ventures, a private investment firm. From 2003 to 2009, Mr. Brown was an independent investor and served on a number of private company boards of directors. From 1999 to 2002, he served as executive vice president and general manager of OpenTV, Inc., a technology and media company, where he co-founded and managed the company's applications business unit, prior to its sale to Liberty Media in 2002. Earlier in his career, Mr. Brown was a partner in the law firms of McDermott, Will & Emery and Pillsbury Madison & Sutro. He received his B.S. in accounting from Weber State University and his J.D. from BYU Law School. Our board of directors believes that Mr. Brown's business experience and his success as an investor and entrepreneur qualify him to serve on our board of directors.

## **Table of Contents**

**Reid M. Huber, Ph.D.** has served as a member of our board of directors since October 2014. Dr. Huber currently serves as the Executive Vice President and Chief Scientific Officer of Incyte Corporation, a publicly traded biotechnology company, where he has held various management positions since January 2002. From 1998 to 2002, Dr. Huber held scientific research positions at DuPont Pharmaceuticals Company, a private chemicals and health care company. Prior to DuPont Pharmaceuticals Company, from 1997 to 1998 Dr. Huber held intramural pre-doctoral and post-doctoral fellowships at the National Institutes of Health. Dr. Huber received his B.S. in biochemistry/molecular genetics from Murray State University and his Ph.D. in molecular genetics from Washington University. Our board of directors believes that Dr. Huber's extensive background in the pharmaceutical industry and current management experience at a public biotechnology company qualify him to serve on our board of directors.

**Frank B. McGuyer** has served as a member of our board of directors since March 2009. He is the founder of, and since December 1988 has served as the chairman of the board of directors and chief executive officer of, McGuyer Homebuilders Inc., a private construction company. He received his B.B.A. with honors at Southern Methodist University. Our board of directors believes that Mr. McGuyer's operational, business and investment experience qualifies him to serve on our board of directors.

**Jon P. Stonehouse** has served as a member of our board of directors since December 2014. Since January 2007, Mr. Stonehouse has served as the Chief Executive Officer and a member of the Board of Directors of BioCryst Pharmaceuticals, Inc., a public biopharmaceutical company. Since July 2007, he has also served as President of BioCryst. From March 2002 to December 2006, Mr. Stonehouse served in various positions at Merck KGaA, a pharmaceutical Company, including as Senior Vice President of Corporate Development from July 2002 to December 2006, and Vice President of Global Licensing and Business Development and Integration from March 2002 to December 2006. Prior to Merck, from December 1999 to February 2002, Mr. Stonehouse served as Vice President, Licensing and Business Development-Strategy & Integration and IT at EMD Pharmaceuticals, Inc., the U.S. Ethical Pharma division of Merck. Since November 2008, Mr. Stone has also served as a member of the Advisory Board of Precision Biosciences, Inc., a private biotechnology company. Mr. Stonehouse received his B.S. in Chemistry from the University of Minnesota. Our board of directors believes that Mr. Stonehouse's management background, experience as a director at a public pharmaceutical company and extensive history as an advisory board member in the pharmaceutical industry qualify him to serve on our Board of Directors.

## **Board Composition**

Our business and affairs are organized under the direction of our board of directors, which currently consists of six members. The primary responsibilities of our board of directors are to provide oversight, strategic guidance, counseling and direction to our management. Our board of directors meets on a regular basis and additionally as required.

Our board of directors has determined that all of our directors, except Mr. Farrell and Dr. Slawin, are independent directors, as defined by Rule 5605(a)(2) of the NASDAQ Listing Rules.

In accordance with the terms of our amended and restated certificate of incorporation and bylaws, which will be effective immediately prior to consummation of this offering, our board of directors will be divided into three classes, class I, class II and class III, with members of each class serving staggered three-year terms.

Effective upon the closing of this offering, our board of directors will be comprised of the following classes:

- n Class I, which will consist of Frank. B. McGuyer and Jon P. Stonehouse, whose terms will expire at our annual meeting of stockholders to be held in 2015;
  
- n Class II, which will consist of James F. Brown and Kevin M. Slawin, M.D., and whose terms will expire at our annual meeting of stockholders to be held in 2016; and
  
- n Class III, which will consist of Thomas J. Farrell and Reid M. Huber, Ph.D., and whose terms will expire at our annual meeting of stockholders to be held in 2017.

At each annual meeting of stockholders to be held after the initial classification, the successors to directors whose terms then expire will serve until the third annual meeting following their election and until their successors are duly elected and qualified. The authorized size of our board of directors is currently six members. The authorized number



## **Table of Contents**

of directors may be changed only by resolution by a majority of the board of directors. This classification of the board of directors may have the effect of delaying or preventing changes in our control or management. Our directors may be removed for cause by the affirmative vote of the holders of at least 66 2/3% of our voting stock.

## **Board Leadership Structure**

Our board of directors is currently chaired by James F. Brown who has authority, among other things, to call and preside over Board of Directors meetings, to set meeting agendas, and to determine materials to be distributed to the Board of Directors. Accordingly, the Chairman has substantial ability to shape the work of the Board of Directors. We believe that separation of the positions of Chairman and Chief Executive Officer reinforces the independence of the Board of Directors in its oversight of our business and affairs. In addition, we have a separate chair for each committee of the Board of Directors. The chairs of each committee are expected to report annually to the Board of Directors on the activities of their committee in fulfilling their responsibilities as detailed in their respective charters or specify any shortcomings should that be the case. In addition, we believe that having a separate Chairman creates an environment that is more conducive to objective evaluation and oversight of management's performance, increasing management accountability and improving the ability of the Board of Directors to monitor whether management's actions are in the best interests of us and our stockholders. As a result, we believe that having a separate Chairman can enhance the effectiveness of the Board of Directors as a whole.

## **Role of the Board in Risk Oversight**

One of the key functions of our board of directors is informed oversight of our risk management process. Our board of directors does not have a standing risk management committee, but rather administers this oversight function directly through the board of directors as a whole, as well as through various standing committees of our board of directors that address risks inherent in their respective areas of oversight. In particular, our board of directors is responsible for monitoring and assessing strategic risk exposure and our audit committee has the responsibility to consider and discuss our major financial risk exposures and the steps our management has taken to monitor and control these exposures, including guidelines and policies to govern the process by which risk assessment and management is undertaken. The audit committee also monitors compliance with legal and regulatory requirements. Our nominating and corporate governance committee monitors the effectiveness of our corporate governance practices, including whether they are successful in preventing illegal or improper liability-creating conduct. Our compensation committee assesses and monitors whether any of our compensation policies and programs has the potential to encourage excessive risk-taking.

## **Board Committees**

Our board of directors has established an audit committee, a compensation committee and a nominating and corporate governance committee.

### ***Audit Committee***

Our audit committee consists of Messrs. Brown, McGuyer and Stonehouse. Our board of directors has determined that each of the members of this committee satisfies the NASDAQ Stock Market independence requirements. Each member of our audit committee can read and understand fundamental financial statements in accordance with NASDAQ audit committee requirements. In arriving at this determination, the board has examined each audit committee member's scope of experience and the nature of their prior and/or current employment.

Mr. Brown serves as the chair of our audit committee. Our board of directors has determined that Mr. Brown qualifies as an audit committee financial expert within the meaning of SEC regulations and meets the financial sophistication requirements of the NASDAQ Listing Rules. In making this determination, our board has considered Mr. Brown's formal education and previous and current experience in financial roles. Both our independent registered public accounting firm and management periodically meet privately with our audit committee.

The functions of this committee include, among other things:

- n evaluating the performance, independence and qualifications of our independent auditors and determining whether to retain our existing independent auditors or engage new independent auditors;

**Table of Contents**

- n reviewing and approving the engagement of our independent auditors to perform audit services and any permissible non-audit services;
- n monitoring the rotation of partners of our independent auditors on our engagement team as required by law;
- n prior to engagement of any independent auditor, and at least annually thereafter, reviewing relationships that may reasonably be thought to bear on their independence, and assessing and otherwise taking the appropriate action to oversee the independence of our independent auditor;
- n reviewing our annual and quarterly financial statements and reports, including the disclosures contained under the caption Management's Discussion and Analysis of Financial Condition and Results of Operations, and discussing the statements and reports with our independent auditors and management;
- n reviewing with our independent auditors and management significant issues that arise regarding accounting principles and financial statement presentation and matters concerning the scope, adequacy and effectiveness of our financial controls;
- n reviewing with management and our auditors any earnings announcements and other public announcements regarding material developments;
- n establishing procedures for the receipt, retention and treatment of complaints received by us regarding financial controls, accounting or auditing matters and other matters;
- n preparing the report that the SEC requires in our annual proxy statement;
- n reviewing and providing oversight of any related-person transactions in accordance with our related person transaction policy and reviewing and monitoring compliance with legal and regulatory responsibilities, including our code of business conduct and ethics;
- n reviewing our major financial risk exposures, including the guidelines and policies to govern the process by which risk assessment and risk management is implemented;
- n reviewing on a periodic basis our investment policy; and
- n reviewing and evaluating on an annual basis the performance of the audit committee and the audit committee charter.

We believe that the composition and functioning of our audit committee complies with all applicable requirements of the Sarbanes-Oxley Act of 2002, as amended, or the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

### *Compensation Committee*

Our compensation committee consists of Messrs. Brown, McGuyer and Stonehouse and Dr. Huber. Mr. Stonehouse serves as the chair of our compensation committee. Our board of directors has determined that each of the members of our compensation committee is a non-employee director, as defined in Rule 16b-3 promulgated under the Securities Exchange Act of 1934, or the Exchange Act, is an outside director, as defined pursuant to Section 162(m) of the Internal Revenue Code of 1986, as amended, or the Code, and satisfies the NASDAQ Stock Market independence requirements. The functions of this committee include, among other things:

- n reviewing, modifying and approving (or if it deems appropriate, making recommendations to the full board of directors regarding) our overall compensation strategy and policies;
- n making recommendations to the full board of directors regarding the compensation and other terms of employment of our executive officers;
- n reviewing and making recommendations to the full board of directors regarding performance goals and objectives relevant to the compensation of our executive officers and assessing their performance against these goals and objectives;
- n reviewing and approving (or if it deems it appropriate, making recommendations to the full board of directors regarding) the equity incentive plans, compensation plans and similar programs advisable for us, as well as modifying, amending or terminating existing plans and programs;
- n evaluating risks associated with our compensation policies and practices and assessing whether risks arising from our compensation policies and practices for our employees are reasonably likely to have a material adverse effect on us;

**Table of Contents**

- n reviewing and making recommendations to the full board of directors regarding the type and amount of compensation to be paid or awarded to our non-employee board members;
- n establishing policies with respect to votes by our stockholders to approve executive compensation to the extent required by Section 14A of the Exchange Act and, if applicable, determining our recommendations regarding the frequency of advisory votes on executive compensation;
- n reviewing and assessing the independence of compensation consultants, legal counsel and other advisors as required by Section 10C of the Exchange Act;
- n administering our equity incentive plans;
- n establishing policies with respect to equity compensation arrangements;
- n reviewing the competitiveness of our executive compensation programs and evaluating the effectiveness of our compensation policy and strategy in achieving expected benefits to us;
- n reviewing and making recommendations to the full board of directors regarding the terms of any employment agreements, severance arrangements, change in control protections and any other compensatory arrangements for our executive officers;
- n reviewing with management and approving our disclosures under the caption "Compensation Discussion and Analysis" in our periodic reports or proxy statements to be filed with the SEC, to the extent such caption is included in any such report or proxy statement;
- n preparing the report that the SEC requires in our annual proxy statement; and
- n reviewing and evaluating on an annual basis the performance of the compensation committee and the compensation committee charter.

We believe that the composition and functioning of our compensation committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

***Nominating and Corporate Governance Committee***

Our nominating and corporate governance committee consists of Dr. Huber and Mr. Brown. Our board of directors has determined that each of the members of this committee satisfies the NASDAQ Stock Market independence requirements. Dr. Huber serves as the chair of our nominating and corporate governance committee. The functions of this committee include, among other things:

- n identifying, reviewing and evaluating candidates to serve on our board of directors;
- n determining the minimum qualifications for service on our board of directors;
- n evaluating director performance on the board and applicable committees of the board and determining whether continued service on our board is appropriate;
- n evaluating, nominating and recommending individuals for membership on our board of directors;
- n evaluating nominations by stockholders of candidates for election to our board of directors;
- n considering and assessing the independence of members of our board of directors;
- n developing a set of corporate governance policies and principles and recommending to our board of directors any changes to such policies and principles;
- n considering questions of possible conflicts of interest of directors as such questions arise; and
- n reviewing and evaluating on an annual basis the performance of the nominating and corporate governance committee and the nominating and corporate governance committee charter.

We believe that the composition and functioning of our nominating and corporate governance committee complies with all applicable requirements of the Sarbanes-Oxley Act, and all applicable SEC and NASDAQ rules and regulations. We intend to comply with future requirements to the extent they become applicable to us.

#### **Compensation Committee Interlocks and Insider Participation**

None of the members of our compensation committee has ever been an executive officer or employee of ours. None of our executive officers currently serves, or has served during the last completed fiscal year, on the compensation committee or board of directors of any other entity that has one or more executive officers serving as a member of our board of directors or compensation committee.

**Table of Contents**

**Limitation on Liability and Indemnification of Directors and Officers**

Our amended and restated certificate of incorporation, which will be effective immediately prior to consummation of this offering, limit our directors' liability to the fullest extent permitted under Delaware corporate law. Delaware corporate law provides that directors of a corporation will not be personally liable for monetary damages for breach of their fiduciary duties as directors, except for liability:

- n for any transaction from which the director derives an improper personal benefit;
- n for any act or omission not in good faith or that involves intentional misconduct or a knowing violation of law;
- n under Section 174 of the Delaware General Corporation Law (unlawful payment of dividends or redemption of shares); or
- n for any breach of a director's duty of loyalty to the corporation or its stockholders.

If the Delaware General Corporation Law is amended to authorize corporate action further eliminating or limiting the personal liability of directors, then the liability of our directors shall be eliminated or limited to the fullest extent permitted by the Delaware General Corporation Law, as so amended.

Delaware law and our amended and restated bylaws provide that we will, in certain situations, indemnify our directors and officers and may indemnify other employees and other agents, to the fullest extent permitted by law. Any indemnified person is also entitled, subject to certain limitations, to payment or reimbursement of reasonable expenses (including attorneys' fees and disbursements) in advance of the final disposition of the proceeding.

In addition, we have entered, and intend to continue to enter, into separate indemnification agreements with our directors and officers. These agreements, among other things, require us to indemnify our directors and officers for certain expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by a director or officer in any action or proceeding arising out of their services as one of our directors or officers or any other company or enterprise to which the person provides services at our request.

We maintain a directors' and officers' insurance policy pursuant to which our directors and officers are insured against liability for actions taken in their capacities as directors and officers. We believe that these provisions in our amended and restated certificate of incorporation and amended bylaws and these indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

Insofar as indemnification for liabilities arising under the Securities Act may be permitted to directors, officers or control persons, in the opinion of the SEC, such indemnification is against public policy as expressed in the Securities Act and is therefore unenforceable.

**Table of Contents****EXECUTIVE AND DIRECTOR COMPENSATION**

Our named executive officers for the year ended December 31, 2013, which consist of our principal executive officer and our two other most highly compensated executive officers, are:

- n Thomas J. Farrell, our President and Chief Executive Officer
  
- n Annemarie Moseley, Ph.D., M.D., our Chief Operating Officer and Senior Vice President of Clinical Development and Regulatory Affairs
  
- n Kevin M. Slawin, M.D., our Chief Medical Officer and Chief Technology Officer

**Summary Compensation Table**

NAME AND PRINCIPAL POSITION	YEAR	SALARY (\$)	NON-EQUITY INCENTIVE			TOTAL (\$)
			OPTION AWARDS (\$) <sup>(1)</sup>	PLAN COMPENSATION (\$) <sup>(2)</sup>	ALL OTHER COMPENSATION (\$) <sup>(3)</sup>	
Thomas J. Farrell <i>President and Chief Executive Officer</i>	2013	350,000		84,000	4,006	438,006
Annemarie Moseley, Ph.D., M.D. <i>Chief Operating Officer and Senior Vice President of Clinical Development and Regulatory Affairs</i>	2013	320,000	93,000	76,800	11,429	501,229
Kevin M. Slawin, M.D. <sup>(4)</sup> <i>Chief Medical Officer and Chief Technology Officer</i>	2013	250,000	546,000	60,000	2,100	858,100

(1) In accordance with SEC rules, this column reflects the aggregate grant date fair value of the option awards granted during 2013 computed in accordance with Financial Accounting Standard Board Accounting Standards



Codification Topic 718 for stock-based compensation transactions (ASC 718). Assumptions used in the calculation of these amounts are included in Note 9 to our audited financial statements included elsewhere in this prospectus. These amounts do not reflect the actual economic value that will be realized by the named executive officer upon the vesting of the stock options, the exercise of the stock options, or the sale of the common stock underlying such stock options.

- (2) Amounts shown represent annual performance-based bonuses earned for 2013. For more information, see Annual Bonus Opportunity below.
- (3) Amounts in this column reflect the following for 2013: For Mr. Farrell, \$2,563 for reimbursement of costs related to commuting, \$1,200 in parking subsidies and \$243 in reimbursements for life, disability and accidental death and dismemberment insurance premiums; for Dr. Moseley, \$9,793 for reimbursement of costs related to commuting, \$1,200 in parking subsidies and \$436 in reimbursements for life, disability and accidental death and dismemberment insurance premiums; for Dr. Slawin, \$2,100 in parking subsidies.
- (4) All of Dr. Slawin's compensation was paid pursuant to a consulting agreement entered into in November 2011.

**Annual Base Salary**

The compensation of our named executive officers is generally determined and approved by our board of directors. The 2013 base salaries effective as of February 1, 2013 were as follows:

NAME	2013 BASE SALARY (\$)
Thomas J. Farrell	350,000
Annemarie Moseley, Ph.D., M.D.	320,000
Kevin M. Slawin, M.D	250,000

In January 2014, our board of directors approved a 4% increase to the base salaries of each of our named executive officers, effective February 1, 2014.

## **Table of Contents**

### **Annual Bonus Opportunity**

In addition to base salaries, our named executive officers are eligible to receive annual performance-based cash bonuses, which are designed to provide appropriate incentives to our executives to achieve defined annual corporate goals and to reward our executives for individual achievement towards these goals. The annual performance-based bonus each named executive officer is eligible to receive is generally based on the extent to which we achieve the corporate goals that our board of directors establishes each year. At the end of the year, our board of directors reviews our performance against each corporate goal and approves the extent to which we achieved each of our corporate goals.

Our board of directors will generally consider each named executive officer's individual contributions towards reaching our annual corporate goals but does not typically establish specific individual goals for our named executive officers. There is no minimum bonus percentage or amount established for the named executive officers and, as a result, the bonus amounts vary from year to year based on corporate and individual performance. For 2013, the target bonus for Mr. Farrell and Dr. Moseley was 30% of base salary and the target bonus for Dr. Slawin was 30% of base salary (including consulting fees).

Our corporate goals for 2013, established by our board of directors, were to initiate clinical trials of BPX-201 and BPX-501 and to complete the closing of the second tranche of our Series B convertible preferred stock financing (which transaction is described below under Certain Relationships and Related Party Transactions. No specific individual goals were established for any of our named executive officers for 2013.

In January 2014, our board of directors reviewed our corporate goals and determined that on an overall basis, we had substantially achieved our goals and that each of the named executive officers should receive a bonus equivalent to 80% of their target bonuses. Specifically, we initiated our clinical trials of BPX-201 and BPX-501 and completed the closing of the second tranche of our Series B convertible preferred stock financing.

### **Equity-Based Incentive Awards**

Our equity-based incentive awards are designed to align our interests with those of our employees and consultants, including our named executive officers. Our board of directors is responsible for approving equity grants. Vesting of equity awards is generally tied to continuous service with us and serves as an additional retention measure. Our executives generally are awarded an initial new hire grant upon commencement of employment. Additional grants may occur periodically in order to specifically incentivize executives with respect to achieving certain corporate goals or to reward executives for exceptional performance.

Prior to this offering, we have granted all equity awards pursuant to the 2011 Plan and the 2006 stock option plan, as amended, or the 2006 Plan, the terms of which are described below under Equity Benefit Plans. All options are granted with a per share exercise price equal to no less than the fair market value of a share of our common stock on the date of the grant of such award, as determined by our board of directors. Generally our stock option awards vest over a four-year period subject to the holder's continuous service to us.

On August 14, 2013 our board of directors granted an option to purchase 352,941 shares of our common stock to Dr. Slawin at a per share exercise price of \$2.55. Under this grant, 34% of shares vested on July 31, 2013 and an additional 33% vest on each anniversary thereafter until the shares are fully vested, subject to Dr. Slawin's continued service with us.

On August 14, 2013, our board of directors granted an option to purchase 58,823 shares of our common stock to Dr. Moseley at a per share exercise price of \$2.55. The option grant vests as of November 26, 2012 over a four-year period subject to Dr. Moseley's continued service with us.

On November 11, 2014, our board of directors granted an option to purchase 235,294 shares to Mr. Farrell and an option to purchase 108,823 shares to each of Dr. Moseley and Dr. Slawin. Each of these options were granted at a per share exercise price of \$7.463 and vest over a four-year period subject to the named executive officer's continued service with us. On December 4, 2014, our board of directors approved the grant of an option to purchase 117,647 shares and a restricted stock award covering 117,647 shares to Mr. Musso in connection with his commencement of employment with us. The option and restricted stock award will be granted under our 2014 Plan effective and

## **Table of Contents**

contingent upon the execution and delivery of the underwriting agreement relating to this offering, and with respect to the option, at a per share exercise price equal to the price per share at which our common stock is first sold to the public in this offering. Each of the option and restricted stock awards vest over a four-year period subject to Mr. Musso's continued service with us.

### **Agreements with our Named Executive Officers**

Below are descriptions of our employment agreements and offer letter agreements with our named executive officers and, to provide additional information, with Mr. Musso. For a discussion of the severance pay and other benefits to be provided in connection with a termination of employment and/or a change in control under the arrangements with our named executive officers, please see [Potential Payments Upon Termination or Change in Control](#) below.

*Thomas Farrell.* We entered into a Second Amended & Restated Employment Agreement with Mr. Farrell in November 2011 which governs the current terms of his employment with us. Pursuant to the agreement, Mr. Farrell is entitled to an annual base salary of \$350,000, is eligible to receive an annual performance bonus of up to 30% of his base salary, as determined by our board of directors and options to purchase 258,823 shares of our common stock which were granted in November 2011. Mr. Farrell is additionally entitled to certain severance benefits, the terms of which are described below under [Potential Payments Upon Termination or Change of Control](#). Mr. Farrell's Employment Agreement provides of an initial term of one year that will automatically renew for successive one year terms unless either party terminates the agreement.

We entered into a Third Amended and Restated Employment Agreement with Mr. Farrell in November 2014 that will replace his Second Amended and Restated Employment Agreement described above and become effective in connection with the execution and delivery of the underwriting agreement related to this offering. Under the Third Amended and Restated Employment Agreement, Mr. Farrell is entitled to an annual base salary of \$415,000, is eligible to receive an annual target performance bonus of 40% of his base salary as determined by the board of directors and is eligible to receive certain severance benefits, the terms of which are described below under [Potential Payments Upon Termination or Change of Control](#).

*Annemarie Moseley, Ph.D., M.D.* We entered into an Employment Agreement with Dr. Moseley in October 2011, as amended in November 2012, which governs the current terms of her employment with us. Pursuant to the agreement, Dr. Moseley is entitled to an annual base salary of \$320,000, is eligible to receive an annual target performance bonus of up to 25% of her base salary, as determined by our board of directors, and an option to purchase 88,235 shares of our common stock pursuant to the October 2011 employment agreement, which was granted in October 2011, and an additional option to purchase 58,823 shares of our common stock pursuant to the November 2012 amendment, which was granted in November 2011. Dr. Moseley is additionally entitled to certain severance benefits, the terms of which are described below under [Potential Payments Upon Termination or Change of Control](#). Dr. Moseley's Employment Agreement provides of an initial term of one year that will automatically renew for successive one year terms unless either party terminates the agreement.

We intend to enter into an Amended and Restated Employment Agreement with Dr. Moseley that will replace her amended Employment Agreement described above and become effective on or after the execution and delivery of the underwriting agreement related to this offering. Under the Amended and Restated Employment Agreement, Dr. Moseley will be entitled to an annual base salary of \$360,000, will be eligible to receive an annual target performance bonus of 35% of her base salary as determined by the board of directors and will be eligible to receive certain severance benefits, the terms of which are described below under [Potential Payments Upon Termination or Change of Control](#).

*Kevin M. Slawin, M.D.* We entered into a Third Amended Consulting Agreement with Dr. Slawin in November 2011 which governs the current terms of his consulting relationship with us. Pursuant to the agreement, Dr. Slawin is entitled to an annual base consulting fee of \$250,000, is eligible to receive an annual performance bonus, of up to 30% of his base consulting fee, as determined by our board of directors, an option to purchase 258,823 shares of our common stock, which was granted in November 2011 and an additional option to purchase 352,941 shares of our common stock, which was granted in July 2013 upon the closing of the second tranche of our Series B convertible preferred stock financing (which transaction is described below under Certain Relationships and Related Party Transactions ). Dr. Slawin is additionally entitled to certain severance benefits, the terms of which are

---

**Table of Contents**

described below under Potential Payments Upon Termination or Change of Control. Dr. Slawin's Third Amended Consulting Agreement provides of an initial term of three years that will automatically renew for two successive one year terms unless either party terminates the agreement. We intend to enter into an Employment Agreement with Dr. Slawin, that will replace his Third Amended Consulting Agreement described above and become effective on or after the execution and delivery of the underwriting agreement related to this offering. Under the Employment Agreement, it is expected that Dr. Slawin will be employed by us on an 80% of full-time basis, will be entitled to an annual base salary of \$360,000, will be eligible to receive an annual target performance bonus of 35% of his base salary as determined by the board of directors and will be eligible to receive certain severance benefits, the terms of which are described below under Potential Payments Upon Termination or Change of Control.

We entered into an Employment Agreement with Mr. Musso in connection with his commencement of employment with us in November 2014 that will become effective in connection with the execution and delivery of the underwriting agreement related to this offering. Under the Employment Agreement, Mr. Musso is entitled to an annual base salary of \$335,000 and in 2015 will be eligible to receive an annual target performance bonus of 35% of his base salary as determined by the board of directors. In addition, Mr. Musso will be paid a \$100,000 signing bonus within forty-five days of his start date (which he will be required to repay to us if he is terminated by us with cause, dies or becomes disabled or resigns without good reason within the twelve months following his November 22, 2014 start date) and will be eligible to receive reimbursement from us for his commuting costs for up to two years following his start date. The Employment Agreement also provides that Mr. Musso will be eligible to receive certain severance benefits, the terms of which are described below under Potential Payments Upon Termination or Change of Control.

**Potential Payments Upon Termination or Change of Control**

Regardless of the manner in which a named executive officer's service terminates, the named executive officer is entitled to receive amounts earned during his or her term of service, including salary and unused vacation pay. In addition, each of our named executive officers is eligible to receive certain benefits pursuant to his or her employment or consulting agreement with us described above under Agreements with our Named Executive Officers.

Upon a termination without cause or resignation for good reason (each as defined below), under the terms of each named executive officer's agreement described above which is currently effective, each of Mr. Farrell, Dr. Moseley and Dr. Slawin will be eligible to receive payments equal to his or her base salary or consulting fee, as applicable, then in effect for 12 months, and, other than under Dr. Slawin's consulting agreement, a pro rated annual performance bonus and reimbursement for continuation of healthcare benefits for 12 months.

In each of our named executive officers agreements, cause generally means the occurrence of any of the following events, conditions or actions with respect to the executive: (1) willful misconduct that is demonstrably and materially injurious to our reputation, financial condition, or business relationships; (2) failure to attempt in good faith to follow the legal written direction of our board of directors; (3) failure to attempt in good faith to perform his or her duties (other than any such failure resulting from incapacity due to physical or mental illness) after receiving a written demand for substantial performance from our board of directors; (4) conviction of, indictment for, or a plea of guilty or nolo contendere to, a felony or any crime involving dishonesty, fraud or moral turpitude; (5) dishonesty with regard to us or in the performance of his or her duties hereunder, which in either case has a material adverse effect; or (6) material breach of his or her agreement unless corrected within ten days of written notification of such breach from us. For purposes of Mr. Farrell and Dr. Moseley's employment agreements, cause also includes the failure to comply in any material respect with our policies and/or procedures, unless corrected within ten days of written notification to the executive of such breach.

In addition, "good reason" generally means the following events, conditions or actions taken by us with respect to the executive without cause and without the executive's express written consent: (1) any reduction in base compensation; (2) a material adverse change in any of the terms of his or her agreement, including title, status, authority, duties and responsibilities; (3) our failure to obtain a satisfactory agreement from any successor of the company requiring such successor to assume and agree to perform our obligations under his or her employment or consulting agreement; or (4) our failure to comply with any material provision of his or her employment or consulting agreement.

Under the terms of each named executive officer's and Mr. Musso's employment agreement described above that becomes effective in connection with the execution and delivery of the underwriting agreement related to this offering

**Table of Contents**

or that we intend to enter into, or the IPO agreements, each officer's employment is or will be at will and may be terminated at any time. Under the IPO agreements, upon a termination without cause or resignation for good reason (each as defined below), each of Mr. Farrell, Dr. Moseley, Dr. Slawin and Mr. Musso is or will be eligible to receive payments equal to his or her base salary and COBRA premium payments for 12 months and a pro-rated annual performance bonus. If such termination without cause or resignation for good reason occurs immediately prior to, on or within the 12 months following a change of control (as defined in the 2014 Plan), instead of the benefits described above, the officer will be eligible to receive (1) continued base salary payments and COBRA premium payments for 18 months for Mr. Farrell and 12 months for Dr. Moseley, Dr. Slawin and Mr. Musso, (2) a lump sum payment equal to the officer's full target bonus for the year of termination and (3) full vesting acceleration of all outstanding equity awards that are subject to time-based vesting. All severance benefits under the IPO agreements are contingent upon the officer executing an effective release and waiver of claims against us as well as complying with certain other post-termination obligations to us.

For purposes of the IPO agreements, cause generally has the same meaning as described above under Mr. Farrell and Dr. Moseley's employment agreements in place prior to the IPO agreements. Good reason under the IPO agreements generally means the following events, conditions or actions taken by us with respect to the executive without the executive's written consent: (1) a material reduction in base salary; (2) a material reduction in

the executive's authority, duties or responsibilities; (3) a relocation of the executive's principal place of employment to a place that increases the executive's one-way commute by more than 50 miles; or (4) our material breach of any material provision of the amended agreement.

Each of our named executive officers holds stock options under our equity incentive plans that were granted subject to the general terms of our equity incentive plans and form of stock option agreements. A description of the termination and change of control provisions in such equity incentive plans and stock options granted thereunder is provided below under Equity Benefit Plans and the specific vesting terms of each named executive officer's stock options are described below under Outstanding Equity Awards at Fiscal Year-End.

**Outstanding Equity Awards at Fiscal Year-End**

The following table sets forth certain information regarding equity awards granted to our named executive officers that remain outstanding as of December 31, 2013.

	OPTION AWARDS <sup>(1)</sup>				
	GRANT DATE	NUMBER OF SECURITIES UNDERLYING UNEXERCISED OPTIONS EXERCISABLE (#)	NUMBER OF SECURITIES UNDERLYING UNEXERCISED OPTIONS (#)	OPTION EXERCISE PRICE PER SHARE (\$) <sup>(2)</sup>	OPTION EXPIRATION DATE
Thomas Farrell	12/6/2010 <sup>(3)</sup>	79,686	9,696	\$ 0.51	12/5/2020



Edgar Filing: BELLICUM PHARMACEUTICALS, INC - Form 424B4

	11/9/2011 <sup>(4)</sup>	88,235	29,411	\$ 2.55	11/8/2021
	11/9/2011 <sup>(4)</sup>	105,882	35,294	\$ 2.55	11/8/2021
Annemarie Moseley, Ph.D., M.D.	11/9/2011	45,955	42,279	\$ 2.55	11/8/2021
	8/14/2013 <sup>(5)</sup>	15,931	42,892	\$ 2.55	8/13/2023
Kevin M. Slawin, M.D.	12/6/2010	36,176	12,058	\$ 0.51	12/5/2020
	11/9/2011 <sup>(4)</sup>	194,117	64,705	\$ 2.55	11/8/2021
	8/14/2013 <sup>(6)</sup>	120,000	232,941	\$ 2.55	8/13/2023

- (1) All of the option awards were granted under the 2006 Plan and the 2011 Plan, the terms of which plans and option agreements are described below under Equity Benefit Plans.
- (2) All of the option awards were granted with a per share exercise price not less than the fair market value of one share of our common stock on the date of grant, as determined in good faith by our board of directors. Unless otherwise noted, all options granted provide for the following standard vesting schedule: 25% of the shares subject to the option vest on the 12-month anniversary of the grant date and 1/36th of the remaining shares subject to the option vest in equal monthly installments over the next three years subject to the officer's continued service to us.
- (3) 50,588 shares subject to the option vest on the grant date, 9,705 shares subject to the option vest on the 12-month anniversary of the grant date, and 808 shares subject to the option vest in equal monthly installments over the remaining three years.

## **Table of Contents**

- (4) 25% of the shares subject to the option vest on the grant date and 25% of the remaining shares subject to the option vest in equal annual installments over the next three years.
- (5) The shares vest according to the standard vesting schedule, measured from November 26, 2012.
- (6) 34% of the shares subject to the option vest on July 31, 2013 and 33% of the remaining shares subject to the option vest in equal annual installments over the next two years and the shares will continue to vest following termination of Dr. Slawin's service without cause, for good reason or upon death or disability.

## **Perquisites Health, Welfare and Retirement Benefits**

All of our current named executive officers, other than Dr. Slawin, are eligible to participate in our employee benefit plans, including our medical, dental, vision, life, disability and accidental death and dismemberment insurance plans, in each case on the same basis as all of our other employees. We pay the premiums for the life, disability, accidental death and dismemberment insurance for all of our employees, including our named executive officers. In addition, we provide a 401(k) plan to our employees, including our named executive officers, as discussed in the section below entitled 401(k) Plan. We do not provide perquisites or personal benefits to our named executive officers.

### **401(k) Plan**

We maintain a defined contribution employee retirement plan, or 401(k) plan, for our employees. Our named executive officers, other than Dr. Slawin, are also eligible to participate in the 401(k) plan on the same basis as our other employees. The 401(k) plan is intended to qualify as a tax-qualified plan under Section 401(k) of the Code. The plan provides that each participant may contribute 100% of his or her eligible compensation or the statutory limit, which is \$17,500 for calendar year 2013. Participants that are 50 years or older can also make catch-up contributions, which in calendar year 2013 may be up to an additional \$5,500 above the statutory limit. We may also elect to provide for discretionary profit sharing contributions, but we did not provide any such contributions in 2013. In general, eligible compensation for purposes of the 401(k) plan includes an employee's earnings reportable on IRS Form W-2 subject to certain adjustments and exclusions required under the Code. The 401(k) plan currently does not offer the ability to invest in our securities.

### **Nonqualified Deferred Compensation**

None of our named executive officers participate in or have account balances in nonqualified defined contribution plans or other nonqualified deferred compensation plans maintained by us. Our board of directors may elect to provide our officers and other employees with nonqualified defined contribution or other nonqualified deferred compensation benefits in the future if it determines that doing so is in our best interests.

### **Equity Benefit Plans**

#### ***2014 Equity Incentive Plan***

Our board of directors adopted the 2014 Plan in December 2014 and our stockholders approved the 2014 Plan in December 2014, which will become effective upon the execution and delivery of the underwriting agreement related to this offering. The 2014 Plan is a successor to and continuation of our 2011 Plan. Once the 2014 Plan is effective, no further grants will be made under the 2011 Plan.

*Stock Awards.* The 2014 Plan provides for the grant of incentive stock options, or ISOs, nonstatutory stock options, or NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards, performance-based stock awards, and other forms of equity compensation, or collectively, stock awards, all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. Additionally, the 2014

Plan provides for the grant of performance cash awards. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

*Share Reserve.* Initially, the aggregate number of shares of our common stock that may be issued pursuant to stock awards under the 2014 Plan after the 2014 Plan becomes effective is the sum of (1) 2,600,000 shares, plus (2) the number of shares (not to exceed 3,216,795 shares) (i) reserved for issuance under our 2011 Plan at the time our 2014 Plan becomes effective, and (ii) any shares subject to outstanding stock options or other stock awards that were granted under our 2011 Plan or 2006 Plan that are forfeited, terminate, expire or are otherwise not issued. The maximum number of shares of our common stock that may be issued upon the exercise of ISOs under our 2014 Plan is 5,200,000 shares. On December 4, 2014, our board of directors approved the grant of stock options to

## Table of Contents

Mr. Musso and Mr. Stonehouse to purchase an aggregate of 141,176 shares of common stock and a restricted stock award to Mr. Musso covering 117,647 shares of our common stock under the 2014 Plan. These awards will be granted effective and contingent upon the execution and delivery of the underwriting agreement relating to this offering, and with respect to the options, will have a per share exercise price equal to the price per share at which our common stock is first sold to the public in this offering.

No person may be granted stock awards covering more than 1,000,000 shares of our common stock under our 2014 Plan during any calendar year pursuant to stock options, stock appreciation rights and other stock awards whose value is determined by reference to an increase over an exercise or strike price of at least 100% of the fair market value on the date the stock award is granted. Additionally, no person may be granted in a calendar year a performance stock award covering more than 1,000,000 shares of our common stock or a performance cash award having a maximum value in excess of \$3,000,000. Such limitations are designed to help assure that any deductions to which we would otherwise be entitled with respect to such awards will not be subject to the \$1,000,000 limitation on the income tax deductibility of compensation paid to any covered executive officer imposed by Section 162(m) of the Code.

If a stock award granted under the 2014 Plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the 2014 Plan. In addition, the following types of shares of our common stock under the 2014 Plan may become available for the grant of new stock awards under the 2014 Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Shares issued under the 2014 Plan may be previously unissued shares or reacquired shares bought by us on the open market. As of the date hereof, no awards have been granted and no shares of our common stock have been issued under the 2014 Plan.

*Administration.* Our board of directors, or a duly authorized committee thereof, has the authority to administer the 2014 Plan. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, and (2) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the 2014 Plan, our board of directors or the authorized committee, referred to herein as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under our 2014 Plan. Subject to the terms of our 2014 Plan, the plan administrator has the authority to reduce the exercise, purchase or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

*Stock Options.* ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2014 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2014 Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the 2014 Plan, up to a maximum of ten years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service

relationship with us, or any of our affiliates, ceases for any reason other than disability, death or cause, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. The option term may be extended in the event that exercise of the option following such a termination of service is prohibited by applicable securities laws or our insider trading policy. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain

## Table of Contents

period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, and (5) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder's death.

*Tax Limitations On Incentive Stock Options.* The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the term of the ISO does not exceed five years from the date of grant.

*Restricted Stock Awards.* Restricted stock awards are granted pursuant to restricted stock award agreements adopted by the plan administrator. Restricted stock awards may be granted in consideration for (1) cash, check, bank draft or money order, (2) services rendered to us or our affiliates, or (3) any other form of legal consideration. Common stock acquired under a restricted stock award may, but need not, be subject to a share repurchase option in our favor in accordance with a vesting schedule to be determined by the plan administrator. A restricted stock award may be transferred only upon such terms and conditions as set by the plan administrator. Except as otherwise provided in the applicable award agreement, restricted stock awards that have not vested may be forfeited or repurchased by us upon the participant's cessation of continuous service for any reason.

*Restricted Stock Unit Awards.* Restricted stock unit awards are granted pursuant to restricted stock unit award agreements adopted by the plan administrator. Restricted stock unit awards may be granted in consideration for any form of legal consideration. A restricted stock unit award may be settled by cash, delivery of stock, a combination of cash and stock as deemed appropriate by the plan administrator, or in any other form of consideration set forth in the restricted stock unit award agreement. Additionally, dividend equivalents may be credited in respect of shares covered by a restricted stock unit award. Except as otherwise provided in the applicable award agreement, restricted stock units that have not vested will be forfeited upon the participant's cessation of continuous service for any reason.

*Stock Appreciation Rights.* Stock appreciation rights are granted pursuant to stock appreciation grant agreements adopted by the plan administrator. The plan administrator determines the strike price for a stock appreciation right, which generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Upon the exercise of a stock appreciation right, we will pay the participant an amount equal to the product of (1) the excess of the per share fair market value of our common stock on the date of exercise over the strike price, multiplied by (2) the number of shares of common stock with respect to which the stock appreciation right is exercised. A stock appreciation right granted under the 2014 Plan vests at the rate specified in the stock appreciation right agreement as determined by the plan administrator.

The plan administrator determines the term of stock appreciation rights granted under the 2014 Plan, up to a maximum of ten years. Unless the terms of a participant's stock appreciation right agreement provides otherwise, if a participant's service relationship with us or any of our affiliates ceases for any reason other than cause, disability or death, the participant may generally exercise any vested stock appreciation right for a period of three months following the cessation of service. The stock appreciation right term may be further extended in the event that exercise of the stock appreciation right following such a termination of service is prohibited by applicable securities laws. If a participant's service relationship with us, or any of our affiliates, ceases due to disability or death, or a participant dies within a certain period following cessation of service, the participant or a beneficiary may generally exercise any

**Table of Contents**

vested stock appreciation right for a period of 12 months in the event of disability and 18 months in the event of death. In the event of a termination for cause, stock appreciation rights generally terminate immediately upon the occurrence of the event giving rise to the termination of the individual for cause. In no event may a stock appreciation right be exercised beyond the expiration of its term.

*Performance Awards.* The 2014 Plan permits the grant of performance-based stock and cash awards that may qualify as performance-based compensation that is not subject to the \$1,000,000 limitation on the income tax deductibility of compensation paid to a covered executive officer imposed by Section 162(m) of the Code. To help assure that the compensation attributable to performance-based awards will so qualify, our compensation committee can structure such awards so that stock or cash will be issued or paid pursuant to such award only after the achievement of certain pre-established performance goals during a designated performance period.

The performance goals that may be selected include one or more of the following: (1) earnings (including earnings per share and net earnings); (2) earnings before interest, taxes and depreciation; (3) earnings before interest, taxes, depreciation and amortization; (4) earnings before interest, taxes, depreciation, amortization and legal settlements; (5) earnings before interest, taxes, depreciation, amortization, legal settlements and other income (expense); (6) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense) and stock-based compensation; (7) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation and changes in deferred revenue; (8) earnings before interest, taxes, depreciation, amortization, legal settlements, other income (expense), stock-based compensation, other non-cash expenses and changes in deferred revenue; (9) total stockholder return; (10) return on equity or average stockholder return; (11) return on assets, investment, or capital employed; (12) stock price; (13) margin (including gross margin); (14) income (before or after taxes); (15) operating income; (16) operating income after taxes; (17) pre-tax profit; (18) operating cash flow; (19) sales or revenue targets; (20) increases in revenue or product revenue; (21) expenses and cost reduction goals; (22) improvement in or attainment of working capital levels; (23) economic value added (or an equivalent metric); (24) market share; (25) cash flow; (26) cash flow per share; (27) cash balance; (28) cash burn; (29) cash collections; (30) share price performance; (31) debt reduction; (32) implementation or completion of projects or processes (including, without limitation, clinical trial initiation, clinical trial enrollment and dates, clinical trial results, regulatory filing submissions, regulatory filing acceptances, regulatory or advisory committee interactions, regulatory approvals, and product supply); (33) stockholders' equity; (34) capital expenditures; (35) debt levels; (36) operating profit or net operating profit; (37) workforce diversity; (38) growth of net income or operating income; (39) billings; (40) bookings; (41) employee retention; (42) initiation of studies by specific dates; (43) budget management; (44) submission to, or approval by, a regulatory body (including, but not limited to the FDA) of an applicable filing or a product; (45) regulatory milestones; (46) progress of internal research or development programs; (47) acquisition of new customers; (48) customer retention and/or repeat order rate; (49) improvements in sample and test processing times; (50) progress of partnered programs; (51) partner satisfaction; (52) timely completion of clinical trials; (53) submission of 510(k)s or pre-market approvals and other regulatory achievements; (54) milestones related to samples received and/or tests or panels run; (55) expansion of sales in additional geographies or markets; (56) research progress, including the development of programs; (57) strategic partnerships or transactions (including in-licensing and out-licensing of intellectual property); and (58) and to the extent that an award is not intended to comply with Section 162(m) of the Code, other measures of performance selected by our board of directors.

The performance goals may be based on a company-wide basis, with respect to one or more business units, divisions, affiliates, or business segments, and in either absolute terms or relative to the performance of one or more comparable companies or the performance of one or more relevant indices. Unless specified otherwise (i) in the award agreement at the time the award is granted or (ii) in such other document setting forth the performance goals at the time the goals are established, we will appropriately make adjustments in the method of calculating the attainment of performance goals as follows: (1) to exclude restructuring and/or other nonrecurring charges; (2) to exclude exchange rate effects;



(3) to exclude the effects of changes to generally accepted accounting principles; (4) to exclude the effects of any statutory adjustments to corporate tax rates; (5) to exclude the effects of any extraordinary items as determined under generally accepted accounting principles; (6) to exclude the dilutive effects of acquisitions or joint ventures; (7) to assume that any business divested by us achieved performance objectives at targeted levels during the balance of a performance period following such divestiture; (8) to exclude the

**Table of Contents**

effect of any change in the outstanding shares of our common stock by reason of any stock dividend or split, stock repurchase, reorganization, recapitalization, merger, consolidation, spin-off, combination or exchange of shares or other similar corporate change, or any distributions to common stockholders other than regular cash dividends; (9) to exclude the effects of stock-based compensation and the award of bonuses under our bonus plans; (10) to exclude costs incurred in connection with potential acquisitions or divestitures that are required to be expensed under generally accepted accounting principles; (11) to exclude the goodwill and intangible asset impairment charges that are required to be recorded under generally accepted accounting principles; (12) to exclude the effect of any other unusual, non-recurring gain or loss or other extraordinary item; and (13) to exclude the effects of the timing of acceptance for review and/or approval of submissions to the FDA or any other regulatory body. In addition, we retain the discretion to reduce or eliminate the compensation or economic benefit due upon attainment of the performance goals and to define the manner of calculating the performance criteria we select to use for such performance period. The performance goals may differ from participant to participant and from award to award.

*Other Stock Awards.* The plan administrator may grant other awards based in whole or in part by reference to our common stock. The plan administrator will set the number of shares under the stock award and all other terms and conditions of such awards.

*Changes to Capital Structure.* In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to (1) the class and maximum number of shares reserved for issuance under the 2014 Plan, (2) the class and maximum number of shares that may be issued upon the exercise of ISOs, (3) the class and maximum number of shares subject to stock awards that can be granted in a calendar year (as established under the 2014 Plan pursuant to Section 162(m) of the Code) and (4) the class and number of shares and exercise price, strike price, or purchase price, if applicable, of all outstanding stock awards.

*Corporate Transactions.* In the event of certain specified significant corporate transactions, the plan administrator has the discretion to take any of the following actions with respect to stock awards:

- n arrange for the assumption, continuation or substitution of a stock award by a surviving or acquiring entity or parent company;
- n arrange for the assignment of any reacquisition or repurchase rights held by us to the surviving or acquiring entity or parent company;
- n accelerate the vesting of the stock award and provide for its termination at or prior to the effective time of the corporate transaction;
- n arrange for the lapse of any reacquisition or repurchase right held by us;
- n cancel or arrange for the cancellation of the stock award in exchange for such cash consideration, if any, as our board of directors may deem appropriate; or

n make a payment equal to the excess of (1) the value of the property the participant would have received upon exercise of the stock award over (2) the exercise price otherwise payable in connection with the stock award. Our plan administrator is not obligated to treat all stock awards, even those that are of the same type, in the same manner.

Under the 2014 Plan, a corporate transaction is generally the consummation of (1) a sale or other disposition of all or substantially all of our assets, (2) a sale or other disposition of at least 90% of our outstanding securities, (3) a merger, consolidation or similar transaction following which we are not the surviving corporation, or (4) a merger, consolidation or similar transaction following which we are the surviving corporation but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction.

*Change of Control.* The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. For example, certain of our employees, including our named executive officers and Mr. Musso, may receive an award agreement that provides for vesting acceleration upon the individual's termination without cause or resignation for good reason (including a material reduction in the individual's base salary, duties, responsibilities or authority, or a material relocation of the individual's principal

## **Table of Contents**

place of employment with us) in connection with a change of control. Under the 2014 Plan, a change of control is generally (1) the acquisition by a person or entity of more than 50% of our combined voting power other than by merger, consolidation or similar transaction; (2) a consummated merger, consolidation or similar transaction immediately after which our stockholders cease to own more than 50% of the combined voting power of the surviving entity; (3) a consummated sale, lease or exclusive license or other disposition of all or substantially of our assets; (4) our complete dissolution or liquidation, except for a liquidation into a parent corporation; or (5) when a majority of our board of directors becomes comprised of individuals who were not serving on our board of directors on the date of adoption of the 2014 Plan, or the incumbent board, or whose nomination, appointment, or election was not approved by a majority of the incumbent board still in office.

*Amendment and Termination.* Our board of directors has the authority to amend, suspend, or terminate our 2014 Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent. No ISOs may be granted after the tenth anniversary of the date our board of directors adopted our 2014 Plan.

### ***2006 Stock Option Plan***

Our board of directors and our stockholders approved our 2006 Plan, which became effective in February 2006, and was further amended by our board of directors and stockholders most recently in November 2011. As of September 30, 2014, there were 5,882 shares remaining available for the grant of stock awards under our 2006 Plan and there were outstanding stock awards covering a total of 167,056 shares that were granted under our 2006 Plan.

In October 2014, our board of directors terminated the 2006 Plan and no additional awards will be granted under the 2006 Plan. All awards granted under the 2006 Plan that are repurchased, forfeited, expired or are cancelled on or after the effective date of the 2014 Plan will become available for grant under the 2014 Plan in accordance with its terms.

*Stock Awards.* The 2006 Plan provides for the grant of ISO, NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards and other forms of stock awards, or collectively, stock awards, all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

*Share Reserve.* The aggregate number of shares of our common stock reserved for issuance pursuant to stock awards under the 2006 Plan is 177,352 shares. The initial number of shares we reserved for issuance pursuant to stock awards under the 2006 Plan was 70,588 shares, which was increased in November 2006 to 177,352 shares. The maximum number of shares that may be issued upon the exercise of ISOs under our 2006 Plan is 177,352 shares.

If a stock award granted under the 2006 Plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the 2006 Plan. In addition, the following types of shares under the 2006 Plan may become available for the grant of new stock awards under the 2006 Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award.

*Administration.* Our board of directors, or a duly authorized committee thereof, has the authority to administer the 2006 Plan. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, and (2) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the 2006 Plan, our board of directors or

the authorized committee, referred to herein as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.

The plan administrator has the authority to modify outstanding awards under our 2006 Plan. Subject to the terms of our 2006 Plan, the plan administrator has the authority to reduce the exercise, purchase or strike price of any

## **Table of Contents**

outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

*Stock Options.* ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2006 Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2006 Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the 2006 Plan, up to a maximum of ten years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service relationship with us, or any of our affiliates, ceases for any reason other than disability, voluntary termination, death or cause, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability or death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and included in the option agreement and may include cash or cashier's check, check, bank draft or money order, or a cashless exercise.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder's death.

*Tax Limitations On Incentive Stock Options.* The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the option is not exercisable after the expiration of five years from the date of grant.

*Changes to Capital Structure.* In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to the number and kind of shares subject to the plan, and the option prices, so as to maintain the proportionate number of shares without changing the aggregate option price.

*Corporate Transactions.* In the event of certain significant corporate transactions, including a recapitalization or other change in capital structure, merger, consolidation, sale of all assets, or dissolution other than a change in control (as defined below), any holder of options under the 2006 Plan may be entitled to purchase the number and class of shares resulting from such corporate transactions equivalent to the number and class of shares to which the optionholder would have been entitled prior to the occurrence of such transactions.

*Change of Control.* The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. Under the 2006 Plan, a change of control is generally defined as (1) a merger in which we are not the surviving entity and the members of our board of directors do not constitute a majority of the board of directors of the successor entity, (2) a dissolution or liquidation or (3) a consummated sale, lease or exchange of all or substantially all of our assets.

In November 2011 our board of directors amended the 2006 Plan to include, among other things, a restated definition of a change in control. Under the amendment to the 2006 Plan, a change of control is generally defined

## **Table of Contents**

as (1) a merger or other reorganization in which we are not the surviving entity, (2) a sale, lease or exclusive license or exchange of all or substantially all of our assets, (3) a dissolution or liquidation, or (4) if any person or entity, including a group as contemplated by Section 13(d)(3) of the 1934 Act, acquires or gains ownership or control of more than 50% of our outstanding shares of voting stock, (5) as a result or in connection with a contested election of directors, if the members of our board of directors before such election do not constitute a majority of the board of directors after such election.

### ***2011 Stock Option Plan***

Our board of directors and our stockholders approved our 2011 Plan, which became effective in November 2011, and was further amended by our board of directors and stockholders most recently in February 2014. As of September 30, 2014, there were 1,382,481 shares remaining available for the grant of stock awards under our 2011 Plan and there were outstanding stock awards covering a total of 1,435,283 shares that were granted under our 2011 Plan. Subsequent to September 30, 2014, and prior to this offering, additional stock options were granted under our 2011 Plan for an aggregate of 1,031,454 shares.

After the effective date of the 2014 Plan, no additional awards will be granted under the 2011 Plan, and all awards granted under the 2011 Plan that are repurchased, forfeited, expire or are cancelled will become available for grant under the 2014 Plan in accordance with its terms.

*Stock Awards.* The 2011 Plan provides for the grant of ISO, NSOs, stock appreciation rights, restricted stock awards, restricted stock unit awards and other forms of stock awards, or collectively, stock awards, all of which may be granted to employees, including officers, non-employee directors and consultants of us and our affiliates. ISOs may be granted only to employees. All other awards may be granted to employees, including officers, and to non-employee directors and consultants.

*Share Reserve.* The aggregate number of shares of our common stock reserved for issuance pursuant to stock awards under the 2011 Plan is 2,822,647 shares. The initial number of shares we reserved for issuance pursuant to stock awards under the 2011 Plan was 999,117 shares, which was increased in August 2014 to 2,822,647 shares. The maximum number of shares that may be issued upon the exercise of ISOs under our 2011 Plan is 2,822,647 shares.

If a stock award granted under the 2011 Plan expires or otherwise terminates without being exercised in full, or is settled in cash, the shares of our common stock not acquired pursuant to the stock award again will become available for subsequent issuance under the 2011 Plan. In addition, the following types of shares under the 2011 Plan may become available for the grant of new stock awards under the 2011 Plan: (1) shares that are forfeited to or repurchased by us prior to becoming fully vested; (2) shares withheld to satisfy income or employment withholding taxes; or (3) shares used to pay the exercise or purchase price of a stock award. Shares issued under the 2011 Plan may be previously unissued shares or reacquired shares bought by us on the open market.

*Administration.* Our board of directors, or a duly authorized committee thereof, has the authority to administer the 2011 Plan. Our board of directors may also delegate to one or more of our officers the authority to (1) designate employees (other than other officers) to be recipients of certain stock awards, and (2) determine the number of shares of common stock to be subject to such stock awards. Subject to the terms of the 2011 Plan, our board of directors or the authorized committee, referred to herein as the plan administrator, determines recipients, dates of grant, the numbers and types of stock awards to be granted and the terms and conditions of the stock awards, including the period of their exercisability and vesting schedule applicable to a stock award. Subject to the limitations set forth below, the plan administrator will also determine the exercise price, strike price or purchase price of awards granted and the types of consideration to be paid for the award.



The plan administrator has the authority to modify outstanding awards under our 2011 Plan. Subject to the terms of our 2011 Plan, the plan administrator has the authority to reduce the exercise, purchase or strike price of any outstanding stock award, cancel any outstanding stock award in exchange for new stock awards, cash or other consideration, or take any other action that is treated as a repricing under generally accepted accounting principles, with the consent of any adversely affected participant.

*Stock Options.* ISOs and NSOs are granted pursuant to stock option agreements adopted by the plan administrator. The plan administrator determines the exercise price for a stock option, within the terms and conditions of the 2011

## **Table of Contents**

Plan, provided that the exercise price of a stock option generally cannot be less than 100% of the fair market value of our common stock on the date of grant. Options granted under the 2011 Plan vest at the rate specified by the plan administrator.

The plan administrator determines the term of stock options granted under the 2011 Plan, up to a maximum of ten years. Unless the terms of an optionholder's stock option agreement provide otherwise, if an optionholder's service relationship with us, or any of our affiliates, ceases for any reason other than disability or death, the optionholder may generally exercise any vested options for a period of three months following the cessation of service. If an optionholder's service relationship with us or any of our affiliates ceases due to disability or death, or an optionholder dies within a certain period following cessation of service, the optionholder or a beneficiary may generally exercise any vested options for a period of 12 months in the event of disability or death. In the event of a termination for cause, options generally terminate immediately upon the termination of the individual for cause. In no event may an option be exercised beyond the expiration of its term.

Acceptable consideration for the purchase of common stock issued upon the exercise of a stock option will be determined by the plan administrator and included in the option agreement and may include (1) cash, check, bank draft or money order, (2) a broker-assisted cashless exercise, (3) the tender of shares of our common stock previously owned by the optionholder, (4) a net exercise of the option if it is an NSO, (5) a deferred payment or similar arrangement subject to certain conditions and (6) other legal consideration approved by the plan administrator.

Unless the plan administrator provides otherwise, options generally are not transferable except by will, the laws of descent and distribution, or pursuant to a domestic relations order. An optionholder may designate a beneficiary, however, who may exercise the option following the optionholder's death.

*Tax Limitations On Incentive Stock Options.* The aggregate fair market value, determined at the time of grant, of our common stock with respect to ISOs that are exercisable for the first time by an optionholder during any calendar year under all of our stock plans may not exceed \$100,000. Options or portions thereof that exceed such limit will generally be treated as NSOs. No ISO may be granted to any person who, at the time of the grant, owns or is deemed to own stock possessing more than 10% of our total combined voting power or that of any of our affiliates unless (1) the option exercise price is at least 110% of the fair market value of the stock subject to the option on the date of grant, and (2) the option is not exercisable after the expiration of five years from the date of grant.

*Changes to Capital Structure.* In the event that there is a specified type of change in our capital structure, such as a stock split or recapitalization, appropriate adjustments will be made to the number and kind of shares subject to the plan, and the exercise prices, so as to maintain the proportionate number of shares without changing the aggregate exercise price.

*Corporate Transactions.* In the event of certain significant corporate transactions, including a merger, consolidation, sale of all assets, or dissolution, any holder of options under the 2006 Plan may be entitled to purchase the number and class of shares resulting from such corporate transactions equivalent to the number and class of shares to which the optionholder would have been entitled prior to the occurrence of such transactions.

*Change of Control.* The plan administrator may provide, in an individual award agreement or in any other written agreement between a participant and us that the stock award will be subject to additional acceleration of vesting and exercisability in the event of a change of control. Under the 2011 Plan, a change of control is generally defined as (1) a merger or other reorganization in which we are not the surviving entity, (2) a sale, lease or exclusive license or exchange of all or substantially all of our assets, (3) a dissolution or liquidation, or (4) if any person or entity, including a group as contemplated by Section 13(d)(3) of the 1934 Act, acquires or gains ownership or control of

more than 50% of our outstanding shares of voting stock, (5) as a result or in connection with a contested election of directors, if the members of our board of directors before such election do not constitute a majority of the board of directors after such election.

Under our form of stock option award agreement, upon a change of control, all options will immediately vest and become exercisable. Beginning with options granted in October 2014, we amended this form of option award agreement to provide that all options will immediately vest and become exercisable only upon both (1) a change of control and (2) the optionholder's termination without cause or resignation for good reason (including a material reduction in base salary, authorities, duties or responsibilities, failure by us to continue a material benefit plan or program, or a relocation of principle place of employment) within the 12 months following such change of control.

## **Table of Contents**

*Amendment and Termination.* The 2011 Plan will terminate on November 9, 2021. However, our board of directors has the authority to amend, suspend, or terminate our 2011 Plan, provided that such action does not materially impair the existing rights of any participant without such participant's written consent.

### ***2014 Employee Stock Purchase Plan***

Our board of directors adopted the ESPP in December 2014 and our stockholders approved the ESPP in December 2014. The ESPP will become effective immediately upon the execution and delivery of the underwriting agreement related to this offering. The purpose of the ESPP is to retain the services of new employees and secure the services of new and existing employees while providing incentives for such individuals to exert maximum efforts toward our success and that of our affiliates.

*Share Reserve.* Following this offering, the ESPP authorizes the issuance of 550,000 shares of our common stock pursuant to purchase rights granted to our employees or to employees of any of our designated affiliates. The ESPP is intended to qualify as an employee stock purchase plan within the meaning of Section 423 of the Code. As of the date hereof, no shares of our common stock have been purchased under the ESPP.

*Administration.* Our board of directors has delegated its authority to administer the ESPP to our compensation committee. The ESPP is implemented through a series of offerings of purchase rights to eligible employees. Under the ESPP, we may specify offerings with durations of not more than 27 months, and may specify shorter purchase periods within each offering. Each offering will have one or more purchase dates on which shares of our common stock will be purchased for employees participating in the offering. An offering may be terminated under certain circumstances.

*Payroll Deductions.* Generally, all regular employees, including executive officers, employed by us or by any of our designated affiliates, may participate in the ESPP and may contribute, normally through payroll deductions, up to 15% of their earnings for the purchase of our common stock under the ESPP. Unless otherwise determined by our board of directors, common stock will be purchased for accounts of employees participating in the ESPP at a price per share equal to the lower of (1) 85% of the fair market value of a share of our common stock on the first date of an offering or (2) 85% of the fair market value of a share of our common stock on the date of purchase.

*Limitations.* Employees may have to satisfy one or more of the following service requirements before participating in the ESPP, as determined by our board of directors: (1) customarily employed for more than 20 hours per week, (2) customarily employed for more than five months per calendar year, or (3) continuous employment with us or one of our affiliates for a period of time (not to exceed two years). No employee may purchase shares under the ESPP at a rate in excess of \$25,000 worth of our common stock based on the fair market value per share of our common stock at the beginning of an offering for each year such a purchase right is outstanding. Finally, no employee will be eligible for the grant of any purchase rights under the ESPP if immediately after such rights are granted, such employee has voting power over 5% or more of our outstanding capital stock measured by vote or value pursuant to Section 424(d) of the Code.

*Changes to Capital Structure.* In the event that there occurs a change in our capital structure through such actions as a stock split, merger, consolidation, reorganization, recapitalization, reincorporation, stock dividend, dividend in property other than cash, large nonrecurring cash dividend, liquidating dividend, combination of shares, exchange of shares or change in corporate structure or similar transaction, the board of directors will make appropriate adjustments to (1) the number of shares reserved under the ESPP, and (2) the number of shares and purchase price of all outstanding purchase rights.

*Corporate Transactions.* In the event of certain significant corporate transactions, including the consummation of: (1) a sale of all our assets, (2) the sale or disposition of 90% of our outstanding securities, (3) a merger or consolidation where we do not survive the transaction, and (4) a merger or consolidation where we do survive the transaction but the shares of our common stock outstanding immediately prior to such transaction are converted or exchanged into other property by virtue of the transaction, any then-outstanding rights to purchase our stock under the ESPP may be assumed, continued or substituted for by any surviving or acquiring entity (or its parent company). If the surviving or acquiring entity (or its parent company) elects not to assume, continue or substitute for such purchase rights, then the participants' accumulated payroll contributions will be used to purchase shares of our common stock within ten business days prior to such corporate transaction, and such purchase rights will terminate immediately.

---

**Table of Contents**

*Plan Amendments, Termination.* Our board of directors has the authority to amend or terminate the ESPP, provided that except in certain circumstances any such amendment or termination may not materially impair any outstanding purchase rights without the holder's consent. We will obtain stockholder approval of any amendment to the ESPP as required by applicable law or listing requirements.

**Non-Employee Director Compensation**

With the exception of Dr. Slawin, who is compensated under a consulting agreement as described above, we have not historically paid cash or equity compensation to directors who are also our employees for their service on our board of directors, nor have we paid cash or equity compensation to our non-employee directors who are associated with our principal stockholders for service on our board of directors. We have reimbursed and will continue to reimburse all of our non-employee directors for their travel, lodging and other reasonable expenses incurred in attending meetings of our board of directors and committees of our board of directors. In 2013, we did not pay cash or equity compensation to any of our non-employee directors.

In November 2014, we granted an option to purchase up to 23,529 shares of our common stock to each of Dr. Huber and Messrs. Brown and McGuyer at a \$7.463 exercise price per share under the 2011 Plan that vests over a four year period subject to each individual's continued service with us. In connection with Mr. Stonehouse's commencement of service on our board in December 2014, in December 2014, the board of directors approved an option grant to Mr. Stonehouse to purchase up to 23,529 shares of our common stock. The option will be granted effective and contingent upon the execution and delivery of the underwriting agreement relating to this offering, under our 2014 Plan, at a per share exercise price equal to the price per share at which our common stock is first sold to the public in this offering, subject to vesting over a four year period from his commencement of service, subject to Mr. Stonehouse's continued service with us.

Our board of directors adopted a new compensation policy in December 2014 that will become effective upon the execution and delivery of the underwriting agreement related to this offering and will be applicable to all of our directors who are not serving as our employees or consultants (other than solely as a result of serving on our board), or our eligible directors. This compensation policy provides that each such eligible director will receive the following compensation for service on our board of directors:

- n an annual cash retainer of \$35,000;
- n an additional annual cash retainer of \$25,000 for service as chairman of the board of directors;
- n an additional annual cash retainer of \$15,000 for service as our lead independent director;
- n an additional annual cash retainer of \$7,500, \$5,000 and \$3,500 for service as a member of the audit committee, compensation committee and the nominating and corporate governance committee, respectively;
- n an additional annual cash retainer of \$7,500, \$5,000 and \$4,000 for service as chairman of the audit committee, compensation committee and the nominating and corporate governance committee, respectively;

n an initial option grant to purchase 20,000 shares of our common stock on the date of each new non-employee director's appointment to our board of directors, vesting monthly over a three year period; and

n an annual option grant to purchase 10,000 shares of our common stock on the date of each of our annual stockholder meetings vesting monthly until our next annual meeting.

Each of the initial and annual option grants described above will vest and become exercisable subject to the director's continuous service to us, provided that each option will vest in full upon a change of control (as defined under our 2014 Plan). The term of each option will be 10 years, subject to earlier termination as provided in the 2014 Plan, except that the post-termination exercise period will be for 12 months from the date of termination, if such termination is other than for cause or due to death or disability. The options will be granted under our 2014 Plan, the terms of which are described in more detail above under Equity Benefit Plans 2014 Equity Incentive Plan.

**Table of Contents****CERTAIN RELATIONSHIPS AND RELATED PARTY TRANSACTIONS**

The following includes a summary of transactions since January 1, 2011 to which we have been a party, in which the amount involved in the transaction exceeded \$120,000, and in which any of our directors, executive officers or, to our knowledge, beneficial owners of more than 5% of our capital stock or any member of the immediate family of any of the foregoing persons had or will have a direct or indirect material interest, other than equity and other compensation, termination, change of control and other arrangements, which are described under Executive and Director Compensation.

**Note and Convertible Preferred Stock Financings*****Note Financing***

In February 2013, we issued and sold to investors, including beneficial owners of more than 5% of our capital stock, promissory notes, or the 2013 notes, in the aggregate principal amount of \$3.5 million. The 2013 notes carried an interest rate of 0.21% per annum. In connection with the second closing of our Series B convertible preferred stock financing (discussed below), all of the outstanding principal and accrued and unpaid interest of the 2013 notes were cancelled in exchange for shares of Series B convertible preferred stock. The aggregate of \$3.5 million representing the outstanding principal and accrued and unpaid interest on the notes was exchanged for 757,497 shares of Series B convertible preferred stock at a price of \$4.625 per share

The participants in this note financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table presents the aggregate principal amount of promissory notes issued to these related parties for more than \$120,000:

<b>PARTICIPANTS</b>	<b>AGGREGATE PRINCIPAL AMOUNT OF NOTES</b>	
<b>Greater than 5% stockholders</b>		
McGuyer Investments Ltd.	\$	1,196,580
Remeditex Ventures LLC	\$	1,435,896
AVG Ventures, LP	\$	717,949

Certain of our current and former directors have affiliations with the investors that participated in the note financing described above, as indicated in the table below:



**DIRECTORS**

Frank B. McGuyer  
James Brown  
Dennis Stone, M.D. <sup>(1)</sup>

**PRINCIPAL STOCKHOLDER**

McGuyer Investments Ltd.  
AVG Ventures, LP  
Remeditex Ventures LLC

<sup>(1)</sup> Dr. Stone resigned from our board of directors in November 2014.

***Exchange of Notes for Series A Convertible Preferred Stock***

In October 2009 and March 2010, we issued and sold to investors promissory notes in the aggregate principal amount of \$2.9 million. In November 2011, all of the outstanding principal and accrued and unpaid interest due under these notes were cancelled in exchange for shares of Series A convertible preferred stock in connection with our Series B convertible preferred stock financing (discussed below). The aggregate of \$2.9 million representing the outstanding principal and accrued and unpaid interest on these notes was exchanged for 957,961 shares of Series A convertible preferred stock at a price of \$3.00 per share.

**Table of Contents**

The participants in this convertible preferred stock issuance included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series A convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES A CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
McGuyer Investments Ltd.	76,369
<b>Greater than 5% stockholder, Director and Executive Officer</b>	
Kevin M. Slawin, M.D. <sup>(1)</sup>	28,737

<sup>(1)</sup> Consists of 28,737 shares issued to the 2009 Slawin Family Partnership  
 Certain of our directors have affiliations with the investors that participated in the exchange transaction described above, as indicated in the table below:

**DIRECTORS**  
 Frank B. McGuyer

**PRINCIPAL STOCKHOLDER**  
 McGuyer Investments Ltd.

***Series B Convertible Preferred Stock Financing***

In November 2011 we entered into a Series B convertible preferred stock purchase agreement, or the first Series B purchase agreement, pursuant to which we issued and sold to investors an aggregate of 2,174,824 shares of our Series B convertible preferred stock in two issuances. We received proceeds of approximately \$6.8 million for which we issued an initial 1,475,144 shares of Series B convertible preferred stock, at a purchase price of \$4.625 per share. In addition, the aggregate of approximately \$3.2 million of accrued and unpaid interest on convertible notes issued in September 2010 and December 2010 automatically converted into 699,680 shares of Series B convertible preferred stock at a conversion price equal to \$4.625 per share.

The participants in this convertible preferred stock financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series B convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES B CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
McGuyer Investments Ltd.	680,720
AVG Ventures, LP	324,325
<b>Greater than 5% stockholder, Director and Executive Officer</b>	
Thomas J. Farrell	10,811
Kevin M. Slawin, M.D. <sup>(1)</sup>	42,946

<sup>(1)</sup> Consists of 13,514 shares purchased by the 2009 Slawin Family Partnership  
 Certain of our directors have affiliations with the investors that participated in the convertible preferred stock financing described above, as indicated in the table below:

**DIRECTORS**  
 Frank B. McGuyer  
 James Brown

**PRINCIPAL STOCKHOLDER**  
 McGuyer Investments Ltd.  
 AVG Ventures, LP

**Table of Contents**

In March 2012, we entered into a second Series B stock purchase agreement, or the second Series B stock purchase agreement, with certain new Series B investors, pursuant to which we received proceeds of approximately \$3.1 million for which we issued 675,105 shares of Series B convertible preferred stock, at a purchase price of \$4.625 per share.

The participants in this convertible preferred stock financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series B convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES B CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
Remeditex Ventures LLC	648,649

<sup>(1)</sup> Consists of 13,514 shares purchased by the 2009 Slawin Family Partnership  
Certain of our former directors have affiliations with the investors that participated in the convertible preferred stock financing described above, as indicated in the table below:

**FORMER DIRECTORS**  
Dennis Stone, M.D.

**PRINCIPAL STOCKHOLDER**  
Remeditex Ventures LLC

In July 2013, we entered into a second closing of our Series B financing. Pursuant to our first Series B stock purchase agreement, we received proceeds of approximately \$6.6 million for which we issued 1,431,900 shares of Series B convertible preferred stock at a purchase price of \$4.625 per share. Pursuant to our second Series B stock purchase agreement, we received proceeds of approximately \$3.1 million for which we issued 666,319 shares of Series B convertible preferred stock at a purchase price of \$4.625 per share. In addition, the aggregate of approximately \$3.5 million of accrued and unpaid interest on promissory notes issued in February 2013 were paid with 757,497 shares of Series B convertible preferred stock at a conversion price equal to \$4.625 per share.

The participants in this convertible preferred stock financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series B convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES B CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
McGuyer Investments Ltd.	540,541
AVG Ventures, LP	324,325
Remeditex Ventures LLC	648,649
<b>Greater than 5% stockholder, Director and Executive Officer</b>	
Thomas J. Farrell	10,811
Kevin M. Slawin, M.D. <sup>(1)</sup>	13,514

<sup>(1)</sup> Consists of 13,514 shares purchased by the 2009 Slawin Family Partnership

**Table of Contents**

Certain of our current and former directors have affiliations with the investors that participated in the convertible preferred stock financing described above, as indicated in the table below:

**DIRECTORS**

Frank B. McGuyer  
James Brown  
Dennis Stone, M.D.

**PRINCIPAL STOCKHOLDER**

McGuyer Investments Ltd.  
AVG Ventures, LP  
Remeditex Ventures LLC

In November 2013, we entered into our first over-allotment closing of our Series B financing pursuant to amendments to our first Series B stock purchase agreement and second Series B stock purchase agreement. We received proceeds of approximately \$7.5 million for which we issued 1,615,135 shares of Series B convertible preferred stock at a purchase price of \$4.625 per share.

The participants in this convertible preferred stock financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series B convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES B CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
McGuyer Investments Ltd.	432,432
AVG Ventures, LP	253,378
Remeditex Ventures LLC	506,754
<b>Greater than 5% stockholder, Director and Executive Officer</b>	
Kevin M. Slawin, M.D. <sup>(1)</sup>	43,243

<sup>(1)</sup> Consists of 43,243 shares purchased by the 2009 Slawin Family Partnership

Certain of our current and former directors have affiliations with the investors that participated in the convertible preferred stock financing described above, as indicated in the table below:

**DIRECTORS**

Frank B. McGuyer

James Brown

Dennis Stone, M.D.

**PRINCIPAL STOCKHOLDER**

McGuyer Investments Ltd.

AVG Ventures, LP

Remeditex Ventures LLC

In January 2014, we entered into a second over-allotment closing of our Series B financing pursuant to amendments to our first Series B stock purchase agreement and second Series B stock purchase agreement, in which we received proceeds of approximately \$7.3 million for which we issued 1,582,705 shares of Series B convertible preferred stock at a purchase price of \$4.625 per share.

**Table of Contents**

The participants in this convertible preferred stock financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series B convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES B CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
McGuyer Investments Ltd.	432,432
AVG Ventures, LP	253,377
Remeditex Ventures LLC	506,754
<b>Greater than 5% stockholder, Director and Executive Officer</b>	
Kevin M. Slawin, M.D. <sup>(1)</sup>	10,811

<sup>(1)</sup> Consists of 10,811 shares purchased by the 2009 Slawin Family Partnership

Certain of our current and former directors have affiliations with the investors that participated in the convertible preferred stock financing described above, as indicated in the table below:

**DIRECTORS**

Frank B. McGuyer  
James Brown  
Dennis Stone, M.D.

**PRINCIPAL STOCKHOLDER**

McGuyer Investments Ltd.  
AVG Ventures, LP  
Remeditex Ventures LLC

***Series C Convertible Preferred Stock Financing***

In August 2014, we entered into a Series C convertible preferred stock purchase agreement, or the Series C purchase agreement, pursuant to which we received proceeds of approximately \$55 million for which we issued 10,091,743 shares of Series C convertible preferred stock, at a purchase price of \$5.45 per share.



The participants in this convertible preferred stock financing included the following members of our board of directors and holders of more than 5% of our capital stock or entities affiliated with them. The following table sets forth the aggregate number of shares of Series C convertible preferred stock issued to these related parties in this convertible preferred stock financing:

<b>PARTICIPANTS</b>	<b>SHARES OF SERIES C CONVERTIBLE PREFERRED STOCK</b>
<b>Greater than 5% stockholders</b>	
Baker Biotech Capital, L.P. <sup>(1)</sup>	3,302,752
McGuyer Investments Ltd.	513,133
Remeditex Ventures, LLC	417,009
AVG Ventures, LP	208,508
RA Capital Healthcare Fund, LP	825,688
<b>Greater than 5% stockholder, Director and Executive Officer</b>	
Kevin M. Slawin, M.D. <sup>(2)</sup>	270,133

(1) Consists of 283,820 shares held by 667, L.P., 2,946,332 shares held by Baker Brothers Life Sciences, L.P., and 72,600 shares held by 14159, L.P.

(2) Consists of 38,889 shares held by Kevin M. Slawin, M.D., 109,328 shares held by the Jordana Slawin 2012 Family Trust, 95,283 shares held by the Kevin Slawin 2009 Family Trust, and 26,633 shares held by the 2009 Slawin Family Partnership.

## **Table of Contents**

Certain of our current and former directors have affiliations with the investors that participated in the convertible preferred stock financing described above, as indicated in the table below:

### **DIRECTORS**

Frank B. McGuyer  
James Brown  
Dennis Stone, M.D.

### **PRINCIPAL STOCKHOLDER**

McGuyer Investments Ltd.  
AVG Ventures, LP  
Remeditex Ventures, LLC

## **Consulting Agreements**

In November 2011, we entered into a consulting agreement with Kevin M. Slawin, M.D. to expand Dr. Slawin's role to serve as our Executive Chairman and Chief Medical Officer. While this agreement is still in effect, Dr. Slawin currently serves as a member of our board of directors and holds the titles of Chief Medical Officer and Chief Technology Officer. Per that agreement, Dr. Slawin's annual base compensation was \$250,000.

## **Investor Agreements**

In connection with our convertible preferred stock financings, we entered into an investors' rights agreement containing voting rights, information rights, rights of first refusal and co-sale and registration rights, among other things, with certain holders of our convertible preferred stock and certain holders of our common stock, including all of the holders of more than 5% of our capital stock or entities affiliated with them. In August 2014, this agreement was amended to provide for similar rights to the purchasers of the Series C convertible preferred stock. These rights will terminate upon the closing of this offering, except for the registration rights as more fully described below in Description of Capital Stock Registration Rights.

## **Participation in Offering**

Certain of our existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering.

## **Indemnification Agreements**

We have entered, and intend to continue to enter, into separate indemnification agreements with our directors and executive officers, in addition to the indemnification provided for in our amended and restated bylaws. These agreements, among other things, require us to indemnify our directors and executive officers for certain expenses, including attorneys' fees, judgments, fines and settlement amounts incurred by a director or executive officer in any action or proceeding arising out of their services as one of our directors or executive officers or as a director or

executive officer of any other company or enterprise to which the person provides services at our request. For more information regarding these indemnification arrangements, see Management Limitation on Liability and Indemnification of Directors and Officers. We believe that these bylaw provisions and indemnification agreements are necessary to attract and retain qualified persons as directors and officers.

The limitation of liability and indemnification provisions in our amended and restated certificate of incorporation and amended and restated bylaws may discourage stockholders from bringing a lawsuit against directors for breach of their fiduciary duties. They may also reduce the likelihood of derivative litigation against directors and officers, even though an action, if successful, might benefit us and our stockholders. A stockholder's investment may decline in value to the extent we pay the costs of settlement and damage awards against directors and officers pursuant to these indemnification provisions.

### **Policies and Procedures for Transactions with Related Persons**

We have adopted a written related-person transactions policy that sets forth our policies and procedures regarding the identification, review, consideration and oversight of related-person transactions. For purposes of our policy

**Table of Contents**

only, a related-person transaction is a transaction, arrangement or relationship (or any series of similar transactions, arrangements or relationships) in which we and any related person are participants involving an amount that exceeds \$120,000.

Transactions involving compensation for services provided to us as an employee, consultant or director are not considered related-person transactions under this policy. A related person is any executive officer, director, nominee to become a director or a holder of more than 5% of our common stock, including any of their immediate family members and affiliates, including entities owned or controlled by such persons.

Under the policy, where a transaction has been identified as a related-person transaction, management must present information regarding the proposed related-person transaction to our audit committee (or, where review by our audit committee would be inappropriate, to another independent body of our board of directors) for review. The presentation must include a description of, among other things, all of the parties, the direct and indirect interests of the related persons, the purpose of the transaction, the material facts, the benefits of the transaction to us and whether any alternative transactions are available, an assessment of whether the terms are comparable to the terms available from unrelated third parties and management's recommendation. To identify related-person transactions in advance, we rely on information supplied by our executive officers, directors and certain significant stockholders. In considering related-person transactions, our audit committee or another independent body of our board of directors takes into account the relevant available facts and circumstances including, but not limited to:

- n the risks, costs and benefits to us;
- n the impact on a director's independence in the event the related person is a director, immediate family member of a director or an entity with which a director is affiliated;
- n the terms of the transaction;
- n the availability of other sources for comparable services or products; and
- n the terms available to or from, as the case may be, unrelated third parties.

In the event a director has an interest in the proposed transaction, the director must recuse himself or herself from the deliberations and approval.

---

**Table of Contents**

**PRINCIPAL STOCKHOLDERS**

The following table sets forth information regarding beneficial ownership of our capital stock by:

- n each person, or group of affiliated persons, known by us to beneficially own more than 5% of our common stock
  
- n each of our directors
  
- n each of our named executive officers
  
- n all of our current executive officers and directors as a group

The percentage ownership information under the column entitled "Before Offering" is based on 14,349,205 shares of common stock outstanding as of September 30, 2014, assuming conversion of all outstanding shares of our convertible preferred stock into 12,224,819 shares of common stock. The percentage ownership information under the column entitled "After Offering" is based on the sale of shares of common stock in this offering, and assuming (1) no exercise of the underwriters' option to purchase additional shares, (2) no exercise of outstanding options, (3) the net exercise in full of all outstanding warrants to purchase common stock other than the warrant for 355,392 shares of common stock held by the state of Texas, (4) the cash exercise in full of all outstanding warrants to purchase Series C convertible preferred stock and (5) election by all of the holders of Series B convertible preferred stock to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock, in each case based on the initial public offering price of \$19.00 per share.

Information with respect to beneficial ownership has been furnished by each director, officer or beneficial owner of more than 5% of our common stock. We have determined beneficial ownership in accordance with the rules of the SEC. These rules generally attribute beneficial ownership of securities to persons who possess sole or shared voting power or investment power with respect to those securities. In addition, the rules include shares of our common stock issuable pursuant to the exercise of stock options or warrants that are either immediately exercisable or exercisable within 60 days of September 30, 2014. As noted in the applicable footnotes to the table, some of the options are not vested but are exercisable at any time and, if exercised, subject to a lapsing right of repurchase until the options are fully vested. These shares are deemed to be outstanding and beneficially owned by the person holding those options or warrants for the purpose of computing the percentage ownership of that person, but they are not treated as outstanding for the purpose of computing the percentage ownership of any other person. Unless otherwise indicated, the persons or entities identified in this table have sole voting and investment power with respect to all shares shown as beneficially owned by them, subject to applicable community property laws.

Certain of our existing stockholders and their affiliated entities have indicated an interest in purchasing up to approximately \$50.0 million of shares of our common stock in this offering at the public offering price. However, because indications of interest are not binding agreements or commitments to purchase, these entities may determine to purchase more or fewer shares than they have indicated or not to purchase any shares in this offering. The following table does not reflect any potential purchases by our existing stockholders.



**Table of Contents**

Except as otherwise noted below, the address for each person or entity listed in the table is c/o Bellicum Pharmaceuticals, Inc., 2130 W. Holcombe Blvd., Ste. 800, Houston, TX 77030.

NAME AND ADDRESS OF BENEFICIAL OWNER	NUMBER OF SHARES BENEFICIALLY OWNED		PERCENTAGE OF SHARES BENEFICIALLY OWNED	
	NUMBER OF SHARES BENEFICIALLY OWNED	NUMBER OF SHARES OWNED AFTER THE OFFERING	BEFORE OFFERING	AFTER OFFERING
<b>Greater than 5% stockholders</b>				
Baker Biotech Capital, L.P. <sup>(1)</sup> 667 Madison Avenue, 21st Floor New York, NY 10065	3,205,607	3,205,607	19.4%	11.8%
McGuyer Investments Ltd. <sup>(2)</sup> 11007 Wickwood Dr. Houston, TX 77024	2,170,664	2,214,767	14.7%	8.5%
Remeditex Ventures LLC <sup>(3)</sup> 2101 Cedar Springs Road, Suite 601 Dallas, TX 75201	1,764,041	1,807,604	12.1%	6.9%
Kevin Slawin, M.D. <sup>(4)</sup> AVG Ventures, LP <sup>(5)</sup> 500 Ygnacio Valley Rd., # 360 Walnut Creek, CA 94596	1,685,239	1,687,644	10.9%	6.4%
RA Capital Healthcare Fund, LP <sup>(6)</sup> 20 Park Plaza, Suite 1200 Boston, MA 02116	882,022	905,322	6.1%	3.5%
RA Capital Healthcare Fund, LP <sup>(6)</sup> 20 Park Plaza, Suite 1200 Boston, MA 02116	801,402	801,402	5.4%	3.1%
<b>Directors and Named Executive Officers</b>				
Thomas J. Farrell <sup>(7)</sup>	417,536	418,143	2.8%	1.6%
Kevin M. Slawin, M.D. <sup>(4)</sup>	1,685,239	1,687,644	10.9%	6.4%
Annemarie Moseley, Ph.D., M.D. <sup>(8)</sup>	122,547	122,547	*	*
Frank B. McGuyer <sup>(2)</sup>	2,170,664	2,214,767	14.7%	8.5%
James Brown <sup>(5)</sup>	882,022	905,322	6.1%	3.5%
Reid M. Huber, Ph.D.	0	0	*	*
Jon P. Stonehouse	0	0	*	*
All current executive officers and directors as a group (12 persons) <sup>(9)</sup>	5,628,016	5,698,431	37.8%	21.9%

- \* Represents beneficial ownership of less than one percent.
- (1) Consists of (i) 166,952 shares of common stock and 108,518 shares of common stock issuable upon the exercise of the warrants held by 667, L.P., (ii) 1,733,136 shares of common stock and 1,126,538 shares of common stock issuable upon the exercise of the warrants held by Baker Brothers Life Sciences, L.P., and (iii) 42,705 shares of common stock and 27,758 shares of common stock issuable upon the exercise of the warrants held by 14159, L.P. Baker Biotech Capital, L.P. is a general partner of each of the funds listed above.
  - (2) Consists of 1,950,939 shares of common stock and 219,725 shares of common stock issuable upon the exercise of warrants. Frank B. McGuyer, one of our directors, has voting and investment power held by McGuyer Investments Ltd. Mr. McGuyer disclaims beneficial ownership of such shares except to the extent of his pecuniary interest therein. The number of shares beneficially owned after offering reflects (a) the net exercise of warrants to purchase 23,528 shares of common stock for 22,896 shares of common stock and (b) the issuance of 44,735 shares of common stock as payment for the accrued dividend on shares of Series B convertible preferred stock.
  - (3) Consists of 1,604,597 shares of common stock and 159,444 shares of common stock issuable upon the exercise of warrants. The number of shares beneficially owned after offering reflects the issuance of 43,563 shares of common stock as payment for the accrued dividend on shares of Series B convertible preferred stock.
  - (4) Consists of (i) 149,639 shares of common stock and 14,868 shares of common stock issuable upon the exercise of warrants, (ii) 420,680 shares of common stock and 41,801 shares of common stock issuable upon the exercise of warrants beneficially owned by the Jordana Slawin 2012 Family Trust, for which Dr. Slawin's wife is a trustee, (iii) 366,636 shares of common stock and 36,431 shares of common stock issuable upon the exercise of warrants beneficially owned by the Kevin Slawin 2009 Family Trust, for which Dr. Slawin is a trustee and as such has the dispositive power and control over the securities held by such trust, (iv) 97,578 shares of



---

**Table of Contents**

common stock and 15,083 shares of common stock issuable upon the exercise of the warrants held by the 2009 Slawin Family Partnership, for which Dr. Slawin is the managing partner and as such has the dispositive power and control over the securities held by such trust, and (v) 542,533 shares of common stock subject to options exercisable as of November 29, 2014. The number of shares beneficially owned after offering reflects (a) the net exercise of warrants to purchase 4,901 shares of common stock for 4,769 shares of common stock and (b) the issuance of 2,537 shares of common stock as payment for the accrued dividend on shares of Series B convertible preferred stock.

- (5) Consists of 802,300 shares of common stock and 79,722 shares of common stock issuable upon the exercise of warrants by AVG Ventures, LP. and is managed by its general partner, AVG Ventures GP, LLC. Mr. Brown, one of our directors, is the manager of AVG Ventures GP, LLC and as such, shares voting and investment power with respect to shares held by AVG Ventures, LP. Mr. Brown disclaims beneficial ownership of such shares except to the extent of his pecuniary interest therein. The number of shares beneficially owned after offering reflects the issuance of 23,300 shares of common stock as payment for the accrued dividend on shares of Series B convertible preferred stock.
- (6) Consists of 485,698 shares of common stock and 315,704 shares of common stock issuable upon the exercise of warrants.
- (7) Consists of (i) 68,358 shares of common stock, (ii) 347,425 shares of common stock subject to options exercisable as of November 29, 2014 and (iii) 1,753 shares of common stock issuable upon the exercise of warrants. The number of shares beneficially owned after the offering reflects the issuance of 607 shares of common stock as payment for the accrued dividend on shares of Series B convertible preferred stock.
- (8) Consists of 26,960 shares of common stock subject to options exercisable as of November 29, 2014 by Ken Moseley and 95,587 shares of common stock subject to options exercisable as of November 29, 2014 by Annemarie Moseley. Mr. Moseley and Dr. Moseley are married.
- (9) Consists of shares identified in footnotes (2), (4), (5), (7) and (8) and includes the following: 179,813 shares of common stock owned by David Spencer, Ph.D., 15,784 shares of common stock issuable upon the exercise of warrants by Dr. Spencer, and 110,294 shares of common stock subject to options exercisable as of November 29, 2014 by Dr. Spencer, 26,960 shares of common stock subject to options exercisable as of November 29, 2014 by Ken Moseley; and 44,117 shares of common stock subject to options exercisable as of November 29, 2014 by Joseph Senesac. This does not include the 117,647 shares of common stock that have been granted to Alan Musso pursuant to a restricted stock grant that is contingent and effective upon the effectiveness of the registration statement of which this prospectus forms a part.

**Table of Contents**

**DESCRIPTION OF CAPITAL STOCK**

The following is a summary of the rights of our common and preferred stock and some of the provisions of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon the closing of this offering, and of the Delaware General Corporation Law. This summary is not complete. For more detailed information, please see our amended and restated certificate of incorporation and amended and restated bylaws, which are filed as exhibits to the registration statement of which this prospectus is a part, as well as the relevant provisions of the Delaware General Corporation Law.

**General**

Upon closing of this offering and the filing of our amended and restated certificate of incorporation, our authorized capital stock will consist of 200,000,000 shares of common stock, par value \$0.01 per share and 10,000,000 shares of preferred stock, par value \$0.01 per share. All of our authorized preferred stock upon the closing of this offering will be undesignated.

**Common Stock**

***Outstanding Shares***

On September 30, 2014, there were 2,124,386 shares of common stock outstanding, held of record by 21 stockholders. Based on such number of shares of common stock outstanding as of September 30, 2014, and assuming (1) the conversion of all outstanding shares of our preferred stock into 12,224,819 shares of common stock in connection with the closing of this offering, (2) the net exercise of outstanding warrants to purchase common stock for an aggregate of 114,468 shares of common stock, (3) that all of the holders of Series B convertible preferred stock will elect to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock in connection with this offering, which will result in the issuance by us of 177,349 shares of common stock, (4) the issuance by us of 6,559,598 shares of Series C convertible preferred stock issuable upon the exercise of warrants issued by us in August 2014, pursuant to that certain Series C Preferred Stock and Warrant Purchase Agreement, or the Series C Purchase Agreement and the conversion of these shares of Series C convertible preferred stock into an aggregate of 3,858,549 shares of common stock; and (5) the issuance by us of 7,350,000 shares of common stock in this offering, there will be 25,849,571 shares of common stock outstanding upon closing of this offering.

As of September 30, 2014, there were 1,602,339 shares of common stock subject to outstanding options under our equity incentive plans.

***Voting***

Our common stock is entitled to one vote for each share held of record on all matters submitted to a vote of the stockholders, including the election of directors, and does not have cumulative voting rights. Accordingly, the holders of a majority of the shares of our common stock entitled to vote in any election of directors can elect all of the directors standing for election.

***Dividends***

Subject to preferences that may be applicable to any then outstanding preferred stock, the holders of common stock are entitled to receive dividends, if any, as may be declared from time to time by our board of directors out of legally

available funds.

***Liquidation***

In the event of our liquidation, dissolution or winding up, holders of our common stock will be entitled to share ratably in the net assets legally available for distribution to stockholders after the payment of all of our debts and other liabilities, subject to the satisfaction of any liquidation preference granted to the holders of any outstanding shares of preferred stock.

## **Table of Contents**

### ***Rights and Preferences***

Holders of our common stock have no preemptive, conversion or subscription rights, and there are no redemption or sinking fund provisions applicable to our common stock. The rights, preferences and privileges of the holders of our common stock are subject to, and may be adversely affected by, the rights of the holders of shares of any series of our preferred stock that we may designate and issue in the future.

### ***Fully Paid and Nonassessable***

All of our outstanding shares of common stock are, and the shares of common stock to be issued in this offering will be, fully paid and nonassessable.

### **Convertible Preferred Stock**

As of September 30, 2014, we had outstanding an aggregate of 20,782,270 shares of convertible preferred stock held of record by 96 stockholders.

Upon closing of this offering, all outstanding shares of preferred stock will convert into 12,224,819 shares of our common stock.

Immediately prior to closing of this offering, our certificate of incorporation will be amended and restated to delete all references to such shares of convertible preferred stock. Under the amended and restated certificate of incorporation, our board of directors will have the authority, without further action by the stockholders, to issue up to 10,000,000 shares of preferred stock in one or more series, to establish from time to time the number of shares to be included in each such series, to fix the rights, preferences and privileges of the shares of each wholly unissued series and any qualifications, limitations or restrictions thereon and to increase or decrease the number of shares of any such series, but not below the number of shares of such series then outstanding.

Our board of directors may authorize the issuance of preferred stock with voting or conversion rights that could adversely affect the voting power or other rights of the holders of the common stock. The issuance of preferred stock, while providing flexibility in connection with possible acquisitions and other corporate purposes, could, among other things, have the effect of delaying, deferring or preventing a change in our control that may otherwise benefit holders of our common stock and may adversely affect the market price of the common stock and the voting and other rights of the holders of common stock. We have no current plans to issue any shares of preferred stock.

### **Stock Options**

As of September 30, 2014, 1,602,339 shares of common stock were issuable upon the exercise of outstanding stock options, at a weighted-average exercise price of \$2.33 per share.

### **Warrants**

As of September 30, 2014, 473,031 shares of common stock were issuable upon the exercise of outstanding warrants to purchase common stock with a weighted-average exercise price of \$0.128 per share. The warrants provide for the adjustment of the number of shares issuable upon the exercise of the warrants in the event of stock splits, recapitalizations, reclassifications and consolidations. Upon closing of this offering, warrants to purchase 117,639 shares of common stock will be automatically cancelled if not previously exercised.

As of September 30, 2014, 6,599,598 shares of Series C preferred stock were issuable upon the exercise of outstanding warrants to purchase Series C preferred stock with a weighted-average exercise price of \$6.00 per share. The warrants provide for the adjustment of the number of shares issuable upon the exercise of the warrants in the event of stock splits, recapitalizations, reclassifications and consolidations. Upon the date immediately following the date that the registration statement, of which this prospectus forms a part, is deemed effective by the SEC, on or prior to March 31, 2015, the warrants will be terminated if not previously exercised.

### **Registration Rights**

Following the closing of this offering, certain holders of our common stock, or their transferees, will be entitled to the registration rights set forth below with respect to registration of the resale of such shares under the Securities Act pursuant to the investors' rights agreement by and among us and certain of our stockholders.

## **Table of Contents**

### ***Demand Registration Rights***

At any time beginning six months after the public offering date set forth on the cover page of this prospectus, upon the written request of a holder of our preferred stock or at least 30% of the holders of the registrable securities then outstanding that we file a registration statement under the Securities Act covering the registration of the registrable securities having an aggregate offering price to the public of not less than \$10.0 million, we will be obligated to notify all holders of registrable securities of such request and to use our reasonable best efforts to register the sale of all registrable securities that holders may request to be registered. Holders of our preferred stock may not request more than two registration statements which are declared or ordered effective, except that one preferred stockholder may demand one registration if it does not participate in either of the two demand registrations. We may postpone the filing or effectiveness of a registration statement if (1) we are engaged in or plan to engage in a firm commitment underwritten public offering during the period starting with the date 60 days prior to our good faith estimate of, and ending on a date 180 days following the effective date of, the date of filing a registration statement, or (2) for up to 90 days once in any 12-month period our board of directors reasonably determines that such registration and offering would be materially detrimental to us and our stockholders, and we are not required to effect the filing of a registration statement during the period starting with the date of the filing of, and ending on a date 180 days following the effective date of the registration statement for this offering. The underwriters of any underwritten offering will have the right to limit the number of shares having registration rights to be included in the registration statement.

### ***Piggyback Registration Rights***

If we register any securities for public sale, holders of registration rights will have the right to include their shares in the registration statement. The underwriters of any underwritten offering will have the right to limit the number of shares having registration rights to be included in the registration statement, but not below 25% of the total number of shares included in the registration statement, except this offering, in which the holders may be entirely excluded.

In addition, pursuant to a Common Stock Purchase Warrant with the State of Texas, the State of Texas, acting by and through the Office of Governor Economic Development and Tourism, is entitled to standard piggyback registration rights granted by us to any shareholder on terms no less favorable than granted to any such shareholder with respect to the securities covered by that warrant.

### ***Form S-3 Registration Rights***

If we are eligible to file a registration statement on Form S-3, holders of registrable securities will have the right to demand that we file a registration statement on Form S-3 so long as the aggregate price to the public of the securities to be sold under the registration statement on Form S-3 is at least \$3.0 million, subject to specified exceptions, conditions and limitations. We are not required to effect more than two registrations on Form S-3 in any 12-month period.

### ***Expenses of Registration***

Generally, we are required to bear all registration and selling expenses incurred in connection with the demand, piggyback and Form S-3 registrations described above, other than underwriting discounts and commissions, in an amount not to exceed \$50,000 per registration.

### ***Expiration of Registration Rights***

The demand, piggyback and Form S-3 registration rights discussed above will terminate as to a given holder of registrable securities upon the earlier of (1) three years following the closing of this offering, (2) as to any holder of registrable securities, the first date after our initial public offering on which such holder is able to dispose of all of its registrable securities without restriction under Rule 144 of the Securities Act, or (3) after the consummation of a liquidation event.

**Table of Contents**

**Anti-Takeover Effects of Provisions of Our Amended and Restated Certificate of Incorporation, Our Bylaws and Delaware Law**

***Delaware Anti-Takeover Law***

We are subject to Section 203 of the Delaware General Corporation Law, or Section 203. Section 203 generally prohibits a public Delaware corporation from engaging in a business combination with an interested stockholder for a period of three years after the date of the transaction in which the person became an interested stockholder, unless:

- n prior to the date of the transaction, the board of directors of the corporation approved either the business combination or the transaction which resulted in the stockholder becoming an interested stockholder;
- n the interested stockholder owned at least 85% of the voting stock of the corporation outstanding upon consummation of the transaction, excluding for purposes of determining the number of shares outstanding (1) shares owned by persons who are directors and also officers and (2) shares owned by employee stock plans in which employee participants do not have the right to determine confidentially whether shares held subject to the plan will be tendered in a tender or exchange offer; or
- n on or subsequent to the consummation of the transaction, the business combination is approved by the board and authorized at an annual or special meeting of stockholders, and not by written consent, by the affirmative vote of at least 66 2/3% of the outstanding voting stock which is not owned by the interested stockholder.

Section 203 defines a business combination to include:

- n any merger or consolidation involving the corporation and the interested stockholder;
- n any sale, transfer, pledge or other disposition involving the interested stockholder of 10% or more of the assets of the corporation;
- n subject to exceptions, any transaction involving the corporation that has the effect of increasing the proportionate share of the stock of any class or series of the corporation beneficially owned by the interested stockholder;
- n subject to exceptions, any transaction that results in the issuance or transfer by the corporation of any stock of the corporation to the interested stockholder; and
- n the receipt by the interested stockholder of the benefit of any loans, advances, guarantees, pledges or other financial benefits provided by or through the corporation.



In general, Section 203 defines an interested stockholder as any entity or person beneficially owning 15% or more of the outstanding voting stock of the corporation and any entity or person affiliated with or controlling or controlled by the entity or person.

***Amended and Restated Certificate of Incorporation and Amended and Restated Bylaws***

Provisions of our amended and restated certificate of incorporation and amended and restated bylaws, which will become effective upon the closing of this offering, may delay or discourage transactions involving an actual or potential change in our control or change in our management, including transactions in which stockholders might otherwise receive a premium for their shares or transactions that our stockholders might otherwise deem to be in their best interests. Therefore, these provisions could adversely affect the price of our common stock. Among other things, our amended and restated certificate of incorporation and amended and restated bylaws:

- n permit our board of directors to issue up to 10,000,000 shares of preferred stock, with any rights, preferences and privileges as they may designate;
- n provide that the authorized number of directors may be changed only by resolution adopted by a majority of the board of directors;
- n provide that the board of directors or any individual director may only be removed with cause and the affirmative vote of the holders of at least 66 2/3% of the voting power of all of our then outstanding common stock;
- n provide that all vacancies, including newly created directorships, may, except as otherwise required by law or subject to the rights of holders of preferred stock as designated from time to time, be filled by the

## **Table of Contents**

affirmative vote of a majority of directors then in office, even if less than a quorum;

- n divide our board of directors into three classes;
- n require that any action to be taken by our stockholders must be effected at a duly called annual or special meeting of stockholders and not be taken by written consent or electronic transmission;
- n provide that stockholders seeking to present proposals before a meeting of stockholders or to nominate candidates for election as directors at a meeting of stockholders must provide notice in writing in a timely manner and also specify requirements as to the form and content of a stockholder's notice;
- n do not provide for cumulative voting rights (therefore allowing the holders of a majority of the shares of common stock entitled to vote in any election of directors to elect all of the directors standing for election, if they should so choose);
- n provide that special meetings of our stockholders may be called only by the chairman of the board, our Chief Executive Officer or by the board of directors pursuant to a resolution adopted by a majority of the total number of authorized directors (whether or not there exists any vacancies); and
- n provide that the Court of Chancery of the State of Delaware will be the sole and exclusive forum for (1) any derivative action or proceeding brought on our behalf, (2) any action asserting a claim of breach of a fiduciary duty owed by any of our directors or officers to us or our stockholders, (3) any action asserting a claim against us arising pursuant to any provision of the DGCL or our certificate of incorporation or bylaws, or (4) any action asserting a claim against us governed by the internal affairs doctrine.

The amendment of any of these provisions, with the exception of the ability of our board of directors to issue shares of preferred stock and designate any rights, preferences and privileges thereto, would require the affirmative vote of the holders of at least 66 2/3% of the voting power of all of our then outstanding common stock.

## **NASDAQ Global Market Listing**

We have applied to list our common stock on The NASDAQ Global Market under the symbol BLCM.

## **Transfer Agent and Registrar**

The transfer agent and registrar for our common stock is American Stock Transfer & Trust Company, LLC. The transfer agent and registrar's address is 6201 1<sup>st</sup> Avenue, Brooklyn, NY 11219.

---

**Table of Contents**

**SHARES ELIGIBLE FOR FUTURE SALE**

Immediately prior to this offering, there has been no public market for our common stock. Future sales of substantial amounts of common stock in the public market could adversely affect prevailing market prices. Furthermore, since only a limited number of shares will be available for sale shortly after this offering because of contractual and legal restrictions on resale described below, sales of substantial amounts of common stock in the public market after the restrictions lapse could adversely affect the prevailing market price for our common stock as well as our ability to raise equity capital in the future.

Based on the number of shares of common stock outstanding as of September 30, 2014, upon the closing of this offering, 25,849,571 shares of common stock will be outstanding, assuming (1) the conversion of all outstanding shares of our preferred stock into 12,224,819 shares of common stock in connection with the closing of this offering, (2) the net exercise of outstanding warrants to purchase common stock for an aggregate of 114,468 shares of common stock, (3) that all of the holders of Series B convertible preferred stock will elect to have their accrued dividends converted into common stock at the time of conversion of their shares of Series B convertible preferred stock into shares of common stock in connection with this offering, which will result in the issuance by us of 177,349 shares of common stock, (4) the issuance by us of 6,559,598 shares of Series C convertible preferred stock issuable upon the exercise of warrants issued by us in August 2014, pursuant to that certain Series C Preferred Stock and Warrant Purchase Agreement, or the Series C Purchase Agreement and the conversion of these shares of Series C convertible preferred stock into an aggregate of 3,858,549 shares of common stock; and (5) the issuance by us of 7,350,000 shares of common stock in this offering. All of the shares sold in this offering will be freely tradable unless purchased by our affiliates as that term is defined in Rule 144 under the Securities Act or purchased by existing stockholders and their affiliated entities who are subject to lock-up agreements. The remaining shares of common stock outstanding after this offering will be restricted as a result of securities laws or lock-up agreements. These remaining 18,499,571 shares will generally become available for sale in the public market as follows:

- n No restricted shares will be eligible for immediate sale upon the closing of this offering; and
- n The restricted shares will be eligible for sale from time to time upon expiration of lock-up agreements at least 180 days after the date of this offering and under Rule 144 or Rule 701, subject to the volume limitations, manner of sale and notice provisions described under Rule 144 below, but could be sold earlier if the holders exercise any available registration rights.

**Rule 144**

In general, under Rule 144 as currently in effect, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, any person who is not an affiliate of ours and has held their shares for at least six months, including the holding period of any prior owner other than one of our affiliates, may sell shares without restriction, provided current public information about us is available. In addition, under Rule 144, any person who is not an affiliate of ours and has held their shares for at least one year, including the holding period of any prior owner other than one of our affiliates, would be entitled to sell an unlimited number of shares immediately upon the closing of this offering without regard to whether current public information about us is available.

Beginning 90 days after the effective date of the registration statement of which this prospectus is a part, a person who is an affiliate of ours and who has beneficially owned restricted securities for at least six months, including the holding period of any prior owner other than one of our affiliates, is entitled to sell a number of restricted shares

within any three-month period that does not exceed the greater of:

- n 1% of the number of shares of our common stock then outstanding, which will equal approximately 258,495 shares immediately after this offering; or
  
- n the average weekly trading volume of our common stock on The NASDAQ Global Market during the four calendar weeks preceding the filing of a notice on Form 144 with respect to the sale.

## **Table of Contents**

Sales of restricted shares under Rule 144 held by our affiliates are also subject to requirements regarding the manner of sale, notice and the availability of current public information about us. Rule 144 also provides that affiliates relying on Rule 144 to sell shares of our common stock that are not restricted shares must nonetheless comply with the same restrictions applicable to restricted shares, other than the holding period requirement.

Notwithstanding the availability of Rule 144, the holders of substantially all of our restricted shares have entered into lock-up agreements as described below and their restricted shares will become eligible for sale at the expiration of the restrictions set forth in those agreements.

### **Rule 701**

Under Rule 701, shares of our common stock acquired upon the exercise of currently outstanding options or pursuant to other rights granted under our stock plans may be resold by:

- n persons other than affiliates, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, subject only to the manner-of-sale provisions of Rule 144; and
- n our affiliates, beginning 90 days after the effective date of the registration statement of which this prospectus is a part, subject to the manner-of-sale and volume limitations, current public information and filing requirements of Rule 144, in each case, without compliance with the six-month holding period requirement of Rule 144.

As of September 30, 2014, options to purchase a total of 1,602,339 shares of common stock were outstanding, of which 1,113,884 were vested. Of the total number of shares of our common stock issuable under these options, substantially all are subject to contractual lock-up agreements with us or the underwriters described below under Underwriting and will become eligible for sale in accordance with Rule 701 at the expiration of those agreements.

### **Lock-Up Agreements**

We, along with our directors, executive officers and substantially all of our other stockholders, optionholders, convertible noteholders and warrant holders, have agreed with the underwriters that for a period of 180 days (the restricted period), after the date of this prospectus, subject to specified exceptions, we or they will not offer, pledge, sell, contract to sell, sell any option or contract to purchase, purchase any option or contract to sell, grant any option, right or warrant to purchase, lend or otherwise transfer or dispose of, directly or indirectly, any shares of common stock or any securities convertible into or exercisable or exchangeable for shares of common stock, or enter into any swap or other arrangement that transfers to another, in whole or in part, any of the economic consequences of ownership of the common stock. Upon expiration of the restricted period, certain of our stockholders and warrant holders will have the right to require us to register their shares under the Securities Act. See Registration Rights below and Description of Capital Stock Registration Rights.

After this offering, certain of our employees, including our executive officers and/or directors, may enter into written trading plans that are intended to comply with Rule 10b5-1 under the Exchange Act. Sales under these trading plans would not be permitted until the expiration of the lock-up agreements relating to the offering described above.

### **Registration Rights**

Upon closing of this offering, the holders of approximately 16.9 million shares of our common stock will be entitled to rights with respect to the registration of their shares under the Securities Act, subject to the lock-up agreements described under **Lock-Up Agreements** above. Registration of these shares under the Securities Act would result in the shares becoming freely tradable without restriction under the Securities Act, except for shares purchased by affiliates, immediately upon the effectiveness of the registration statement of which this prospectus is a part. Any sales of securities by these stockholders could have a material adverse effect on the trading price of our common stock. See **Description of Capital Stock** **Registration Rights**.

### **Equity Incentive Plans**

We intend to file with the SEC a registration statement on Form S-8 under the Securities Act covering the shares of common stock reserved for issuance under the EIP and the ESPP. The registration statement is expected to be filed and become effective as soon as practicable after the closing of this offering. Accordingly, shares registered under the registration statement will be available for sale in the open market following its effective date, subject to Rule 144 volume limitations and the lock-up agreements described above, if applicable.

---

**Table of Contents**

**MATERIAL U.S. FEDERAL INCOME TAX CONSEQUENCES  
TO NON-U.S. HOLDERS OF OUR COMMON STOCK**

The following discussion describes the material U.S. federal income tax consequences of the acquisition, ownership and disposition of our common stock acquired in this offering by Non-U.S. Holders (as defined below). This discussion does not address all aspects of U.S. federal income taxes that may be relevant to Non-U.S. Holders in light of their particular circumstances, nor does it address any U.S. federal estate or gift tax, any state, local or non-U.S. tax consequences or U.S. federal tax consequences other than income taxes. Rules different from those described below may apply to certain Non-U.S. Holders that are subject to special treatment under the Code such as financial institutions, insurance companies, tax-exempt organizations, tax-qualified retirement plans, broker-dealers and traders in securities, commodities or currencies, U.S. expatriates, controlled foreign corporations, passive foreign investment companies, corporations that accumulate earnings to avoid U.S. federal income tax, persons that hold our common stock as part of a straddle, conversion transaction, or other risk reduction strategy, holders deemed to sell our common stock under the constructive sale provisions of the Code, holders who hold or receive our common stock pursuant to the exercise of employee stock options or otherwise as compensation, holders who are subject to the alternative minimum tax or Medicare contribution tax, partnerships and other pass-through entities, and investors in such pass-through entities or entities that are treated as disregarded entities for U.S. federal income tax purposes (regardless of their places of organization or formation). Such Non-U.S. Holders are urged to consult their own tax advisors to determine the U.S. federal, state, local and other tax consequences that may be relevant to them. Furthermore, the discussion below is based upon the provisions of the Code, and Treasury regulations, published administrative pronouncements, rulings and judicial decisions thereunder as of the date hereof. Such authorities may be repealed, revoked or modified, perhaps retroactively, so as to result in U.S. federal income tax consequences different from those discussed below. We have not requested a ruling from the U.S. Internal Revenue Service, or the IRS, with respect to the statements made and the conclusions reached in the following summary. This discussion assumes that the Non-U.S. Holder holds our common stock as a capital asset within the meaning of Section 1221 of the Code (generally, property held for investment).

The following discussion is for general information only and is not tax advice for any Non-U.S. Holders under their particular circumstances. Persons considering the purchase of our common stock pursuant to this offering should consult their own tax advisors concerning the U.S. federal income tax consequences of acquiring, owning and disposing of our common stock in light of their particular situations as well as any consequences arising under the laws of any other taxing jurisdiction, including any state, local and non-U.S. tax consequences and any U.S. federal non-income tax consequences.

For the purposes of this discussion, a Non-U.S. Holder is, for U.S. federal income tax purposes, a beneficial owner of common stock that is not a U.S. Holder. A U.S. Holder means a beneficial owner of our common stock that is for U.S. federal income tax purposes (a) an individual who is a citizen or resident of the United States, (b) a corporation or other entity treated as a corporation created or organized in or under the laws of the United States, any state thereof or the District of Columbia, (c) an estate the income of which is subject to U.S. federal income taxation regardless of its source or (d) a trust if it (1) is subject to the primary supervision of a court within the United States and one or more U.S. persons have the authority to control all substantial decisions of the trust or (2) has a valid election in effect under applicable U.S. Treasury regulations to be treated as a U.S. person. Also, partnerships, or other entities that are treated as partnerships for U.S. federal income tax purposes (regardless of their place of organization or formation) and entities that are treated as disregarded entities for U.S. federal income tax purposes (regardless of their place of organization or formation) are not addressed by this discussion and are, therefore, not considered to be Non-U.S. Holders for the purposes of this discussion.

### **Distributions on Our Common Stock**

Subject to the discussion below regarding backup withholding and foreign accounts, distributions, if any, made on our common stock to a Non-U.S. Holder of our common stock generally will constitute dividends for U.S. tax purposes to the extent made out of our current or accumulated earnings and profits (as determined under U.S. federal income tax principles) and will be subject to withholding tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. To obtain a reduced rate of withholding under a treaty, a Non-U.S.



---

## **Table of Contents**

Holder generally will be required to provide us with a properly executed IRS Form W-8BEN or W-8BEN-E, or other appropriate form, certifying the Non-U.S. Holder's entitlement to benefits under that treaty. In the case of a Non-U.S. Holder that is an entity, Treasury regulations and the relevant tax treaty provide rules to determine whether, for purposes of determining the applicability of a tax treaty, dividends will be treated as paid to the entity or to those holding an interest in that entity. If a Non-U.S. Holder holds stock through a financial institution or other agent acting on the holder's behalf, the holder will be required to provide appropriate documentation to such agent. The holder's agent will then be required to provide certification to the applicable agent, either directly or through other intermediaries. If you are eligible for a reduced rate of U.S. federal withholding tax under an income tax treaty, you should consult with your own tax advisor to determine if you are able to obtain a refund or credit of any excess amounts withheld by timely filing an appropriate claim for a refund with the IRS.

We generally are not required to withhold tax on dividends paid to a Non-U.S. Holder that are effectively connected with the Non-U.S. Holder's conduct of a trade or business within the United States (and, if required by an applicable income tax treaty, are attributable to a permanent establishment that such holder maintains in the United States) if a properly executed IRS Form W-8ECI, stating that the dividends are so connected, is furnished to us (or, if stock is held through a financial institution or other agent, to such agent). In general, such effectively connected dividends will be subject to U.S. federal income tax, on a net income basis at the regular graduated rates, unless a specific treaty exemption applies. A corporate Non-U.S. Holder receiving effectively connected dividends may also be subject to an additional branch profits tax, which is imposed, under certain circumstances, at a rate of 30% (or such lower rate as may be specified by an applicable treaty) on the corporate Non-U.S. Holder's effectively connected earnings and profits, subject to certain adjustments.

To the extent distributions on our common stock, if any, exceed our current and accumulated earnings and profits, they will first reduce your adjusted basis in our common stock as a non-taxable return of capital, but not below zero, and then any excess will be treated as gain and taxed in the same manner as gain realized from a sale or other disposition of common stock as described in the next section.

### **Gain on Disposition of Our Common Stock**

Subject to the discussion below regarding backup withholding and foreign accounts, a Non-U.S. Holder generally will not be subject to U.S. federal income tax with respect to gain realized on a sale or other disposition of our common stock unless (1) the gain is effectively connected with a trade or business of such holder in the United States (and, if required by an applicable income tax treaty, is attributable to a permanent establishment that such holder maintains in the United States), (2) the Non-U.S. Holder is a nonresident alien individual and is present in the United States for 183 or more days in the taxable year of the disposition and certain other conditions are met, or (3) we are or have been a United States real property holding corporation, or a USRPHC, within the meaning of Code Section 897(c)(2) at any time within the shorter of the five-year period preceding such disposition or such holder's holding period.

If you are a Non-U.S. Holder described in (1) above, you will be required to pay tax on the net gain derived from the sale at regular graduated U.S. federal income tax rates, unless a specific treaty exemption applies, and corporate Non-U.S. Holders described in (1) above may be subject to the additional branch profits tax at a 30% rate or such lower rate as may be specified by an applicable income tax treaty. If you are an individual Non-U.S. Holder described in (2) above, you will be required to pay a flat 30% tax on the gain derived from the sale, which gain may be offset by U.S. source capital losses (even though you are not considered a resident of the United States). With respect to (3) above, in general, we would be a USRPHC if interests in U.S. real estate constituted (by fair market value) at least half of our assets. We believe that we are not, and do not anticipate becoming a USRPHC, however, there can be no assurance that we will not become a USRPHC in the future. Even if we are treated as a USRPHC, gain realized by a Non-U.S. Holder on a disposition of our common stock will not be subject to U.S. federal income tax so long as (i) the

Non-U.S. Holder owned, directly, indirectly and constructively, no more than 5% of our common stock at all times within the shorter of (a) the five-year period preceding the disposition or (b) the holder's holding period and (ii) our common stock is regularly traded on an established securities market. There can be no assurance that our common stock will continue to qualify as regularly traded on an established securities market.

## **Table of Contents**

### **Information Reporting Requirements and Backup Withholding**

Generally, we or certain financial middlemen must report information to the IRS with respect to any dividends we pay on our common stock including the amount of any such dividends, the name and address of the recipient, and the amount, if any, of tax withheld. A similar report is sent to the holder to whom any such dividends are paid. Pursuant to tax treaties or certain other agreements, the IRS may make its reports available to tax authorities in the recipient's country of residence.

Dividends paid by us (or our paying agents) to a Non-U.S. Holder may also be subject to U.S. backup withholding. U.S. backup withholding generally will not apply to a Non-U.S. Holder who provides a properly executed IRS Form W-8BEN or W-8BEN-E, or otherwise establishes an exemption.

Under current U.S. federal income tax law, U.S. information reporting and backup withholding requirements generally will apply to the proceeds of a disposition of our common stock effected by or through a U.S. office of any broker, U.S. or non-U.S., unless the holder provides a properly executed IRS Form W-8BEN or W-8BEN-E, or otherwise establishes an exemption. Generally, U.S. information reporting and backup withholding requirements will not apply to a payment of disposition proceeds to a Non-U.S. Holder where the transaction is effected outside the United States through a non-U.S. office of a non-U.S. broker. For information reporting purposes, certain brokers with substantial U.S. ownership or operations will generally be treated in a manner similar to U.S. brokers.

If backup withholding is applied to you, you should consult with your own tax advisor to determine if you are able to obtain a tax refund or credit with respect to the amount withheld.

### **Foreign Accounts**

A U.S. federal withholding tax of 30% may apply to dividends and the gross proceeds of a disposition of our common stock paid to a foreign financial institution (as specifically defined by applicable rules), including when the foreign financial institution holds our common stock on behalf of a Non-U.S. Holder, unless such institution enters into an agreement with the U.S. government to withhold on certain payments and to collect and provide to the U.S. tax authorities substantial information regarding U.S. account holders of such institution (which may include certain equity holders of such institution, as well as certain account holders that are foreign entities with U.S. owners). Foreign financial institutions located in jurisdictions that have an intergovernmental agreement with the United States governing these withholding and reporting requirements may be subject to different rules. This U.S. federal withholding tax of 30% will also apply to dividends and the gross proceeds of a disposition of our common stock paid to a non-financial foreign entity unless such entity provides the withholding agent with either a certification that it does not have any substantial direct or indirect U.S. owners or provides information regarding direct and indirect U.S. owners of the entity. The withholding tax described above will not apply if the foreign financial institution or non-financial foreign entity otherwise qualifies for an exemption from the rules. Under certain circumstances, a Non-U.S. Holder might be eligible for refunds or credits of such taxes. Holders are encouraged to consult with their own tax advisors regarding the possible implications of the legislation on their investment in our common stock.

The withholding provisions described above will generally apply to payments of dividends made on or after July 1, 2014 and to payments of gross proceeds from a sale or other disposition of common stock on or after January 1, 2017.

**EACH PROSPECTIVE INVESTOR SHOULD CONSULT ITS OWN TAX ADVISOR REGARDING THE TAX CONSEQUENCES OF PURCHASING, HOLDING AND DISPOSING OF OUR COMMON STOCK, INCLUDING THE CONSEQUENCES OF ANY PROPOSED CHANGE IN APPLICABLE LAW, AS WELL AS TAX CONSEQUENCES ARISING UNDER ANY STATE, LOCAL, NON-U.S. OR U.S. FEDERAL NON-INCOME**

TAX LAWS.

**Table of Contents****UNDERWRITING**

Subject to the terms and conditions set forth in the underwriting agreement, dated December 17, 2014, between us and Jefferies LLC and Citigroup Global Markets Inc., as the representatives of the underwriters named below and the book-running managers of this offering, we have agreed to sell to the underwriters, and each of the underwriters has agreed, severally and not jointly, to purchase from us, the respective number of shares of common stock shown opposite its name below:

<b>UNDERWRITER</b>	<b>NUMBER OF SHARES</b>
Jefferies LLC	3,197,250
Citigroup Global Markets Inc.	2,976,750
Piper Jaffray & Co.	1,175,900
Trout Capital LLC	100
<b>Total</b>	<b>7,350,000</b>

The underwriting agreement provides that the obligations of the several underwriters are subject to certain conditions precedent such as the receipt by the underwriters of officers' certificates and legal opinions and approval of certain legal matters by their counsel. The underwriting agreement provides that the underwriters will purchase all of the shares of common stock if any of them are purchased. If an underwriter defaults, the underwriting agreement provides that the purchase commitments of the nondefaulting underwriters may be increased or the underwriting agreement may be terminated. We have agreed to indemnify the underwriters and certain of their controlling persons against certain liabilities, including liabilities under the Securities Act, and to contribute to payments that the underwriters may be required to make in respect of those liabilities.

The underwriters have advised us that, following the completion of this offering, they currently intend to make a market in the common stock as permitted by applicable laws and regulations. However, the underwriters are not obligated to do so, and the underwriters may discontinue any market-making activities at any time without notice in their sole discretion. Accordingly, no assurance can be given as to the liquidity of the trading market for the common stock, that you will be able to sell any of the common stock held by you at a particular time or that the prices that you receive when you sell will be favorable.

The underwriters are offering the shares of common stock subject to their acceptance of the shares of common stock from us and subject to prior sale. The underwriters reserve the right to withdraw, cancel or modify offers to the public

and to reject orders in whole or in part.

### **Commission and Expenses**

The underwriters have advised us that they propose to offer the shares of common stock to the public at the initial public offering price set forth on the cover page of this prospectus and to certain dealers, which may include the underwriters, at that price less a concession not in excess of \$0.798 per share of common stock. After the offering, the initial public offering price and concession may be reduced by the representatives. No such reduction will change the amount of proceeds to be received by us as set forth on the cover page of this prospectus.

**Table of Contents**

The following table shows the public offering price, the underwriting discounts and commissions that we are to pay the underwriters and the proceeds, before expenses, to us in connection with this offering. Such amounts are shown assuming both no exercise and full exercise of the underwriters' option to purchase additional shares.

	PER SHARE		TOTAL	
	WITHOUT OPTION TO PURCHASE ADDITIONAL SHARES	WITH OPTION TO PURCHASE ADDITIONAL SHARES	WITHOUT OPTION TO PURCHASE ADDITIONAL SHARES	WITH OPTION TO PURCHASE ADDITIONAL SHARES
Public offering price	\$ 19.00	\$ 19.00	\$ 139,650,000	\$ 160,597,500
Underwriting discounts and commissions paid by us	\$ 1.33	\$ 1.33	\$ 9,775,500	\$ 11,241,825
Proceeds to us, before expenses	\$ 17.67	\$ 17.67	\$ 129,874,500	\$ 149,355,675

We estimate expenses payable by us in connection with this offering, other than the underwriting discounts and commissions referred to above, will be approximately \$2.8 million. We have agreed to reimburse the underwriters for up to \$35,000 for their FINRA counsel fee. In accordance with FINRA Rule 5110, this reimbursed FINRA counsel fee is deemed underwriting compensation for this offering. Trout Group LLC ( Trout ) is an affiliate of Trout Capital LLC, a FINRA member and an underwriter for this offering. Pursuant to an advisory services agreement between us and Trout, Trout will receive a maximum aggregate amount of \$193,000 in fees and expenses that are deemed underwriting compensation for this offering in accordance with FINRA Rule 5110. Jefferies LLC has been granted a right to participate in future financings by the Company. This right is deemed to constitute 1% in underwriting compensation for this offering.

**Determination of Offering Price**

Prior to this offering, there has not been a public market for our common stock. Consequently, the initial public offering price for our common stock will be determined by negotiations between us and the representatives. Among the factors to be considered in these negotiations will be prevailing market conditions, our financial information, market valuations of other companies that we and the underwriters believe to be comparable to us, estimates of our business potential, the present state of our development and other factors deemed relevant.

We offer no assurances that the initial public offering price will correspond to the price at which the common stock will trade in the public market subsequent to the offering or that an active trading market for the common stock will develop and continue after the offering.

**Listing**

We have been approved to list our common stock listed on The NASDAQ Global Market under the trading symbol BLCM.

### **Stamp Taxes**

If you purchase shares of common stock offered in this prospectus, you may be required to pay stamp taxes and other charges under the laws and practices of the country of purchase, in addition to the offering price listed on the cover page of this prospectus.

### **Option to Purchase Additional Shares**

We have granted to the underwriters an option, exercisable for 30 days from the date of this prospectus, to purchase, from time to time, in whole or in part, up to an aggregate of 1,102,500 shares from us at the public offering price set forth on the cover page of this prospectus, less underwriting discounts and commissions. If the underwriters exercise this option, each underwriter will be obligated, subject to specified conditions, to purchase a number of additional shares proportionate to that underwriter's initial purchase commitment as indicated in the table above. This option may be exercised only if the underwriters sell more shares than the total number set forth on the cover page of this prospectus.



## **Table of Contents**

### **No Sales of Similar Securities**

We, our officers, directors and holders of all or substantially all our outstanding capital stock and other securities have agreed, subject to specified exceptions, not to directly or indirectly:

- n sell, offer, contract or grant any option to sell (including any short sale), pledge, transfer, establish an open put equivalent position within the meaning of Rule 16a-1(h) under the Securities Exchange Act of 1934, as amended, or
- n otherwise dispose of any shares of common stock, options or warrants to acquire shares of common stock, or securities exchangeable or exercisable for or convertible into shares of common stock currently or hereafter owned either of record or beneficially, or
- n publicly announce an intention to do any of the foregoing for a period of 180 days after the date of this prospectus without the prior written consent of Jefferies LLC and Citigroup Global Markets Inc.

This restriction terminates after the close of trading of the common stock on and including the 180<sup>th</sup> day after the date of this prospectus.

Jefferies LLC and Citigroup Global Markets Inc. may, in their sole discretion and at any time or from time to time before the termination of the 180-day period release all or any portion of the securities subject to lock-up agreements. There are no existing agreements between the underwriters and any of our shareholders who will execute a lock-up agreement, providing consent to the sale of shares prior to the expiration of the lock-up period.

### **Stabilization**

The underwriters have advised us that they, pursuant to Regulation M under the Securities Exchange Act of 1934, as amended, certain persons participating in the offering may engage in short sale transactions, stabilizing transactions, syndicate covering transactions or the imposition of penalty bids in connection with this offering. These activities may have the effect of stabilizing or maintaining the market price of the common stock at a level above that which might otherwise prevail in the open market. Establishing short sales positions may involve either covered short sales or naked short sales.

Covered short sales are sales made in an amount not greater than the underwriters' option to purchase additional shares of our common stock in this offering. The underwriters may close out any covered short position by either exercising their option to purchase additional shares of our common stock or purchasing shares of our common stock in the open market. In determining the source of shares to close out the covered short position, the underwriters will consider, among other things, the price of shares available for purchase in the open market as compared to the price at which they may purchase shares through the option to purchase additional shares.

Naked short sales are sales in excess of the option to purchase additional shares of our common stock. The underwriters must close out any naked short position by purchasing shares in the open market. A naked short position is more likely to be created if the underwriters are concerned that there may be downward pressure on the price of the shares of our common stock in the open market after pricing that could adversely affect investors who purchase in this offering.

A stabilizing bid is a bid for the purchase of shares of common stock on behalf of the underwriters for the purpose of fixing or maintaining the price of the common stock. A syndicate covering transaction is the bid for or the purchase of shares of common stock on behalf of the underwriters to reduce a short position incurred by the underwriters in connection with the offering. Similar to other purchase transactions, the underwriter's purchases to cover the syndicate short sales may have the effect of raising or maintaining the market price of our common stock or preventing or retarding a decline in the market price of our common stock. As a result, the price of our common stock may be higher than the price that might otherwise exist in the open market. A penalty bid is an arrangement permitting the underwriters to reclaim the selling concession otherwise accruing to a syndicate member in connection with the offering if the common stock originally sold by such syndicate member are purchased in a syndicate covering transaction and therefore have not been effectively placed by such syndicate member.

## **Table of Contents**

Neither we, nor any of the underwriters make any representation or prediction as to the direction or magnitude of any effect that the transactions described above may have on the price of our common stock. The underwriters are not obligated to engage in these activities and, if commenced, any of the activities may be discontinued at any time.

The underwriters may also engage in passive market making transactions in our common stock on The NASDAQ Global Select Market in accordance with Rule 103 of Regulation M during a period before the commencement of offers or sales of shares of our common stock in this offering and extending through the completion of distribution. A passive market maker must display its bid at a price not in excess of the highest independent bid of that security. However, if all independent bids are lowered below the passive market maker's bid, that bid must then be lowered when specified purchase limits are exceeded.

## **Electronic Distribution**

A prospectus in electronic format may be made available by e-mail or on the web sites or through online services maintained by one or more of the underwriters or their affiliates. In those cases, prospective investors may view offering terms online and may be allowed to place orders online. The underwriters may agree with us to allocate a specific number of shares of common stock for sale to online brokerage account holders. Any such allocation for online distributions will be made by the underwriters on the same basis as other allocations. Other than the prospectus in electronic format, the information on the underwriters' web sites and any information contained in any other web site maintained by any of the underwriters is not part of this prospectus, has not been approved and/or endorsed by us or the underwriters and should not be relied upon by investors.

## **Other Activities and Relationships**

The underwriters and certain of their affiliates are full service financial institutions engaged in various activities, which may include securities trading, commercial and investment banking, financial advisory, investment management, investment research, principal investment, hedging, financing and brokerage activities. The underwriters and certain of their affiliates have, from time to time, performed, and may in the future perform, various commercial and investment banking and financial advisory services for us and our affiliates, for which they received or will receive customary fees and expenses.

In the ordinary course of their various business activities, the underwriters and certain of their affiliates may make or hold a broad array of investments and actively trade debt and equity securities (or related derivative securities) and financial instruments (including bank loans) for their own account and for the accounts of their customers, and such investment and securities activities may involve securities and/or instruments issued by us and our affiliates. If the underwriters or their respective affiliates have a lending relationship with us, they routinely hedge their credit exposure to us consistent with their customary risk management policies. The underwriters and their respective affiliates may hedge such exposure by entering into transactions which consist of either the purchase of credit default swaps or the creation of short positions in our securities or the securities of our affiliates, including potentially the common stock offered hereby. Any such short positions could adversely affect future trading prices of the common stock offered hereby. The underwriters and certain of their respective affiliates may also communicate independent investment recommendations, market color or trading ideas and/or publish or express independent research views in respect of such securities or instruments and may at any time hold, or recommend to clients that they acquire, long and/or short positions in such securities and instruments.

---

**Table of Contents**

**NOTICE TO INVESTORS**

**Australia**

This prospectus is not a disclosure document for the purposes of Australia's Corporations Act 2001 (Cth) of Australia, or Corporations Act, has not been lodged with the Australian Securities & Investments Commission and is only directed to the categories of exempt persons set out below. Accordingly, if you receive this prospectus in Australia:

**You confirm and warrant that you are either:**

- n a sophisticated investor under section 708(8)(a) or (b) of the Corporations Act;
- n a sophisticated investor under section 708(8)(c) or (d) of the Corporations Act and that you have provided an accountant's certificate to the company which complies with the requirements of section 708(8)(c)(i) or (ii) of the Corporations Act and related regulations before the offer has been made; or
- n a professional investor within the meaning of section 708(11)(a) or (b) of the Corporations Act.

To the extent that you are unable to confirm or warrant that you are an exempt sophisticated investor or professional investor under the Corporations Act any offer made to you under this prospectus is void and incapable of acceptance.

You warrant and agree that you will not offer any of the shares issued to you pursuant to this prospectus for resale in Australia within 12 months of those shares being issued unless any such resale offer is exempt from the requirement to issue a disclosure document under section 708 of the Corporations Act.

**European Economic Area**

In relation to each member state of the European Economic Area which has implemented the Prospectus Directive, each referred to herein as a Relevant Member State, with effect from and including the date on which the Prospectus Directive is implemented in that Relevant Member State, referred to herein as the Relevant Implementation Date, no offer of any securities which are the subject of the offering contemplated by this prospectus has been or will be made to the public in that Relevant Member State other than any offer where a prospectus has been or will be published in relation to such securities that has been approved by the competent authority in that Relevant Member State or, where appropriate, approved in another Relevant Member State and notified to the relevant competent authority in that Relevant Member State in accordance with the Prospectus Directive, except that with effect from and including the Relevant Implementation Date, an offer of such securities may be made to the public in that Relevant Member State:

- n to any legal entity which is a qualified investor as defined in the Prospectus Directive;
- n to fewer than 100 or, if the Relevant Member State has implemented the relevant provision of the 2010 PD Amending Directive, 150, natural or legal persons (other than qualified investors as defined in the Prospectus Directive), as permitted under the Prospectus Directive, subject to obtaining the prior consent of the

representatives of the underwriters for any such offer; or

n in any other circumstances falling within Article 3(2) of the Prospectus Directive, provided that no such offer of securities shall require the Company or any of the underwriters to publish a prospectus pursuant to Article 3 of the Prospectus Directive or supplement a prospectus pursuant to Article 16 of the Prospectus Directive.

For the purposes of this provision, the expression an offer to the public in relation to any securities in any Relevant Member State means the communication in any form and by any means of sufficient information on the terms of the offer and the securities to be offered so as to enable an investor to decide to purchase or subscribe the securities, as the same may be varied in that Relevant Member State by any measure implementing the Prospectus Directive in that Relevant Member State and the expression Prospectus Directive means Directive 2003/71/EC (and amendments thereto, including the 2010 PD Amending Directive, to the extent implemented in the Relevant Member State), and includes any relevant implementing measure in the Relevant Member State and the expression 2010 PD Amending Directive means Directive 2010/73/EU.

## **Table of Contents**

### **Hong Kong**

No securities have been offered or sold, and no securities may be offered or sold, in Hong Kong, by means of any document, other than to persons whose ordinary business is to buy or sell shares or debentures, whether as principal or agent; or to professional investors as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance; or in other circumstances which do not result in the document being a prospectus as defined in the Companies Ordinance (Cap. 32) of Hong Kong or which do not constitute an offer to the public within the meaning of the Companies Ordinance (Cap.32) of Hong Kong. No document, invitation or advertisement relating to the securities has been issued or may be issued or may be in the possession of any person for the purpose of issue (in each case whether in Hong Kong or elsewhere), which is directed at, or the contents of which are likely to be accessed or read by, the public of Hong Kong (except if permitted under the securities laws of Hong Kong) other than with respect to securities which are or are intended to be disposed of only to persons outside Hong Kong or only to professional investors as defined in the Securities and Futures Ordinance (Cap. 571) of Hong Kong and any rules made under that Ordinance.

This prospectus has not been registered with the Registrar of Companies in Hong Kong. Accordingly, this prospectus may not be issued, circulated or distributed in Hong Kong, and the securities may not be offered for subscription to members of the public in Hong Kong. Each person acquiring the securities will be required, and is deemed by the acquisition of the securities, to confirm that he is aware of the restriction on offers of the securities described in this prospectus and the relevant offering documents and that he is not acquiring, and has not been offered any securities in circumstances that contravene any such restrictions.

### **Japan**

The offering has not been and will not be registered under the Financial Instruments and Exchange Law of Japan (Law No. 25 of 1948 of Japan, as amended), or FIEL, and the Initial Purchaser will not offer or sell any securities, directly or indirectly, in Japan or to, or for the benefit of, any resident of Japan (which term as used herein means, unless otherwise provided herein, any person resident in Japan, including any corporation or other entity organized under the laws of Japan), or to others for re-offering or resale, directly or indirectly, in Japan or to a resident of Japan, except pursuant to an exemption from the registration requirements of, and otherwise in compliance with, the FIEL and any other applicable laws, regulations and ministerial guidelines of Japan.

### **Singapore**

This prospectus has not been and will not be lodged or registered with the Monetary Authority of Singapore. Accordingly, this prospectus and any other document or material in connection with the offer or sale, or the invitation for subscription or purchase of the securities may not be issued, circulated or distributed, nor may the securities be offered or sold, or be made the subject of an invitation for subscription or purchase, whether directly or indirectly, to the public or any member of the public in Singapore other than (1) to an institutional investor under Section 274 of the Securities and Futures Act, Chapter 289 of Singapore, or the SFA, (2) to a relevant person as defined under Section 275(2), or any person pursuant to Section 275(1A) of the SFA, and in accordance with the conditions, specified in Section 275 of the SFA, or (3) otherwise pursuant to, and in accordance with the conditions of any other applicable provision of the SFA.

Where the securities are subscribed or purchased under Section 275 of the SFA by a relevant person which is:

- n a corporation (which is not an accredited investor as defined under Section 4A of the SFA) the sole business of which is to hold investments and the entire share capital of which is owned by one or more individuals, each of whom is an accredited investor; or
  
- n a trust (where the trustee is not an accredited investor) whose sole purpose is to hold investments and each beneficiary is an accredited investor,  
shares, debentures and units of shares and debentures of that corporation or the beneficiaries rights and interest in that trust shall not be transferable for six months after that corporation or that trust has acquired the Offer Shares under Section 275 of the SFA except:
  
- n to an institutional investor under Section 274 of the SFA or to a relevant person defined in Section 275(2) of the SFA, or to any person pursuant to an offer that is made on terms that such shares, debentures and

## **Table of Contents**

units of shares and debentures of that corporation or such rights and interest in that trust are acquired at a consideration of not less than \$200,000 (or its equivalent in a foreign currency) for each transaction, whether such amount is to be paid for in cash or by exchange of securities or other assets, and further for corporations, in accordance with the conditions, specified in Section 275 of the SFA;

n where no consideration is given for the transfer; or

n where the transfer is by operation of law.

### **Switzerland**

The securities may not be publicly offered in Switzerland and will not be listed on the SIX Swiss Exchange, or SIX, or on any other stock exchange or regulated trading facility in Switzerland. This prospectus has been prepared without regard to the disclosure standards for issuance prospectuses under art. 652a or art. 1156 of the Swiss Code of Obligations or the disclosure standards for listing prospectuses under art. 27 ff. of the SIX Listing Rules or the listing rules of any other stock exchange or regulated trading facility in Switzerland. Neither this prospectus nor any other offering or marketing material relating to the securities or the offering may be publicly distributed or otherwise made publicly available in Switzerland.

Neither this prospectus nor any other offering or marketing material relating to the offering, the Company or the securities have been or will be filed with or approved by any Swiss regulatory authority. In particular, this prospectus will not be filed with, and the offer of securities will not be supervised by, the Swiss Financial Market Supervisory Authority FINMA, or FINMA, and the offer of securities has not been and will not be authorized under the Swiss Federal Act on Collective Investment Schemes, or CISA. The investor protection afforded to acquirers of interests in collective investment schemes under the CISA does not extend to acquirers of securities.

### **United Kingdom**

This prospectus is only being distributed to, and is only directed at, persons in the United Kingdom that are qualified investors within the meaning of Article 2(1)(e) of the Prospectus Directive that are also (1) investment professionals falling within Article 19(5) of the Financial Services and Markets Act 2000 (Financial Promotion) Order 2005, as amended, referred to herein as the Order, and/or (2) high net worth entities falling within Article 49(2)(a) to (d) of the Order and other persons to whom it may lawfully be communicated. Each such person is referred to herein as a Relevant Person.

This prospectus and its contents are confidential and should not be distributed, published or reproduced (in whole or in part) or disclosed by recipients to any other persons in the United Kingdom. Any person in the United Kingdom that is not a Relevant Person should not act or rely on this document or any of its contents.



**Table of Contents**

**LEGAL MATTERS**

The validity of the shares of common stock being offered by this prospectus will be passed upon for us by Cooley LLP, San Diego, California. Covington & Burling LLP, New York, New York is counsel for the underwriters in connection with this offering.

**EXPERTS**

The financial statements of Bellicum Pharmaceuticals, Inc. at December 31, 2013 and 2012, and for each of the two years in the period ended December 31, 2013, appearing in this Prospectus and Registration Statement have been audited by Ernst & Young LLP, independent registered public accounting firm, as set forth in their report thereon appearing elsewhere herein, and are included in reliance upon such report given on the authority of such firm as experts in accounting and auditing.

**WHERE YOU CAN FIND ADDITIONAL INFORMATION**

We have filed with the SEC a registration statement on Form S-1, including exhibits and schedules, under the Securities Act, with respect to the shares of common stock being offered by this prospectus. This prospectus, which constitutes part of the registration statement, does not contain all of the information in the registration statement and its exhibits. For further information with respect to us and the common stock offered by this prospectus, we refer you to the registration statement and its exhibits. Statements contained in this prospectus as to the contents of any contract or any other document referred to are not necessarily complete, and in each instance, we refer you to the copy of the contract or other document filed as an exhibit to the registration statement. Each of these statements is qualified in all respects by this reference.

You can read our SEC filings, including the registration statement, over the Internet at the SEC's website at [www.sec.gov](http://www.sec.gov). You may also read and copy any document we file with the SEC at its public reference facilities at 100 F Street, NE, Washington, D.C. 20549. You may also obtain copies of these documents at prescribed rates by writing to the Public Reference Section of the SEC at 100 F Street, NE, Washington, D.C. 20549. Please call the SEC at 1-800-SEC-0330 for further information on the operation of the public reference facilities. You may also request a copy of these filings, at no cost, by writing us at 2130 W. Holcombe Blvd., Ste. 800, Houston, TX 77030.

Upon the closing of this offering, we will be subject to the information reporting requirements of the Securities Exchange Act of 1934 and we will file reports, proxy statements and other information with the SEC. These reports, proxy statements and other information will be available for inspection and copying at the public reference room and web site of the SEC referred to above. We also maintain a website at [www.bellicum.com](http://www.bellicum.com), at which, following the closing of this offering, you may access these materials free of charge as soon as reasonably practicable after they are electronically filed with, or furnished to, the SEC. Information contained on or accessible through our website is not a part of this prospectus, and the inclusion of our website address in this prospectus is an inactive textual reference only.

**Table of Contents**

**INDEX TO FINANCIAL STATEMENTS**

**Bellicum Pharmaceuticals, Inc.**

**Nine Month Periods Ended September 30, 2014 and 2013**

<u>Unaudited Balance Sheet</u>	F-2
<u>Unaudited Statements of Operations</u>	F-3
<u>Unaudited Statements of Cash Flows</u>	F-4
<u>Notes to Unaudited Financial Statements</u>	F-5
<b>Year Ended December 31, 2013 and 2012</b>	
<u>Report of Independent Registered Public Accounting Firm</u>	F-15
<u>Balance Sheets</u>	F-16
<u>Statements of Operations</u>	F-17
<u>Statements of Redeemable and Convertible Preferred Stock and Stockholders' Deficit</u>	F-18
<u>Statements of Cash Flows</u>	F-19
<u>Notes to Financial Statements</u>	F-20

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Balance Sheets**

	<b>September 30, 2014 (Unaudited)</b>	<b>Pro forma September 30, 2014 (Unaudited)</b>	<b>December 31, 2013</b>
<b>ASSETS</b>			
Current assets:			
Cash and cash equivalents	\$ 61,931,951		\$ 11,167,585
Accounts receivable-grants	1,220,402		745,541
Prepaid expenses and other current assets	310,589		254,068
Total current assets	63,462,942		12,167,194
Property and equipment, net of accumulated depreciation	2,206,171		2,289,919
Other assets	661,529		484,525
<b>TOTAL ASSETS</b>	<b>\$ 66,330,642</b>		<b>\$ 14,941,638</b>
<b>LIABILITIES &amp; STOCKHOLDERS DEFICIT</b>			
Current liabilities:			
Accounts payable	\$ 1,691,526		549,594
Accrued payroll			470,961
Accrued liabilities	645,067		664,173
Warrant liability	10,598,453		
Current portion of line of credit	423,785		400,000
Current portion of deferred rent	97,336		102,713
Current portion of deferred manufacturing costs	157,686		17,000
Total current liabilities	13,613,853		2,204,441
Long-term liabilities:			
Line of credit	157,763		400,000
Deferred rent	228,318		288,941
Deferred manufacturing costs	371,771		274,267
<b>TOTAL LIABILITIES</b>	<b>14,371,705</b>		<b>3,167,649</b>
Preferred stock whose redemption is outside the control of the issuer:			
Series A convertible, redeemable preferred stock; \$0.01 par value; 2,600,000 shares authorized; 2,544,539 shares issued and outstanding as of September 30, 2014 and	7,633,617		7,633,617

December 31, 2013; redemption value of \$7,633,617 at September 30, 2014 and December 31, 2013			
Series B convertible, redeemable preferred stock; \$0.01 par value; 8,200,000 shares authorized; 8,145,988 and 6,563,283 shares issued and outstanding as of September 30, 2014 and December 31, 2013, respectively; redemption value of \$41,044,654 and \$32,292,269 at September 30, 2014 and December 31, 2013, respectively	41,044,654		32,292,269
Series C convertible, redeemable preferred stock; \$0.01 par value; 16,700,000 shares authorized; 10,091,743 shares issued and outstanding as of September 30, 2014; redemption value of \$55,000,000 at September 30, 2014	42,074,484		
Stockholders' deficit:			
Common stock: \$0.01 par value; 37,500,000 shares of authorized; 2,124,386 and 1,725,992 shares issued and outstanding as of September 30, 2014 and December 31, 2013, respectively	21,244	258,169	17,260
Additional paid in capital	(169,163)	262,313,770	809,667
Accumulated deficit	(38,645,899)	(83,559,259)	(28,978,824)
Total stockholders' deficit	(38,793,818)	179,012,680	(28,151,897)
TOTAL LIABILITIES AND STOCKHOLDERS' DEFICIT	\$ 66,330,642		14,941,638

The accompanying notes are an integral part of these financial statements

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Statements of Operations (unaudited)**

	<b>Nine Months Ended September 30,</b>	
	<b>2014</b>	<b>2013</b>
<b>REVENUES</b>		
Grants	\$ 1,765,521	\$ 1,121,699
<b>OPERATING EXPENSES</b>		
Research and development	7,077,938	4,563,776
General and administrative	3,135,036	1,997,342
Total operating expenses	10,212,974	6,561,118
<b>LOSS FROM OPERATIONS</b>	<b>(8,447,453)</b>	<b>(5,439,419)</b>
<b>OTHER INCOME (EXPENSE):</b>		
Interest income	15,108	2,344
Interest expense	(37,712)	(38,473)
Change in fair value of warrant liability	(1,197,018)	
Total other income (expenses)	(1,219,622)	(36,129)
<b>NET LOSS</b>	<b>\$ (9,667,075)</b>	<b>\$ (5,475,548)</b>
Preferred stock dividends accrued but not declared	(1,432,370)	(695,072)
Net loss attributable to common stockholders	\$ (11,099,445)	\$ (6,170,620)
Net loss per share attributable to common stockholders, basic and diluted	\$ (5.45)	\$ (3.58)
Weighted average number of common shares outstanding Basic and diluted	2,036,025	1,725,992
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)	(0.98)	
Weighted average common shares used to compute pro forma net loss per share attributable to common shareholders, basic and diluted (unaudited)	9,827,767	

The accompanying notes are an integral part of these financial statements

F-3

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Statements of Cash Flows (unaudited)**

	<b>Nine Months Ended September 30,</b>	
	<b>2014</b>	<b>2013</b>
<b>CASH FLOWS FROM OPERATING ACTIVITIES</b>		
Net Loss	\$ (9,667,075)	\$ (5,475,548)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	484,755	417,590
Stock-based compensation	245,574	292,736
Amortization of lease liability	(71,625)	(71,625)
Loss on warrant liability	1,197,018	
Other		3,391
Changes in operating assets and liabilities		
Accounts receivable-grants	(474,861)	(68,523)
Prepaid expenses and other current assets	(56,521)	600,345
Other assets	288,947	(48,851)
Accounts payable	1,141,932	(210,261)
Accrued payroll	(470,961)	(321,962)
Accrued liabilities	(19,106)	126,397
Deferred revenue-grants		(1,000,129)
Deferred rent	5,625	9,040
Deferred manufacturing	238,190	180,200
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<b>(7,158,108)</b>	<b>(5,567,200)</b>
<b>CASH FLOWS FROM INVESTING ACTIVITIES</b>		
Purchase of property and equipment	(401,007)	(283,260)
<b>NET CASH USED IN INVESTING ACTIVITIES</b>	<b>(401,007)</b>	<b>(283,260)</b>
<b>CASH FLOWS FROM FINANCING ACTIVITIES</b>		
Proceeds from issuance of preferred stock	62,320,015	6,200,840
Payment of issuance costs	(3,524,081)	(6,655)
Proceeds from issuance of common stock	211,950	
Payment of deferred offering costs	(465,951)	
Proceeds from note payable		3,500,000
Proceeds from line of credit	81,548	550,223
Payments on line of credit	(300,000)	(100,000)

NET CASH PROVIDED BY FINANCING ACTIVITIES	58,323,481	10,144,408
NET CHANGE IN CASH AND CASH EQUIVALENTS	50,764,366	4,293,948
CASH AND CASH EQUIVALENTS AT BEGINNING OF PERIOD	11,167,585	1,632,084
CASH AND CASH EQUIVALENTS AT END OF PERIOD	\$ 61,931,951	\$ 5,926,032
NON-CASH INVESTING AND FINANCING ACTIVITIES		
Dividends accreted on preferred stock	\$ 1,432,370	\$ 695,072
Conversion of notes payable into preferred stock		3,500,000

The accompanying notes are an integral part of these financial statements

F-4



**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to Unaudited Financial Statements**

**Note 1 Organization and Business**

Bellicum Pharmaceuticals, Inc. (the Company or Bellicum), was incorporated in Delaware in July 2004 and is based in Houston, Texas. The Company is a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. The Company is devoting substantially all of its present efforts to developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including, hematopoietic stem cell transplantation, CAR T cell therapy and dendritic cell vaccines. The Company has not generated any revenue from product sales to date and does not anticipate generating revenues from product sales in the foreseeable future. The Company's success is dependent on, among other things, its ability to successfully complete the development of, and obtain regulatory approval for, its product candidates, managing the growth of the organization, obtaining additional financing necessary in order launch and commercialize its products candidates, and competing successfully with other companies in its industry.

The future success of the Company is dependent on its ability to develop its product candidates and ultimately upon its ability to attain profitable operations. The Company is subject to a number of risks similar to other life science companies, including, but not limited to, successful discovery and development of its technology, raising additional capital, development by its competitors of new technological innovations, protection of proprietary technology, and market acceptance of the Company's products. As a result of the aforementioned factors and the related uncertainties, there can be no assurance of the Company's future success.

**Note 2 Significant Accounting Policies**

**Basis of Presentation**

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America. Any reference in these notes to applicable guidance is meant to refer to the authoritative U.S. generally accepted accounting principles (GAAP) as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

The accompanying unaudited balance sheet as of September 30, 2014 and the interim statements of operations and cash flows for the nine months ended September 30, 2014 and 2013 have been prepared in accordance with generally accepted accounting principles for interim financial information. Accordingly, they do not include all of the information and footnotes required by generally accepted accounting principles for complete financial statements. In the opinion of management, all normal recurring adjustments considered necessary for a fair presentation have been included. Operating results for the nine-month period ended September 30, 2014 are not necessarily indicative of the results that may be expected for the year ended December 31, 2014. The balance sheet as of December 31, 2013 included herein was derived from the audited financial statements as of that date. These financial statements should be read in conjunction with the Company's audited financial statements included elsewhere in this prospectus.

**Pro Forma Stockholders' Equity**

The pro forma stockholders' equity as of September 30, 2014, presents the Company's stockholders' equity as though all of its then-outstanding convertible preferred stock had automatically converted into shares of common stock upon the

closing of an initial public offering (IPO) of its common stock. In addition, the pro forma stockholders' equity assumes the common stock issuance for payment of the accrued Series B dividends, the exercise of the common stock warrants at the net exercise price, and the exercise of the convertible preferred stock warrant liability into shares of convertible preferred stock, which would expire if not exercised upon the date immediately following the date of effectiveness of the registration statement, of which this prospectus forms a part, on or prior to March 31, 2015.

### **Revenue Recognition**

The Company has not yet generated any revenue from product sales. The Company's sole source of revenue is grant revenue related to a \$5.7 million research grant received from the Cancer Prevention and Research Institute of Texas

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

(CPRIT), covering a three-year period from July 1, 2011 through June 30, 2014, and a \$0.7 million research grant from the National Institutes of Health (NIH). Grant payments received prior to the Company's performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred. (See Note 7)

**Cash and Cash Equivalents**

The Company considers all short-term, highly liquid investments with maturity of three months or less to be cash equivalents.

**Property and Equipment**

Leasehold improvements, furniture, equipment and software are recorded at cost and are depreciated using the straight-line method over the estimated useful lives of the related assets, which range from three to five years.

**Deferred Rent**

The Company recognizes rent expense for leases with increasing annual rents on a straight-line basis over the term of the lease. The amount of rent expense in excess of cash payments is classified as deferred rent. Any lease incentives received are deferred and amortized over the term of the lease.

**Clinical Trials**

The Company estimates its clinical trial expense accrual for a given period based on the number of patients enrolled at each site and the length of time each patient has been in the trial, less amounts previously billed.

**Redeemable Preferred Stock Warrant Liability**

Freestanding warrants for shares that are either puttable or redeemable are classified as liabilities on the balance sheet and are carried at their estimated fair value. The Series C Redeemable Preferred Stock, which is able to be purchased with the warrants, is redeemable upon certain deemed liquidation events outside the control of the Company. As such, these warrants are classified as liabilities, and at the end of each reporting period, changes in the estimated fair value during the period are recorded in other income (expense). The Company will continue to adjust the carrying value of the warrants until the earlier of the exercise or termination of the warrants.

**Fair Value of Financial Instruments**

Accounting standards include disclosure requirements around fair values used for certain financial instruments and establish a fair value hierarchy. The three-tier hierarchy prioritizes valuation inputs into three levels based on the extent to which inputs used in measuring fair value are observable in the market.

Fair value measurements are classified and disclosed in one of the following three categories:

- n Level 1 Quoted unadjusted prices for identical instruments in active markets.
- n Level 2 Quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-derived valuations in which all observable inputs and significant value-drivers are observable in active markets.
- n Level 3 Model derived valuations in which one or more significant inputs or significant value-drivers are unobservable, including assumptions developed by the Company.

**Table of Contents****BELLICUM PHARMACEUTICALS, INC.**

Financial assets and liabilities that have recurring fair value measurements are shown below:

<b>Description</b>	<b>Total</b>	<b>Fair Value Measurements at September 30, 2014, Using</b>		
		<b>Quoted Prices in Active Markets for Identical Assets Level 1</b>	<b>Significant Other Observable Inputs Level 2</b>	<b>Significant Unobservable Inputs Level 3</b>
<b>Financial assets:</b>				
Money market fund	\$ 61,898,983	\$ 61,898,983	\$	\$
<b>Total financial assets</b>	<b>\$ 61,898,983</b>	<b>\$ 61,898,983</b>	<b>\$</b>	<b>\$</b>
<b>Financial liabilities:</b>				
Series C Redeemable Preferred Stock warrants	\$ 10,598,453	\$	\$	\$ 10,598,453
<b>Total financial liabilities</b>	<b>\$ 10,598,453</b>	<b>\$</b>	<b>\$</b>	<b>\$ 10,598,453</b>

<b>Fair Value Measurements at December 31, 2013, Using</b>		
<b>Quoted Prices in Active Markets for</b>	<b>Significant Other Observable Inputs</b>	<b>Significant Unobservable Inputs</b>

<b>Description</b>	<b>Total</b>	<b>Identical Assets Level 1</b>	<b>Level 2</b>	<b>Level 3</b>
<b>Financial assets:</b>				
Money market fund	\$ 10,879,656	\$ 10,879,656	\$	\$
<b>Total financial assets</b>	<b>\$ 10,879,656</b>	<b>\$ 10,879,656</b>	<b>\$</b>	<b>\$</b>

Level 3 liabilities comprise redeemable preferred stock warrant liabilities. These warrants are classified as liabilities because the Series C Redeemable Preferred shares that are able to be purchased by these warrants are redeemable and, therefore, are classified as temporary equity. The following table sets forth a summary of the changes in the estimated fair value of the Company's redeemable preferred stock warrants, which were measured at fair value on a recurring basis since inception:

Balance as of August 22, 2014 (issuance of warrants)	\$ 9,401,435
Net increase in fair value included in other (income) expense	1,197,018
<b>Balance as of September 30, 2014</b>	<b>\$ 10,598,453</b>

The fair value of the outstanding Series C Redeemable Preferred Stock warrants is measured using the Black-Scholes option-pricing model. Inputs used to determine estimated fair value include the estimated fair value of the warrant, as determined by the fair value of the underlying stock relative to the warrant exercise price at the valuation

**Table of Contents****BELLICUM PHARMACEUTICALS, INC.**

measurement date, volatility of the price of the underlying stock, the expected term of the warrants, risk-free interest rates, and expected dividends.

The fair value of the warrants has been estimated, with the following assumptions at each measurement date:

	<b>August 22, 2014</b>	<b>September 30, 2014</b>
Volatility	0.53%	0.58%
Risk-free interest rate	88.6%	88.6%
Expected dividend yield	0%	0%
Expected life	1.93 years	1.82 years

The Company believes the recorded values of their financial instruments, including cash and cash equivalents, accounts receivable, accounts payable, and accrued liabilities approximate their fair values due to the short-term nature of these instruments. The carrying amount of the line of credit approximates fair value as it bears interest at variable rates.

**Financial Instruments and Credit Risks**

Financial instruments that potentially subject the Company to credit risk include cash and cash equivalents and accounts receivable. Cash is deposited in demand accounts in federally insured domestic institutions to minimize risk. Insurance is provided through the Federal Deposit Insurance Corporation (FDIC) and Security Investor Protection Corporation (SIPC). Although the balances in these accounts exceed the federally insured limit from time to time, the Company has not incurred losses related to these deposits.

**Debt**

The Company records proceeds from debt issuances at their face value, less any discounts or the value of any beneficial conversion features or detachable warrants. Interest is accrued over the term of the debt, at the stated interest rate. Discounts are amortized to interest expense through the effective interest method over the term of the debt. Unamortized discounts are immediately recognized as interest expense upon conversion or repayment of the debt.

**Equity Issuance Costs**

Equity issuance costs represent costs paid to third parties in order to obtain equity financing. These costs have been netted against the proceeds of the equity issuances.

**Licenses and Patents**

Licenses and patent costs are expensed as incurred. Costs related to the license of patents from third parties and internally developed patents are classified as research and development expenses. Legal costs related to patent applications and maintenance is classified as general and administrative expenses.

### **Research and Development**

Research and development expenses include salaries, related payroll expenses, consulting fees, laboratory costs, manufacturing costs for clinical trials, licenses and clinical trial expenses. All costs for research and development are expensed as incurred.

### **Contract Manufacturing Services**

Contract manufacturing services are expensed as incurred. Prepaid costs are capitalized and amortized as services are performed.

### **Stock-Based Compensation**

The Company accounts for its stock-based compensation in accordance with ASC 718, *Compensation Stock Compensation*, which requires the measurement and recognition of compensation expense for all stock-based payment awards made to employees and directors to be recognized in the financial statements, based on their fair value. The Company measures stock-based compensation to consultants in accordance with ASC 505-50, *Equity-Based Payments to Non-Employees*, and recognizes the fair value of the award over the period the services are rendered or goods are provided.



**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

The Company uses the Black-Scholes option-pricing model to estimate the fair value of stock option awards. The fair value is recognized as expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award on a straight-line basis. The Company believes that the fair value of stock options granted to non-employee consultants is more reliably measured than the fair value of the services received. The determination of the grant date fair value of options using the Black-Scholes option-pricing model is affected by the Company's estimated common stock fair value, as well as assumptions regarding a number of other complex and subjective variables.

**Income Taxes**

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. This method also requires the recognition of future tax benefits such as net operating loss and tax credit carry forwards, to the extent that realization of such benefits is more likely than not. A valuation allowance is recorded when the realization of future tax benefits is uncertain. The Company records a valuation allowance for the full amount of deferred tax assets, which would otherwise be recorded for tax benefits relating to the operating loss and tax credit carryforwards, as realization of such deferred tax assets cannot be determined to be more likely than not.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740, *Income Taxes*. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of September 30, 2014 and 2013, the Company had no uncertain tax positions and no interest or penalties have been charged to the Company for the nine months ended September 30, 2014 and 2013. If incurred, the Company will classify any interest and penalties as a component of interest expense and operating expense, respectively. The Company is subject to routine audits by taxing jurisdictions; however, there are currently no audits for any tax periods in progress. The tax years 2004 through 2013 remain open to examination by the Internal Revenue Service.

**Comprehensive Income (Loss)**

Comprehensive income (loss) is defined as the change in equity of a business enterprise during a period, from transactions, and other events and circumstances from non-owner sources. For the nine months ended September 30, 2014 and 2013, net loss equaled comprehensive loss.

**Use of Estimates**

The preparation of the financial statements in accordance with GAAP requires management to make certain estimates and judgments that affect the reported amounts of assets, liabilities, and expenses. Actual results could differ from those estimates.

Historically, the fair values of the shares of common stock underlying the Company's share-based awards were estimated on each grant date by its Board of Directors. Given the absence of a public trading market for the Company's common stock, its Board of Directors exercised reasonable judgment and considered a number of objective and subjective factors to determine the best estimate of the fair value of its common stock, including the following:

- n its stage of development;
- n its operational and financial performance;
- n the nature of its services and its competitive position in the marketplace;
- n the value of companies that it considers peers based on a number of factors, including similarity to the Company with respect to industry and business model;

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

- n the likelihood of achieving a liquidity event, such as an initial public offering and the nature and history of its business;
- n issuances of preferred stock and the rights, preferences, and privileges of its preferred stock relative to those of its common stock;
- n business conditions and projections;
- n the history of the Company and progress of its research and development efforts and clinical trials; and
- n the lack of marketability of its common stock.

**Deferred Offering Costs**

Deferred offering costs, consisting of legal, accounting, and filing fees related to the IPO, are capitalized. The deferred offering costs will be offset against proceeds from the IPO upon the effectiveness of the offering. In the event the offering is terminated, all capitalized deferred offering costs will be expensed. As of September 30, 2014, \$465,951 of deferred offering costs were capitalized, which are included in other assets in the accompanying balance sheets. No amounts were deferred at December 31, 2013.

**Net Loss and Unaudited Pro Forma Net Loss per Share of Common Stock Attributable to Common Stockholders**

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding during the period without consideration for common stock equivalents. Diluted net loss per share of common stock is the same as basic net loss per share of common stock, since the effects of potentially dilutive securities are antidilutive. The net loss per share of common stock attributable to common stockholders is computed using the two-class method required for participating securities. All series of the Company's convertible preferred stock are considered to be participating securities as they are entitled to participate in undistributed earnings with shares of common stock. Due to the Company's net loss, there is no impact on the earnings per share calculation in applying the two-class method since the participating securities have no legal requirement to share in any losses.

The unaudited pro forma loss per share attributable to common stockholders for the nine months ended September 30, 2014 was computed using the weighted-average number of shares of common stock outstanding, including the pro forma effect of the conversion of all then-outstanding shares of redeemable convertible preferred stock into shares of common stock and the effect of the exercise of certain then-outstanding warrants that will terminate if not exercised upon the IPO, and excluding the pro forma effect of the common stock issuance for payment of the accrued Series B dividends. For the purpose of the pro forma presentation, the Company has assumed the net exercise of common warrants that will terminate if not exercised and the cash exercise of the Series C preferred warrants that will terminate if not exercised.

The following outstanding shares of common stock equivalents were excluded from the computations of diluted net loss per shares of common stock attributable to common stockholders for the periods presented as the effect of including such securities would be anti-dilutive:

	<b>Nine-months ended</b>	
	<b>September 30,</b>	
	<b>2014</b>	<b>2013</b>
Series A Convertible preferred stock as converted to common stock	1,496,782	1,496,782
Series B Convertible preferred stock as converted to common stock	4,791,740	2,910,675
Series C Convertible preferred stock as converted to common stock	5,936,297	
Warrants to purchase Series C Convertible preferred stock-as converted to common stock	3,858,549	
Warrants to purchase common stock	473,031	866,570
Options to purchase common stock	1,602,339	1,568,529
Stock dividends to be issued as payment for Series B accrued dividends	177,349	80,976
	18,336,087	6,923,532

**Table of Contents****BELLICUM PHARMACEUTICALS, INC.**

The following table sets forth the computations of unaudited pro forma basics and diluted net loss per share attributable to common stockholders:

	<b>Nine Months Ended September 30, 2014</b>
Net Loss used in computing net loss per share attributable to common stockholders, basic and diluted	\$ (9,667,075)
Pro forma net loss used in computing net loss per share attributable to common stockholders, basic and diluted	\$ (9,667,075)
Weighted-average common shares used to compute net loss per share attributable to common stockholders, basic and diluted	2,036,025
Pro forma adjustment to reflect assumed conversion of convertible preferred stock	7,110,591
Pro forma adjustment to reflect assumed conversion of warrants	681,151
Weighted-average common shares used to compute pro forma net loss per share attributable to common stockholders, basic and diluted	9,827,767
Pro forma net loss per share of common stock, basic and diluted	(0.98)

**Recently Issued Accounting Pronouncements**

In June 2014, the Financial Accounting Standards Board issued Accounting Standards Update No. 2014-10 (ASU No. 2014-10), which eliminated the definition of a Development Stage Entity and the related reporting requirements. ASU No. 2014-10 is effective for annual reporting periods beginning after December 15, 2014, with early adoption allowed. The Company early adopted ASU No. 2014-10, effective in its financial statements for the years ended December 31, 2013 and 2012.

In August 2014, the FASB issued Accounting Standard Update No. 2014-15 (ASU No. 2014-15), Presentation of Financial Statements – Going Concern (Subtopic 205-40), which requires management to assess an entity’s ability to continue as a going concern by incorporating and expanding upon certain principles that are currently in U.S. auditing standards. Specifically, ASU 2014-15 provides a definition of the term substantial doubt and requires an assessment for a period of one year after the date that the financial statements are issued (or available to be issued). It also requires certain disclosures when substantial doubt is alleviated as a result of consideration of management’s plans and requires an express statement and other disclosures when substantial doubt is not alleviated. ASU No. 2014-15 is effective for fiscal years, and interim periods within those years, beginning after December 15, 2016, and early application is permitted.

### **Reverse Stock Split**

On December 4, 2014 and December 5, 2014, respectively, our board of directors and our stockholders approved an amendment to our amended and restated certificate of incorporation to effect a reverse split of shares of our common stock on a 1-for-1.7 basis (the Reverse Stock Split). The par value and the authorized shares of the common stock were not adjusted as a result of the Reverse Stock Split. All issued and outstanding common stock, options for common stock, warrants for common stock, and per share amounts contained in the financial statements have been retroactively adjusted to reflect this Reverse Stock Split for all periods presented.

The Company has evaluated other recent accounting pronouncements and believes that none of them will have a material effect on the Company’s financial statements.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Note 3 Series C Preferred Stock**

On August 22, 2014, the Company issued 10,091,743 shares of Series C convertible preferred stock (Series C) at a purchase price of \$5.45 per share and warrants to purchase up to 6,559,598 shares of Series C with an exercise price of \$6.00 per share. The warrants have a five year term, but are subject to earlier termination in the event of a Qualified IPO (defined in the warrant agreement), on or prior to March 31, 2015, or upon a merger or sale of the Company. The Company received net proceeds from the transaction of \$51.5 million, net of offering costs of \$3.5 million.

The rights, preferences and privileges of the Series C, as of September 30, 2014 are as follows:

*Optional Conversion*

Each share of Series C is convertible, at the option of the holder at any time and without additional consideration, on a 1-for-1.7 basis at the conversion price of \$5.45. The rate at which shares of Series C may be converted into shares of common stock, is subject to anti-dilution protection in the event of certain dilutive issuances of capital stock.

*Mandatory Conversion*

Upon the closing of the sale of the Company's common stock in an IPO at a price per share of at least \$6.50 (as adjusted for splits, dividends and the like) and resulting in at least \$50.0 million of gross proceeds to the Company, all of the outstanding shares of Series C will automatically convert into shares of the Company's common stock, at the then-applicable conversion rate, and such shares may not be reissued by the Company.

*Dividends*

The holders of Series C are entitled to 8% non-cumulative dividends per annum payable only when, as and if declared by the Board of Directors of the Company.

*Liquidation*

In the event of any deemed liquidation event, which is defined in the Company's certificate of incorporation to include (1) a merger or consolidation of the Company in which the Company or a subsidiary is a constituent party (subject to certain exceptions), (2) the sale, lease, transfer, exclusive license or other disposition of all or substantially all of the Company's assets, or (3) the sale of outstanding shares of the capital stock of the Company representing at least 50% of the outstanding capital stock or at least 50% of the voting power of the outstanding capital stock (subject to certain exceptions), or any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of Series C then outstanding are entitled to be paid out of the assets of the Company available for distribution to its stockholders, before any payment shall be made to the holders of Series A, B or common stock, an amount per share equal to the original issue price of the Series C, plus any dividends declared but unpaid thereon (Liquidation Amount).

*Redemption*

The Series C is not redeemable except in a deemed liquidation event.

The Company has determined Series C is not probable of becoming redeemable, as such Series C will not be accreted to redemption value until such time as the shares are probable of being redeemable.

*Voting*

Each holder of outstanding shares of Series C is entitled to cast the number of votes equal to the number of whole shares of common stock into which the shares of Series C held by the holder are convertible. Except as provided by law or by the other provisions of the Company's Certificate of Incorporation, holders of Preferred Stock vote together with the holders of common stock as a single class.

**Note 4 Series C Warrants**

In connection with the Series C convertible preferred stock, the Company issued immediately exercisable warrants (Series C warrants) to purchase 6,559,598 shares of Series C convertible preferred stock with an exercise price of \$6.00 per share which are convertible into 3,858,549 common shares. The Company determined the fair value of the Series C warrants on the date of issuance to be \$9,401,435, as determined using the Black-Scholes option-pricing model which was recorded as a warrant liability. The Company revalued the Series C warrant at September 30, 2014



**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

using the Black-Scholes option-pricing model. The fair value of the Series C warrant was estimated to be \$10,598,453 as at September 30, 2014. The Company recorded \$1,197,018 as a non-cash loss on warrant liability as current period expense.

**Note 5 Amendment to Features of Series A and B Convertible Preferred Stock**

During the quarter ended September 30, 2014, the Company filed an Amended and Restated Certificate of Incorporation with which the following features of the Series A and B Preferred Stock have been modified:

*Dividends*

Effective August 22, 2014, the dividend on the Series B Preferred Stock has been changed from cumulative to non-cumulative. Series B holders are entitled to 8% instead of 6% dividend, payable only when declared by the Board of Directors. The 8% dividend applies to both holders of Series A and B.

*Liquidation*

Effective August 22, 2014, Series C has preferential liquidation rights over Series A and B. In the event of any deemed liquidation event or any voluntary or involuntary liquidation, the holders of C are entitled to receive distribution before the holders of Series A and B holders.

*Redemption*

Effective August 22, 2014, Series A and B are redeemable only upon a deemed liquidation event, which is defined in the Company's certificate of incorporation to include (1) a merger or consolidation of the Company in which the Company or a subsidiary is a constituent party (subject to certain exceptions), (2) the sale, lease, transfer, exclusive license or other disposition of all or substantially all of the Company's assets, or (3) the sale of outstanding shares of the capital stock of the Company representing at least 50% of the outstanding capital stock or at least 50% of the voting power of the outstanding capital stock (subject to certain exceptions).

The effect of the modification of Series A and B features resulted in the stock no longer being probable of becoming redeemable. As such, beginning on August 22, 2014, Series A and B will not be accreted to redemption value until such time as the shares are probable of being redeemable.

**Note 6 Share-Based Compensation**

The Company's stock option activity and related information are summarized as follows:

	<b>Options</b>	<b>Weighted Average Exercise Price</b>	<b>Weighted Average Remaining Contractual Term</b>
Options outstanding, January 1, 2014	1,574,398	\$ 2.33	8.26
Options granted	39,706	\$ 2.55	
Options exercised	(4,872)		
Options forfeited	(6,893)		
Options outstanding, September 30, 2014	1,602,339	\$ 2.33	7.55
Exercisable at September 30, 2014	1,113,884	\$ 2.24	7.37

The weighted average grant date fair value of options granted in the nine months ended September 30, 2014 was \$1.62.

The Company calculates the intrinsic value of its options by multiplying the number of options by the difference between the estimated fair value per share for its common stock and the options exercise price. The aggregate intrinsic value of options exercisable and options outstanding at September 30, 2014 was \$5,814,316 and \$8,214,160, respectively. The Company will issue new shares of common stock upon the exercise of vested options.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

Stock-based compensation expense for the nine months ended September 30, 2014 and 2013 was \$245,574 and \$292,736, respectively. At September 30, 2014, total compensation cost not yet recognized was \$328,714, and the weighted average period over which this amount is expected to be recognized is 1.82 years.

**Note 7 Grants**

**CPRIT Grant**

On July 27, 2011, the Company entered into a Cancer Research Grant Contract (Grant Contract) with CPRIT under which CPRIT awarded a grant not to exceed approximately \$5.7 million to be used by the Company for the execution of defined clinical development of BPX-501. In addition, CPRIT could award supplemental funding not to exceed 10% of the total grant amount based upon the Company's progress. The terms of the Grant Contract require the Company to pay tiered royalties on revenues from sales and licenses of intellectual property facilitated by the Grant Contract.

During the nine months ended September 30, 2014 and 2013, Bellicum incurred \$1.7 million and \$1.0 million of expenses under the Grant Contract, respectively. As of September 30, 2014 and December 31, 2013, Bellicum had an outstanding grant receivable of \$1.0 million and \$0.7 million, respectively, for grant expenditures that were paid but have not been reimbursed.

**NIH Grant**

On March 25, 2013, the Company was awarded \$361,644 under a grant from NIH. The award covers the period from April 2013 through March 2014. The award was made pursuant to the authority of 42 USC 241 42 CFR 52, and is subject to the requirements of the statute. Funds spent on the grant are reimbursed through monthly reimbursement requests. In May of 2014, the NIH awarded an additional \$332,608 for the grant year from April 2014 through March 2015.

During the nine months ended September 30, 2014 and 2013, Bellicum incurred \$318,553 and \$121,570 of expenses under the grant, respectively. As of September 30, 2014 and December 31, 2013, Bellicum had an outstanding grant receivable of \$195,760 and \$28,935, respectively, for grant expenditures that were paid but have not been reimbursed.

**Note 8 Subsequent Events**

The Company evaluated subsequent events through the date the accompanying financial statements were available to be issued, which was November 18, 2014.

On October 3, 2014, the Company entered into an omnibus amendment agreement with ARIAD, which amended the Amended ARIAD License to expand the license to cover a broader scope of dimerizers and licensed products for use and exploitation in any field of use other than *in vivo* administration of genetic material directly into a human being using viral vectors for the purpose of producing proteins or other macromolecules that are expressed or secreted for therapeutic or prophylactic purposes. In connection with the amendment, the Company agreed to pay to ARIAD \$50,000,000 for expanded use of the license and termination of all obligations to make milestone and royalty payments to ARIAD in the future.

In connection with the amendment, the Company issued a promissory note to ARIAD for a principal amount of \$35,000,000 (the Note). The principal does not accrue interest unless the Company is in default, in which case it accrues at a rate of 10% per annum. The Company made an initial payment of \$15,000,000 in connection with the execution of the amendment. Pursuant to the Note, the Company is required to pay \$20,000,000 in a lump sum installment on or before June 30, 2015 and \$15,000,000 in a second lump sum installment on or before June 30, 2016 (or earlier under certain specified circumstances). Additionally, in connection with the first installment on the Note, ARIAD agreed to return to the Company all shares of common stock of the Company that ARIAD currently holds.

In November 2014, the Company borrowed an additional \$208,202 on the line of credit.

**Table of Contents**

Report of Independent Registered Public Accounting Firm

The Board of Directors of Bellicum Pharmaceuticals, Inc.

We have audited the accompanying balance sheets of Bellicum Pharmaceuticals, Inc. as of December 31, 2013 and December 31, 2012, and the related statements of operations, statements of redeemable and convertible preferred stock and stockholders' deficit and cash flows for each of the two years in the period ended December 31, 2013. These financial statements are the responsibility of the Company's management. Our responsibility is to express an opinion on these financial statements based on our audits.

We conducted our audits in accordance with the standards of the Public Company Accounting Oversight Board (United States). Those standards require that we plan and perform the audit to obtain reasonable assurance about whether the financial statements are free of material misstatement. We were not engaged to perform an audit of the Company's internal control over financial reporting. Our audits included consideration of internal control over financial reporting as a basis for designing audit procedures that are appropriate in the circumstances, but not for the purpose of expressing an opinion on the effectiveness of the Company's internal control over financial reporting. Accordingly, we express no such opinion. An audit also includes examining, on a test basis, evidence supporting the amounts and disclosures in the financial statements, assessing the accounting principles used and significant estimates made by management, and evaluating the overall financial statement presentation. We believe that our audits provide a reasonable basis for our opinion.

In our opinion, the financial statements referred to above present fairly, in all material respects, the financial position of Bellicum Pharmaceuticals, Inc. at December 31, 2013 and December 31, 2012, and the results of its operations and its cash flows for each of the two years in the period ended December 31, 2013, in conformity with U.S. generally accepted accounting principles.

/s/ Ernst & Young LLP

Houston, Texas

October 17, 2014, except as to Note 14, as to which the date is December 8, 2014

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Balance Sheets**

	<b>DECEMBER 31,</b>	
	<b>2013</b>	<b>2012</b>
<b>ASSETS</b>		
Current assets:		
Cash and cash equivalents	\$ 11,167,585	\$ 1,632,084
Accounts receivable grants	745,541	
Prepaid expenses and other current assets	254,068	845,578
Total current assets	12,167,194	2,477,662
Property and equipment, net of accumulated depreciation	2,289,919	2,510,829
Other assets	484,525	197,738
<b>TOTAL ASSETS</b>	<b>\$ 14,941,638</b>	<b>\$ 5,186,229</b>
<b>LIABILITIES AND STOCKHOLDERS' DEFICIT</b>		
Current liabilities:		
Accounts payable	\$ 549,594	\$ 550,847
Accrued payroll	470,961	321,962
Accrued liabilities	664,173	114,690
Deferred revenue		1,038,763
Current portion of line of credit	400,000	89,955
Current portion of deferred rent	102,713	95,501
Current portion of deferred manufacturing costs	17,000	10,200
Total current liabilities	2,204,441	2,221,918
Long-term liabilities:		
Line of credit	400,000	359,822
Deferred rent	288,941	378,023
Deferred manufacturing costs	274,267	41,933
Total liabilities	3,167,649	3,001,696
Commitments and contingencies		
Preferred stock whose redemption is outside the control of the issuer:		
Series A convertible, redeemable preferred stock: \$0.01 par value; 2,800,000 shares authorized; 2,544,539 shares issued and outstanding as of December 31, 2013 and 2012; redemption value of \$7,633,617 at December 31, 2013 and 2012	7,633,617	7,633,617
	32,292,269	14,024,356

Series B convertible, redeemable preferred stock: \$0.01 par value; 8,900,000 shares authorized; 6,563,283 and 2,849,929 shares issued and outstanding as of December 31, 2013 and 2012, respectively; redemption value of \$32,292,269 and \$14,024,356 at December 31, 2013 and 2012, respectively

Stockholders deficit:

Common stock: \$0.01 par value; 19,200,000 shares authorized; 1,725,992 shares issued and outstanding as of December 31, 2013 and 2012

	17,260	17,260
Additional paid-in capital	809,667	1,519,373
Accumulated deficit	(28,978,824)	(21,010,073)
<b>Total stockholders deficit</b>	<b>(28,151,897)</b>	<b>(19,473,440)</b>
<b>TOTAL LIABILITIES AND STOCKHOLDERS DEFICIT</b>	<b>\$ 14,941,638</b>	<b>\$ 5,186,229</b>

The accompanying notes are an integral part of these financial statements.

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Statements of Operations**

	<b>YEAR ENDED DECEMBER 31,</b>	
	<b>2013</b>	<b>2012</b>
<b>REVENUES</b>		
Grants	\$ 1,940,657	\$ 1,470,330
Total revenues	1,940,657	1,470,330
<b>OPERATING EXPENSES</b>		
Research and development	7,049,420	5,796,233
General and administrative	2,813,190	1,943,206
Total operating expenses	9,862,610	7,739,439
<b>LOSS FROM OPERATIONS</b>	(7,921,953)	(6,269,109)
<b>OTHER INCOME (EXPENSE)</b>		
Interest income	3,921	7,545
Interest expense	(50,719)	(1,405)
Total other income (expense)	(46,798)	6,140
<b>NET LOSS</b>	\$ (7,968,751)	\$ (6,262,969)
Preferred stock dividends	(1,093,648)	(757,492)
Net loss attributable to common stockholders	\$ (9,062,399)	\$ (7,020,461)
Net loss per share attributable to common stockholders, basic and diluted	\$ (5.25)	\$ (4.26)
Weighted-average number of common shares outstanding basic and diluted	1,725,992	1,648,198
Pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)	(1.32)	
Weighted-average common shares used to compute pro forma net loss per share attributable to common stockholders, basic and diluted (unaudited)	6,051,619	



The accompanying notes are an integral part of these financial statements.

F-17

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Statements of Redeemable and Convertible Preferred Stock and Stockholders Deficit**

	<b>SERIES A PREFERRED STOCK</b>		<b>SERIES B PREFERRED STOCK</b>		<b>COMMON STOCK</b>		<b>ADDITIONAL PAID-IN CAPITAL</b>	<b>ACCUMULATED DEFICIT</b>	<b>TOTAL STOCKHOLDERS' DEFICIT</b>
	<b>SHARES</b>	<b>AMOUNT</b>	<b>SHARES</b>	<b>AMOUNT</b>	<b>SHARES</b>	<b>AMOUNT</b>			
Balance, January 1, 2012	2,544,539	\$ 7,633,617	2,174,824	\$ 10,144,504	1,357,662	\$ 13,577	\$ 1,984,259	\$(14,747,104)	(12,749,200)
Stock-based compensation							91,503		91,503
Balance of Series B Preferred Stock for cash,			675,105	3,122,360			(56,358)		(56,358)
Anti-dilutive feature for expense recognition					368,330	3,683	257,461		261,144
Retirement of Series B Preferred Stock to redemption				757,492			(757,492)		(757,492)
Stock issued for R&D expense recognition								(6,262,969)	(6,262,969)
Balance, December 31, 2012	2,544,539	\$ 7,633,617	2,849,929	\$ 14,024,356	1,725,992	\$ 17,260	1,519,373	\$(21,010,073)	\$(19,473,400)
Stock-based compensation							390,595		390,595
Conversion of Series B Preferred Stock into Series B Common Stock			757,497	3,503,426					

ferred k ance of es B ferred k for cash,										
		2,955,857	13,670,839				(6,653)			(6,653)
retion of es B ferred k to emption e loss			1,093,648				(1,093,648)			(1,093,648)
								(7,968,751)		(7,968,751)
ance, ember 31, 3	2,544,539	\$ 7,633,617	6,563,283	\$ 32,292,269	1,725,992	\$ 17,260	\$ 809,667	\$ (28,978,824)		\$ (28,151,824)

The accompanying notes are an integral part of these financial statements

**Table of Contents****BELLICUM PHARMACEUTICALS, INC.****Statements of Cash Flows**

	<b>Year Ended December 31</b>	
	<b>2013</b>	<b>2012</b>
<b>CASH FLOWS FROM OPERATING ACTIVITIES</b>		
Net loss	\$ (7,968,751)	\$ (6,262,969)
Adjustments to reconcile net loss to net cash used in operating activities:		
Depreciation expense	587,248	115,282
Stock-based compensation	390,595	91,503
Stock issued for license agreement		261,144
Loss on disposal of property and equipment		3,274
Amortization of lease liability	(95,500)	(3,979)
Interest expense converted into preferred stock	3,426	
Changes in operating assets and liabilities:		
Accounts receivable grants	(745,541)	
Prepaid expenses and other current assets	591,510	(817,597)
Other assets	(286,787)	58,862
Accounts payable	(1,253)	59,016
Accrued payroll	148,999	321,962
Accrued liabilities	549,483	(215,488)
Deferred revenue grants	(1,038,763)	(1,470,329)
Deferred rent	13,630	63,563
Deferred manufacturing costs	239,134	52,133
<b>NET CASH USED IN OPERATING ACTIVITIES</b>	<b>(7,612,570)</b>	<b>(7,743,623)</b>
<b>CASH FLOWS FROM INVESTING ACTIVITIES</b>		
Purchase of property and equipment	(366,338)	(2,047,483)
<b>CASH USED IN INVESTING ACTIVITIES</b>	<b>(366,338)</b>	<b>(2,047,483)</b>
<b>CASH FLOWS FROM FINANCING ACTIVITIES</b>		
Proceeds from issuance of preferred stock	13,670,839	3,122,360
Payment of issuance costs on preferred stock	(6,653)	(56,358)
Proceeds from notes payable	3,500,000	
Proceeds from line of credit	550,223	449,777
Payments on line of credit	(200,000)	
<b>CASH PROVIDED BY FINANCING ACTIVITIES</b>	<b>17,514,409</b>	<b>3,515,779</b>
<b>NET CHANGE IN CASH AND CASH EQUIVALENTS</b>	<b>9,535,501</b>	<b>(6,275,327)</b>
<b>CASH AND CASH EQUIVALENTS AT BEGINNING OF PERIOD</b>	<b>1,632,084</b>	<b>7,907,411</b>

<b>CASH AND CASH EQUIVALENTS AT END OF PERIOD</b>	\$ 11,167,585	\$ 1,632,084
---	---------------	--------------

**SUPPLEMENTAL CASH FLOW INFORMATION**

Cash paid during the period for:

Interest	\$ 47,296	\$ 788
----------	-----------	--------

**NON-CASH INVESTING AND FINANCING ACTIVITIES**

Dividends accrued on preferred stock	\$ 1,093,648	\$ 757,492
--------------------------------------	--------------	------------

Conversion of notes payable into preferred stock	\$ 3,500,000	\$
--	--------------	----

Landlord funded leasehold improvements	\$	\$ 413,940
--	----	------------

The accompanying notes are an integral part of these financial statements.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

**NOTE 1 ORGANIZATION AND BUSINESS DESCRIPTION**

Bellicum Pharmaceuticals, Inc. (the Company or Bellicum), was incorporated in Delaware in July 2004 and is based in Houston, Texas. The Company is a clinical stage biopharmaceutical company focused on discovering and developing novel cellular immunotherapies for various forms of cancer, including both hematological cancers and solid tumors, as well as orphan inherited blood disorders. The Company is devoting substantially all of its present efforts to developing next-generation product candidates in some of the most important areas of cellular immunotherapy, including, hematopoietic stem cell transplantation, CAR T cell therapy and dendritic cell vaccines. The Company has not generated any revenue from product sales to date and does not anticipate generating revenues from product sales in the foreseeable future. The Company's success is dependent on, among other things, its ability to successfully complete the development of, and obtain regulatory approval for, its product candidates, managing the growth of the organization, obtaining additional financing necessary in order launch and commercialize its products candidates, and competing successfully with other companies in its industry.

The future success of the Company is dependent on its ability to develop its product candidates and ultimately upon its ability to attain profitable operations. The Company is subject to a number of risks similar to other life science companies, including, but not limited to, successful discovery and development of its technology, raising additional capital, development by its competitors of new technological innovations, protection of proprietary technology, and market acceptance of the Company's products. As a result of the aforementioned factors and the related uncertainties, there can be no assurance of the Company's future success.

**NOTE 2 SIGNIFICANT ACCOUNTING POLICIES**

***Basis of Presentation***

The accompanying financial statements have been prepared in conformity with accounting principles generally accepted in the United States of America. Any reference in these footnotes to applicable guidance is meant to refer to the authoritative U.S. generally accepted accounting principles (GAAP) as found in the Accounting Standards Codification (ASC) and Accounting Standards Update (ASU) of the Financial Accounting Standards Board (FASB).

***Revenue Recognition***

The Company has not yet generated any revenue from product sales. The Company's sole source of revenue is grant revenue related to a \$5.7 million research grant received from the Cancer Prevention and Research Institute of Texas (CPRIT), covering a three-year period from July 1, 2011 through June 30, 2014, and a \$361,644 research grant from the National Institutes of Health (NIH). Grant payments received prior to the Company's performance of work required by the terms of the research grant are recorded as deferred revenue and recognized as grant revenue once work is performed and qualifying costs are incurred. (See Note 10)

***Cash and Cash Equivalents***

The Company considers all short-term, highly liquid investments with maturity of three months or less to be cash equivalents.

***Property and Equipment***

Leasehold improvements, furniture, equipment and software are recorded at cost and are depreciated using the straight-line method over the estimated useful lives of the related assets, which range from three to five years.

***Impairment of Long-Lived Assets***

The Company reviews long-lived assets for impairment when events or changes in business conditions indicate that their carrying value may not be recoverable. Recoverability of assets to be held and used is measured by a comparison of the carrying amount of an asset to the estimated undiscounted future cash flows expected to be generated by the asset. If the carrying amount of an asset exceeds its estimated future cash flows, an impairment charge is recognized in the amount by which the carrying amount of the asset exceeds the fair value of the asset. There were no impairment charges related to long-lived assets for the years ended December 31, 2013 and 2012.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

***Deferred Rent***

The Company recognizes rent expense for leases with increasing annual rents on a straight-line basis over the term of the lease. The amount of rent expense in excess of cash payments is classified as deferred rent. Any lease incentives received are deferred and amortized over the term of the lease.

***Clinical Trials***

The Company estimates its clinical trial expense accrual for a given period based on the number of patients enrolled at each site and the length of time each patient has been in the trial, less amounts previously billed.

***Fair Value of Financial Instruments***

Accounting standards include disclosure requirements around fair values used for certain financial instruments and establish a fair value hierarchy. The three-tier hierarchy prioritizes valuation inputs into three levels based on the extent to which inputs used in measuring fair value are observable in the market.

Fair value measurements are classified and disclosed in one of the following three categories:

- n Level 1 Quoted unadjusted prices for identical instruments in active markets.
  
- n Level 2 Quoted prices for similar instruments in active markets, quoted prices for identical or similar instruments in markets that are not active and model-derived valuations in which all observable inputs and significant value-drivers are observable in active markets.
  
- n Level 3 Model derived valuations in which one or more significant inputs or significant value-drivers are unobservable, including assumptions developed by the Company.

The Company believes the recorded values of their financial instruments, including cash and cash equivalents, accounts receivable, accounts payable, and accrued liabilities approximate their fair values due to the short-term nature of these instruments. The carrying amount of the line of credit approximates fair value as it bears interest at variable rates.

***Financial Instruments and Credit Risks***

Financial instruments that potentially subject the Company to credit risk include cash and cash equivalents and accounts receivable. Cash is deposited in demand accounts in federally insured domestic institutions to minimize risk.



Insurance is provided through the Federal Deposit Insurance Corporation (FDIC) and Security Investor Protection Corporation (SIPC). Although the balances in these accounts exceed the federally insured limit from time to time, the Company has not incurred losses related to these deposits.

***Debt***

The Company records proceeds from debt issuances at their face value, less any discounts or the value of any beneficial conversion features or detachable warrants. Interest is accrued over the term of the debt, at the stated interest rate. Discounts are amortized to interest expense through the effective interest method over the term of the debt. Unamortized discounts are immediately recognized as interest expense upon conversion or repayment of the debt.

***Equity Issuance Costs***

Equity issuance costs represent costs paid to third parties in order to obtain equity financing. These costs have been netted against the proceeds of the equity issuances.

***Licenses and Patents***

Licenses and patent costs are expensed as incurred. Costs related to the license of patents from third parties and internally developed patents are classified as research and development expenses. Legal costs related to patent applications and maintenance are classified as general and administrative expenses.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

***Research and Development***

Research and development expenses include salaries, related payroll expenses, consulting fees, laboratory costs, manufacturing costs for clinical trials, licenses and clinical trial expenses. All costs for research and development are expensed as incurred.

***Contract Manufacturing Services***

Contract manufacturing services are expensed as incurred. Prepaid costs are capitalized and amortized as services are performed.

***Stock-Based Compensation***

The Company accounts for its stock-based compensation in accordance with ASC 718, *Compensation Stock Compensation*, which requires the measurement and recognition of compensation expense for all stock-based payment awards made to employees and directors to be recognized in the financial statements, based on their fair value. The Company measures stock-based compensation to consultants in accordance with ASC 505-50, *Equity-Based Payments to Non-Employees*, and recognizes the fair value of the award over the period the services are rendered or goods are provided.

The Company uses the Black-Scholes option-pricing model to estimate the fair value of stock option awards. The fair value is recognized as expense, net of estimated forfeitures, over the requisite service period, which is generally the vesting period of the respective award on a straight-line basis. The Company believes that the fair value of stock options granted to non-employee consultants is more reliably measured than the fair value of the services received. The determination of the grant date fair value of options using the Black-Scholes option-pricing model is affected by the Company's estimated common stock fair value, as well as assumptions regarding a number of other complex and subjective variables.

***Income Taxes***

Income taxes are accounted for under the asset and liability method. Deferred tax assets and liabilities are recognized for the future tax consequences attributable to differences between the financial statement carrying amounts of existing assets and liabilities and their respective tax bases. This method also requires the recognition of future tax benefits such as net operating loss and tax credit carry forwards, to the extent that realization of such benefits is more likely than not. A valuation allowance is recorded when the realization of future tax benefits is uncertain. The Company records a valuation allowance for the full amount of deferred tax assets, which would otherwise be recorded for tax benefits relating to the operating loss and tax credit carryforwards, as realization of such deferred tax assets cannot be determined to be more likely than not.

Deferred tax assets and liabilities are measured using enacted tax rates expected to apply to taxable income in the years in which those temporary differences are expected to be recovered or settled. The effect on deferred tax assets and liabilities of a change in tax rates is recognized in the statement of operations in the period that includes the enactment date.

The Company accounts for uncertain tax positions in accordance with the provisions of ASC 740, *Income Taxes*. When uncertain tax positions exist, the Company recognizes the tax benefit of tax positions to the extent that the benefit will more likely than not be realized. The determination as to whether the tax benefit will more likely than not be realized is based upon the technical merits of the tax position as well as consideration of the available facts and circumstances. As of December 31, 2013 and 2012, the Company had no uncertain tax positions and no interest or penalties have been charged to the Company for the years ended December 31, 2013 and 2012. If incurred, the Company will classify any interest and penalties as a component of interest expense and operating expense, respectively. The Company is subject to routine audits by taxing jurisdictions; however, there are currently no audits for any tax periods in progress. The tax years 2004 through 2013 remain open to examination by the Internal Revenue Service.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

***Comprehensive Income (Loss)***

Comprehensive income (loss) is defined as the change in equity of a business enterprise during a period, from transactions, and other events and circumstances from non-owner sources. For the years ended December 31, 2013 and 2012, net loss equaled comprehensive loss.

***Use of Estimates***

The preparation of the financial statements in accordance with GAAP requires management to make certain estimates and judgments that affect the reported amounts of assets, liabilities, and expenses. Actual results could differ from those estimates.

Historically, the fair values of the shares of common stock underlying the Company's share-based awards were estimated on each grant date by its Board of Directors. Given the absence of a public trading market for the Company's common stock, its Board of Directors exercised reasonable judgment and considered a number of objective and subjective factors to determine the best estimate of the fair value of its common stock, including the following:

- n its stage of development;
- n its operational and financial performance;
- n the nature of its services and its competitive position in the marketplace;
- n the value of companies that it considers peers based on a number of factors, including similarity to the Company with respect to industry and business model;
- n the likelihood of achieving a liquidity event, such as an initial public offering and the nature and history of its business;
- n issuances of preferred stock and the rights, preferences, and privileges of its preferred stock relative to those of its common stock;

- n business conditions and projections;
- n the history of the Company and progress of its research and development efforts and clinical trials; and
- n the lack of marketability of its common stock.

***Net Loss and Unaudited Pro Forma Net Loss per Share of Common Stock Attributable to Common Stockholders***

Basic net loss per share attributable to common stockholders is calculated by dividing the net loss attributable to common stockholders by the weighted-average number of shares of common stock outstanding during the period without consideration for common stock equivalents. Diluted net loss per share of common stock is the same as basic net loss per share of common stock, since the effects of potentially dilutive securities are antidilutive. The net loss per share of common stock attributable to common stockholders is computed using the two-class method required for participating securities. All series of the Company's convertible preferred stock are considered to be participating securities as they are entitled to participate in undistributed earnings with shares of common stock. Due to the Company's net loss, there is no impact on the earnings per share calculation in applying the two-class method since the participating securities have no legal requirement to share in any losses.

The unaudited pro forma loss per share attributable to common stockholders for the years ended December 31, 2013 was computed using the weighted-average number of shares of common stock outstanding, including the pro forma effect of the conversion of all then-outstanding shares of redeemable convertible preferred stock into shares of common stock, and the effect of the exercise of certain then-outstanding warrants that will terminate if not exercised upon the IPO, and excluding the pro forma effect of the common stock issuance for payment of the accrued Series B dividends. For the purpose of the pro forma presentation, the Company has assumed the net exercise of common warrants that will terminate if not exercised unless the warrants require cash exercise.

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

The following outstanding shares of common stock equivalents were excluded from the computations of diluted net loss per shares of common stock attributable to common stockholders for the periods presented as the effect of including such securities would be anti-dilutive:

	<b>2013</b>	<b>2012</b>
Series A Convertible preferred stock as converted to common stock	1,496,782	1,496,782
Series B Convertible preferred stock as converted to common stock	3,860,754	1,676,428
Warrants to purchase common stock	866,570	866,570
Options to purchase common stock	1,574,398	1,067,058
Stock dividends to be issued as payment for Series B accrued dividends	101,951	44,393
	7,900,455	5,151,231

The following table sets forth the computation of unaudited pro forma basic and diluted net loss per share attributable to common stockholders, excluding the issuance of shares of common stock as payment of accrued dividends payable to the holders of Series B convertible preferred stock:

	<b>YEAR ENDED DECEMBER 31, 2013</b>
Net loss used in computing net loss per share attributable to common stockholders, basic and diluted	\$ (7,968,751)
Pro forma net loss used in computing net loss per share attributable to common stockholders, basic and diluted	\$ (7,968,751)
Weighted-average common shares used to compute net loss per share attributable to common stockholders, basic and diluted	1,725,992

Pro forma adjustment to reflect assumed conversion of convertible preferred stock	3,816,306
Pro forma adjustment to reflect assumed conversion of warrants	509,321
Weighted-average common shares used to compute pro forma net loss per share attributable to common stockholders, basic and diluted	6,051,619
Pro forma net loss per share of common stock, basic and diluted	(1.32)

### ***Recently Issued Accounting Pronouncements***

In June 2014, the Financial Accounting Standards Board issued Accounting Standards Update No. 2014-10 (ASU No. 2014-10), which eliminated the definition of a Development Stage Entity and the related reporting requirements. ASU No. 2014-10 is effective for annual reporting periods beginning after December 15, 2014, with early adoption allowed. The Company early adopted ASU No. 2014-10, effective in its financial statements for the years ended December 31, 2013 and 2012.

The Company has evaluated other recent accounting pronouncements and believes that none of them will have a material effect on the Company's financial statements.

### **NOTE 3 CASH AND CASH EQUIVALENTS**

As of December 31, 2013 and 2012, the Company invested approximately \$10.9 million and \$1.6 million, respectively.

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

**NOTE 4 FAIR VALUE OF FINANCIAL INSTRUMENTS**

ASC 820, *Fair Value Measurement*, provides a comprehensive framework for measuring the fair value of assets and liabilities, which provides for consistency in how fair value determinations are made under various existing accounting standards that permit, or in some cases require, estimates of fair market value.

Financial assets and liabilities that have recurring fair value measurements are shown below:

	<b>BALANCE AT DECEMBER 31, 2013</b>	<b>QUOTED PRICES IN ACTIVE MARKETS FOR IDENTICAL ASSETS (LEVEL 1)</b>	<b>INSIGNIFICANT OTHER OBSERVABLE INPUTS (LEVEL 2)</b>	<b>SIGNIFICANT UNOBSERVABLE INPUTS (LEVEL 3)</b>
<b>Assets:</b>				
Money market funds	\$ 10,879,656	\$ 10,879,656	\$	\$
<b>Total</b>	<b>\$ 10,879,656</b>	<b>\$ 10,879,656</b>	<b>\$</b>	<b>\$</b>

	<b>BALANCE AT DECEMBER 31, 2012</b>	<b>QUOTED PRICES IN ACTIVE MARKETS FOR IDENTICAL ASSETS (LEVEL 1)</b>	<b>SIGNIFICANT OTHER OBSERVABLE INPUTS (LEVEL 2)</b>	<b>SIGNIFICANT UNOBSERVABLE INPUTS (LEVEL 3)</b>
<b>Assets:</b>				
Money market funds	\$ 1,632,084	\$ 1,632,084	\$	\$



Total	\$	1,632,084	\$	1,632,084	\$	\$
-------	----	-----------	----	-----------	----	----

**NOTE 5 PROPERTY AND EQUIPMENT**

Property and equipment consists of the following:

DESCRIPTION	ESTIMATED USEFUL LIVES	DECEMBER 31,	
		2013	2012
Lab equipment	5 years	\$ 1,133,305	\$ 802,136
Office furniture	5 years	326,595	326,595
Software	3 years	47,890	43,678
Computer equipment	3 to 5 years	151,182	142,056
Leasehold improvements	5 years	1,375,001	1,353,170
Total		3,033,973	2,667,635
Less: accumulated depreciation		(744,054)	(156,806)
		\$ 2,289,919	\$ 2,510,829

During the years ended December 31, 2013 and 2012, the Company recorded \$587,248 and \$115,282 of depreciation expense, respectively.

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

**NOTE 6 ACCRUED LIABILITIES**

Accrued liabilities consist of the following:

	<b>DECEMBER 31,</b>	
	<b>2013</b>	<b>2012</b>
Medical facility fees	\$ 224,166	\$
Patient treatment costs	197,713	
License costs	175,000	17,500
Other	67,294	97,190
<b>Total accrued liabilities</b>	<b>\$ 664,173</b>	<b>\$ 114,690</b>

**NOTE 7 DEBT*****Promissory Notes 2013***

On February 12, 2013, the Company received \$3,500,000 of cash proceeds through the issuance of promissory notes, bearing interest at 0.21% per annum from February 12, 2013 through July 31, 2013. On July 31, 2013, in connection with the issuance of Series B Preferred Stock, the Company repaid the notes with 757,497 shares of Series B preferred convertible redeemable stock at a conversion price of \$4.625 per share. The repaid balance consisted of \$3,500,000 of principal and \$3,426 of outstanding interest payable.

***Line of Credit***

In December 2012, the Company entered into a line of credit agreement with Comerica Bank for up to \$1 million. The annual interest rate is equal to the prime rate plus 2.75%. At December 31, 2013, the interest rate was 6%. Interest accrues from the date of each advance and is due and payable on the first business day of each month. Any principal advances that are outstanding on the line of credit are payable in 30 equal monthly installments of principal beginning on July 1, 2013. Substantially all of the Company's assets, excluding intellectual property, have been pledged to secure

the note. The balance drawn on the line of credit was \$800,000 and \$449,777 at December 31, 2013 and 2012, respectively.

The future principal payments are as follows:

Year:	
2014	\$ 400,000
2015	400,000
Total principal payments	\$ 800,000

#### **NOTE 8 DEFERRED MANUFACTURING COSTS**

On December 5, 2011, the Company entered into a service agreement with a third party to perform manufacturing processes for the Company's products. The agreement contained the following terms: (i) an initial non-refundable commitment fee of \$973,330; (ii) a monthly fee of \$91,200 which will be deducted from the initial commitment fee until it is fully depleted; (iii) a minimum of 110 batches at a cost of \$3,400 per batch plus the cost of materials to be manufactured over a minimum of 18 months, commencing October 2012.

The Company recorded the commitment fee as prepaid manufacturing costs and expensed the monthly fee as research and development costs to offset against the prepaid manufacturing costs. As of December 31, 2013 and 2012, the prepaid manufacturing costs were \$- and \$699,730, respectively.

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

The minimum 110 batches represent the minimum quantity that the Company has to procure. If the Company elects to terminate the agreement before 110 batches are produced, the Company must pay \$3,400 per batch for the production shortfall. The Company initially expected to fulfill this requirement in 18 months, commencing October 2012.

Accordingly, the Company accrued the value of the 110 batches ratably over the 18 months as deferred manufacturing costs. As of December 31, 2013 and 2012, the deferred manufacturing costs were \$291,267 and \$52,133, respectively. Subsequently in 2014, the Company revised its estimate of the expected term of the agreement to extend an additional 21 months.

Additionally, the third party manufacturer agreed to credit Bellicum \$270,000 against its monthly fees beginning April 1, 2014. This credit would be forfeited if the agreement were terminated prior to April 1, 2014. This credit was applied to the monthly fee beginning April 1, 2014 at a rate of \$3,000 per day. The Company is recognizing the credit ratably over the remaining expected term of the agreement as a reduction of research and development expenses commencing April 2014.

**NOTE 9 EQUITY**

As of December 31, 2013 and 2012, the Company had 19,200,000 authorized shares of common stock with a par value of \$0.01 per share and 11,700,000 authorized shares of convertible redeemable preferred stock with a par value of \$0.01 per share.

***Common Stock***

In 2012, the Company issued ARIAD Pharmaceuticals, Inc. (ARIAD) an aggregate of 368,330 shares of the Company's common stock for a total purchase price of \$1.00, concurrently with the issuances by the Company to investors of Series B preferred stock. This common stock issued to ARIAD was valued at \$261,144. The issuance fully satisfied the rights of ARIAD to receive additional shares of the Company's common stock pursuant to its anti-dilution rights (see Note 11).

***Preferred Stock***

The Company had shares of two classes of convertible redeemable preferred stock issued and outstanding as of December 31, 2013 and 2012: Series A convertible redeemable preferred stock (Series A) and Series B convertible redeemable preferred stock (Series B) (collectively, Preferred Stock), each with a par value of \$0.01. The shares of Series A were issued between March 2009 and November 2011 at a price of \$3.00 per share. The shares of Series B were issued between November 2011 and November 2013 at a price of \$4.625 per share.

On March 7, 2012, the Company issued 675,105 shares of Series B for cash proceeds of \$3,066,002, or \$4.625 per share, net of issuance costs.

On July 31, 2013, the Company issued 2,098,219 shares of Series B for aggregate gross proceeds of approximately \$9.7 million, or \$4.625 per share, comprised of 757,497 shares issued upon the cancellation of \$3,500,000 of principal and \$3,426 accrued and unpaid interest on promissory notes, and 1,340,722 shares issued for cash proceeds of approximately \$6.2 million.

On November 15, 2013, the Company issued 1,615,135 shares of Series B for net cash proceeds of approximately \$7.5 million, or \$4.625 per share.

As of December 31, 2013, the Company's total outstanding convertible preferred stock was as follows:

	<b>PREFERRED SHARES</b>		<b>REDEMPTION VALUE</b>	
	<b>ISSUED AND OUTSTANDING</b>	<b>INITIAL VALUE</b>	<b>DECEMBER, 31, 2013</b>	<b>DECEMBER 31, 2012</b>
<b>Series A</b>	2,544,539	\$ 7,633,617	\$ 7,633,617	\$ 7,633,617
<b>Series B</b>	6,563,283	\$ 30,355,184	\$ 32,292,269	\$ 14,024,356

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

The rights, preferences and privileges of the Preferred Stock, as of December 31, 2013 and 2012, are as follows:

*Optional Conversion*

Each share of Series A and Series B is convertible, at the option of the holder at any time and without additional consideration, into one shares of common stock. The Series A conversion price is \$3.00 and the Series B conversion price is \$4.625. The rate at which shares of Preferred Stock may be converted into shares of common stock, is subject to anti-dilution protection in the event of certain dilutive issuances of capital stock.

*Mandatory Conversion*

Upon the closing of the sale of shares the Company's common stock in an IPO resulting in at least \$25.0 million of gross proceeds to the Company, all of the outstanding shares of Preferred Stock will automatically convert into shares of the Company's common stock, at the then-applicable conversion rate, and such shares may not be reissued by the Company.

*Dividends*

At December 31, 2013 and 2012, the holders of Series B were entitled to receive an annual dividend, payable quarterly, if, as and when declared, and if not paid, accrued, equal to 6% of the Series B original issue price, subject to appropriate adjustment in the event of any stock dividend, stock split, combination or other similar recapitalization with respect to Series B. This dividend is cumulative, but not compounded. No dividends or other distributions can be declared or paid with respect to the Series A or common stock, other than dividends payable solely in common stock, unless and until all dividends due on Series B have been paid or declared and set apart for payment. No dividends have been declared or paid since inception. Cumulative dividends are \$1,937,085 and \$843,437 at December 31, 2013 and 2012, respectively.

*Liquidation*

At December 31, 2013 and 2012, in the event of any deemed liquidation event, which is defined in the Company's certificate of incorporation to include (1) a merger or consolidation of the Company in which the Company or a subsidiary is a constituent party (subject to certain exceptions), (2) the sale, lease, transfer, exclusive license or other disposition of all or substantially all of the Company's assets, or (3) the sale of outstanding shares of the capital stock of the Company representing at least 50% of the outstanding capital stock or at least 50% of the voting power of the outstanding capital stock (subject to certain exceptions), or any voluntary or involuntary liquidation, dissolution, or winding up of the Company, the holders of Series B then outstanding were entitled to be paid out of the assets of the Company available for distribution to its stockholders, before any payment shall be made to the holders of Series A or common stock, an amount per share equal to the original issue price of the Series B, plus any dividends accrued or declared but unpaid thereon (Liquidation Amount).

After payment to the holders of Series B of the full amounts due them, the holders of Series A were entitled to be paid out of the remaining assets of the Company available for distribution to its stockholders, before any payment shall be made to the holders of common stock, in an amount per share equal to the original issue price of the Series A, plus any dividends declared but unpaid thereon.

After payment of the preferential amounts discussed above, the holders of shares of Series A and Series B, the remaining assets of the Company available for distribution to its stockholders shall be distributed among the holders of the shares of common stock and Preferred Stock, pro rata on an as-converted basis based on the number of shares held by each holder.

*Redemption*

At December 31, 2013 and 2012, at any time following the seventh anniversary of the original issue date of the Series B, the Company was obligated to redeem all of the outstanding shares of Series B, if requested in writing to do by the holders of not less than 51% of the outstanding shares of Series B. At any time following the seventh anniversary of the original issue date of the Series A, the Company was obligated to redeem all of the outstanding shares of Series A, if requested in writing to do so by the holders of not less than 51% of the outstanding shares of

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

Series A. No redemption of any of the shares of Series A could occur until such a time as no shares of Series B were outstanding. The Company evaluated the redemption feature of the Preferred Stock under ASC 480, *Distinguishing Liabilities from Equity*, and determined that Series A and Series B were contingently redeemable and hence are classified as temporary equity. For the years ended December 31, 2013 and 2012, the Company accreted \$1,093,648 and \$757,492, respectively, to the redemption value. The redemption value is based on the original issue price plus cumulative dividends at each reporting date.

*Voting*

At December 31, 2013 and 2012, on any matter presented to the stockholders of the Company, each holder of outstanding shares of Preferred Stock was entitled to cast the number of votes equal to the number of whole shares of common stock into which the shares of Preferred Stock held by the holder were convertible. Except as provided by law or by the other provisions of the Company's Certificate of Incorporation, holders of Preferred Stock vote together with the holders of common stock as a single class.

***Stock Option Plans***

The Company has two stock-based compensation plans, which authorize the granting of shares of common stock to employees and directors of the Company, as well as non-employee consultants, and allows the holder of the option to purchase common stock at a stated exercise price. Options vest according to the terms of the grant, which may be immediately or based on the passage of time, generally over four years, and have a term of up to 10 years. Unexercised stock options terminate on the expiration date of the grant. The Company recognizes the share-based compensation expense over the requisite service period of the individual grantees, which generally equals the vesting period.

***2011 Stock Option Plan***

Under the 2011 Stock Option Plan, 2,798,500 shares of the Company's authorized but unissued common stock were reserved for issuance to employees, consultants, or to non-employee members of the Board of Directors. The 2011 Stock Option Plan replaced the Company's previous stock option plan (the 2006 Stock Option Plan).

***2006 Stock Option Plan***

Under the 2006 Stock Option Plan, as amended, 301,500 shares of the Company's authorized but unissued common stock were reserved for issuance to optionees, including officers, employees, and other individuals performing services for the Company. As of December 31, 2013, there were no additional shares available for grant under the 2006 Stock Option Plan. A total of 167,058 options were outstanding under this plan as of December 31, 2013 and 2012.





Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

The valuation of the stock-based compensation awards is a significant accounting estimate that requires the use of judgment and assumptions that are likely to have a material impact on the financial statements. The fair value of option grants is determined using the Black-Scholes option-pricing model. Expected volatilities utilized in the model are based on implied volatilities from traded stocks of peer companies. Similarly, the dividend yield is based on historical experience and the estimate of future dividend yields. The risk-free interest rate is derived from the U.S. Treasury yield curve in effect at the time of grant. The expected term of the options is based on the average period the stock options are expected to remain outstanding. As the Company does not have sufficient historical information to develop reasonable expectations about future exercise patterns and post-vesting employment termination behavior, the expected term is calculated as the midpoint between the weighted-average vesting term and the contractual expiration period also known as the simplified method. The Company assumed no awards would be forfeited during the vesting period, as actual forfeitures have been minimal through December 31, 2013. The fair value of the option grants have been estimated, with the following weighted-average assumptions:

	<b>YEAR ENDED DECEMBER 31</b>	
	<b>2013</b>	<b>2012</b>
Volatility	90%	75%
Risk-free interest rate	1.58%	1.97%
Expected dividend yield	0%	0%
Expected life	6.25 years	6.25 years

A summary of the status of the Company's stock option plans as of December 31, 2013 and changes from December 31, 2011 through December 31, 2013 is as follows:

OPTIONS	WEIGHTED- AVERAGE EXERCISE	WEIGHTED-AVERAGE REMAINING
---------	-------------------------------	-------------------------------

		<b>PRICE</b>	<b>CONTRACTUAL TERM</b>
<b>Options outstanding, December 31, 2011</b>	<b>1,015,588</b>	<b>\$ 2.21</b>	<b>9.69</b>
Options granted	60,293	\$ 2.55	
Options exercised			
Options forfeited	(8,823)	\$ 2.55	
<b>Options outstanding, December 31, 2012</b>	<b>1,067,058</b>	<b>\$ 2.23</b>	<b>8.71</b>
Options granted	516,163	\$ 2.55	
Options exercised			
Options forfeited	(8,823)	\$ 2.55	
<b>Options outstanding, December 31, 2013</b>	<b>1,574,398</b>	<b>\$ 2.33</b>	<b>8.26</b>
<b>Exercisable at December 31, 2013</b>	<b>818,680</b>	<b>\$ 2.19</b>	<b>7.98</b>

The weighted average grant date fair value of options granted in each of the years ended December 31, 2013 and 2012 was \$1.38 and \$0.31, respectively.

The Company calculates the intrinsic value of its options by multiplying the number of options by the difference between the estimated fair value per share for its common stock and the options exercise price. The aggregate intrinsic value of options exercisable and options outstanding at December 31, 2013 was \$234,530 and \$273,640, respectively. The Company will issue new shares of common stock upon the exercise of vested options.

**Table of Contents****BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

The following table outlines the options outstanding and exercisable as of December 31, 2013:

<b>INSTRUMENT TYPE</b>	<b>EXERCISE PRICE</b>	<b>OUTSTANDING</b>	<b>REMAINING CONTRACTUAL LIFE IN YEARS</b>
Option	\$ 0.34	5,882	2.76
Option	\$ 0.51	161,176	6.89
Option	\$ 2.55	1,407,340	8.44
Total	\$ 2.33	1,574,398	8.26

<b>INSTRUMENT TYPE</b>	<b>EXERCISE PRICE</b>	<b>EXERCISABLE</b>	<b>REMAINING CONTRACTUAL LIFE IN YEARS</b>
Option	\$ 0.34	5,882	2.76
Option	\$ 0.51	137,212	6.89
Option	\$ 2.55	675,586	8.25
Total	\$ 2.19	818,680	7.98

Stock-based compensation expense for the year ended December 31, 2013 was \$390,595. At December 31, 2013, total compensation cost not yet recognized was \$517,746 and the weighted average period over which this amount is expected to be recognized is 2.16 years.

The following table outlines the options outstanding and exercisable as of December 31, 2012:

<b>INSTRUMENT TYPE</b>	<b>EXERCISE PRICE</b>	<b>OUTSTANDING</b>	<b>REMAINING CONTRACTUAL LIFE IN YEARS</b>
Option	\$ 0.34	5,882	3.76
Option	\$ 0.51	161,176	7.89
Option	\$ 2.55	900,000	8.89
Total	\$ 2.23	1,067,058	8.71

<b>INSTRUMENT TYPE</b>	<b>EXERCISE PRICE</b>	<b>EXERCISABLE</b>	<b>REMAINING CONTRACTUAL LIFE IN YEARS</b>
Option	\$ 0.34	5,882	3.76
Option	\$ 0.51	109,565	7.90
Option	\$ 2.55	381,023	8.88
Total	\$ 2.07	496,470	8.60

Stock-based compensation expense for the year ended December 31, 2012 was \$91,503.



**Table of Contents****BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

**Warrants to Purchase Common Stock 2009 and 2010 Notes Payable**

The following table outlines the warrants outstanding and exercisable as of December 31, 2013 and 2012:

<b>ISSUANCE DATE</b>	<b>EXERCISE</b>		<b>EXERCISABLE</b>	<b>EXPIRATION DATE</b>
	<b>PRICE</b>	<b>OUTSTANDING</b>		
March 2009	\$0.51	393,529	393,529	March 25, 2014
September 2010	\$0.51	46,078	46,078	September 30, 2015
December 2010	\$0.51	71,570	71,570	December 16, 2015

The Company determined that the fair value of warrants granted in connection with certain prior debt agreements was approximately \$418,782, and allocated that portion of the total proceeds of the debt as issuance debt discount at issuance date.

**Warrant to Purchase Common Stock Texas Emerging Technology Fund**

The Texas Emerging Technology Fund (TETF) holds a warrant to purchase up to 355,392 shares of the Company's common stock at an exercise price of \$.0017 per share. The warrant was issued in conjunction with a research grant to the Company by TETF in 2007. This warrant remains outstanding and exercisable indefinitely.

**NOTE 10 GRANT REVENUE*****CPRIT Grant***

On July 27, 2011, the Company entered into a Cancer Research Grant Contract (Grant Contract) with CPRIT under which CPRIT awarded a grant not to exceed approximately \$5.7 million to be used by the Company for the execution of defined clinical development of BPX-501. In addition, CPRIT could award supplemental funding not to exceed 10% of the total grant amount based upon the Company's progress. The Grant Contract terminated on June 30, 2014. The terms of the Grant Contract require the Company to pay tiered royalties on revenues from sales and licenses of intellectual property facilitated by the Grant Contract.

During 2013 and 2012, the Company incurred \$1.8 million and \$1.5 million of expenses under the Grant Contract, respectively. As of December 31, 2012, the Company had an outstanding deferred liability of \$1,038,763 as a result of funds being received in advance of the related grant expenditure being paid. However, during 2013, the remaining deferred liability was fully recognized and at December 31, 2013, the Company had an outstanding grant receivable of \$716,606 for grant expenditures that were paid but have not been reimbursed.

***NIH Grant***

On March 25, 2013, the Company was awarded \$361,644 under a grant from NIH. The award covers the period from April 2013 through March 2014. The award was made pursuant to the authority of 42 USC 241 42 CFR 52, and is subject to the requirements of the statute. Funds spent on the grant are reimbursed through monthly reimbursement requests.

As of December 31, 2013, \$185,289 of such funds have been spent under the grant, of which, \$156,354 in funds have been received and \$28,935 are held as receivables due under the grant.

**NOTE 11 COMMITMENTS AND CONTINGENCIES**

***Leases***

The Company has entered into various short-term leases for office space. The Company incurred rent expense during the years ended December 31, 2013 and 2012 of \$226,779 and \$217,171, respectively.

In December 2012, the Company entered into a five-year office lease agreement. During 2013, the lease was amended to include additional space. The leased premises totals 14,255 square feet. The lease includes escalating



Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

base rent payments, which initially increased on November 1, 2013, and then increased again on December 1, 2013. Subsequently, an increase in the base rent payment will occur during the first month of each year, and remain constant for the next 11 months. During the last month of each year, the monthly base rent payment will increase yet again. This escalating base rent payment structure will continue through the expiration of the lease on December 16, 2017. The future minimum payments under the lease are as follows:

Year:	
2014	\$ 410,119
2015	417,246
2016	424,374
2017	413,825
Total minimum lease payments	\$ 1,665,564

***Litigation***

The Company, from time to time, may be involved in litigation relating to claims arising out of its ordinary course of business. Management believes that there are no material claims or actions pending or threatened against the Company.

***License Agreement with ARIAD Pharmaceuticals, Inc.***

On March 7, 2011, the Company entered into an amended and restated exclusive license agreement with ARIAD (Amended ARIAD License) which amended a license agreement entered into by the parties in 2006. Under the Amended ARIAD License, ARIAD granted to the Company an exclusive (even as to the ARIAD) license, with the right to grant sublicenses, under ARIAD's patent rights relating to dimerizers, genetic constructs coding for dimerizer binding domains, vectors containing said constructs, cells containing said constructs and methods of inducing biological processes in cells containing said constructs. These licensed patent rights were initially limited to the fields of cell transplantation and certain types of cancer.

In connection with the initial license, in 2006, the Company issued 121,242 shares of its common stock to ARIAD which were subject to anti-dilution protection that ultimately resulted in additional issuances to ARIAD by the Company of 556,221 shares of the Company's common stock, such that ARIAD received a total of 677,463 shares of common stock under the license agreement. In addition, the Company paid ARIAD a license fee of \$250,000 in connection with the amendment in 2011. The Amended ARIAD license also provided for certain royalty and milestone payments, which were subsequently terminated pursuant to an omnibus amendment agreement with ARIAD (see Note 14). The issuance of the shares in connection with the initial license and the shares issued in connection with the anti-dilution provision were recorded as research and development expense in the period issued based on the fair value of the common stock on that date.

Under the Amended ARIAD License, the Company is required to diligently proceed with the development, manufacture and sale of licensed products. The Amended ARIAD License is subject at all times to restrictions and obligations under a license agreement by and between ARIAD Gene Therapeutics, Inc. (one of ARIAD's affiliates which merged into ARIAD) and the academic institution from which ARIAD obtained its license to the underlying technology. While the Company is not required to pay royalties or fees to such academic institution, no sublicensee of the Company's may enter into a sublicense with respect to any intellectual property owned by the academic institution without its consent, which terms must be consistent with those included in the agreement between ARIAD and such academic institution.

The Amended ARIAD License will expire upon expiration of the last license term of a licensed product covered by the agreement, which is either the later of (1) 12 years from the date of the first commercial sale of the licensed

**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

product, or (2) expiration of a valid claim on the licensed product. Either party to the license may terminate or modify the Amended ARIAD License upon a material breach by the other party that remains uncured following the date that is 30 days after written notice of a payment breach and 90 days for any other breach, and effective immediately upon bankruptcy of the other party. The Company may terminate the amended ARIAD license in its sole discretion at any time if the Company determines not to develop or commercialize any licensed product. In addition, upon termination of the amended ARIAD license prior to expiration, the Company must transfer any ownership and any beneficial ownership in any orphan drug designation or any similar designation in any jurisdiction of orphan drug status of the ARIAD dimerizer to ARIAD.

***License Agreements with Baylor College of Medicine***

***2008 Baylor License Agreement***

Pursuant to an Exclusive License Agreement with Baylor College of Medicine (Baylor), dated March 20, 2008 (2008 Baylor License), the Company obtained an exclusive, worldwide and fully paid up license to certain intellectual property, including intellectual property related to methods for activating antigen presenting cells and to genetic constructs coding for membrane bound inducible cytoplasmic CD40.

As consideration for the 2008 Baylor License, the Company issued to Baylor 23,529 shares of its common stock and assumed responsibility for all legal fees and expenses, filing or maintenance fees, assessments and all other costs and expenses related to prosecuting, obtaining and maintaining patent protection on the patents subject to the 2008 Baylor License.

The 2008 Baylor License is subject to certain restrictions and is non-exclusive with respect to (1) the making or use of the licensed intellectual property for use in non-commercial research, patient care, teaching, and other educational purposes; (2) any non-exclusive license covering the licensed intellectual property that Baylor grants to other academic or research institutions for non-commercial research purposes; (3) any non-exclusive licenses that Baylor is required to grant to the U.S. or foreign state pursuant to an existing or future treaty with the U.S., and (4) a non-exclusive license granted to ARIAD under the terms of a materials transfer agreement between Baylor and ARIAD.

Baylor may terminate or modify the 2008 Baylor License in the event of a material breach that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. The Company may terminate the 2008 Baylor License, or any portion thereof, at its sole discretion at any time upon 30 days written notice to Baylor. Upon termination of the 2008 Baylor License, all rights to the intellectual property immediately revert to Baylor.

***2010 Baylor License Agreement***

Pursuant to an Exclusive License Agreement with Baylor, dated June 27, 2010 (2010 Baylor License), the Company obtained an exclusive, worldwide license to certain intellectual property, including intellectual property related to methods for treating prostate cancer, methods of administering T cells to a patient, and methods of activating antigen presenting cells with constructs comprising MyD88 and CD40.

Pursuant to the terms of the 2010 Baylor License, the Company paid Baylor a license execution fee of \$30,000. In addition, the Company is required to pay a low annual maintenance fee on beginning on the second anniversary of the agreement date.

The terms of the 2010 Baylor License also require the Company to make royalty payments of less than 1%, subject to certain annual minimums, on net sales of products covered by the license. In addition, to the extent the Company enters into a sublicensing agreement relating to a licensed product, the Company is required to pay Baylor a percentage in the mid-single digits on all non-royalty income received from sublicensing revenue. The Company is required to make milestone payments, of up to \$735,000 in aggregate, upon successful completion of clinical and regulatory milestones regarding the first two product covered by this license.

**Table of Contents****BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

The 2010 Baylor License will expire upon expiration of the last patent contained in the licensed patent rights, on a country-by-country basis, upon which the Company will have a perpetual, paid-in-full license in such country. Baylor may terminate or modify the 2010 Baylor License in the event of a material breach by the Company that remains uncured following the date that is 90 days after written notice of such breach or upon certain insolvency events that remain uncured following the date that is 30 days following written notice of such insolvency event. The Company may terminate the 2010 Baylor License, or any portion thereof, at the Company's sole discretion at any time upon 60 days' written notice to Baylor. Upon termination of the 2010 Baylor License for any reason prior to expiration, the Company must assign to Baylor each authorized sublicense agreement that is currently in effect on the date of termination.

**NOTE 12 RELATED PARTY TRANSACTIONS*****Related Party Licensing Arrangements***

During 2010, the Company entered into a license agreement with Baylor, granting the Company the rights to patents and technology derived from invention disclosure BLG 06-028. The license included \$30,000 of upfront payments during 2010 and 2011, and additional annual fees of \$7,500 beginning on the second anniversary of the license agreement.

During 2008, Bellicum entered into a license agreement with Baylor, granting the Company the rights to patents and technology derived from invention disclosures OTA 01-085 and BLG 08-024. Consideration for the license included issuance in 2009 of 13,529 common shares to Baylor and 10,000 common shares to the inventors (including board members Dr. David Spencer and Dr. Kevin Slawin).

On March 1, 2006, the Company issued 61,764 common shares to Dr. David Spencer, Dr. Brent Hanks and Dr. Kevin Slawin, valued at \$0.34 per share, as consideration for the use of certain patents. During 2006, the Company issued 121,242 common shares to ARIAD as consideration for the licensed rights to use certain patents held by ARIAD. The stock purchase agreement included certain anti-dilutive features which resulted in the issuance of the following additional shares:

	<b>ANTI-DILUTIVE SHARES ISSUED</b>
<b>Year:</b>	
2009	187,891

2012

368,330

**NOTE 13 INCOME TAXES**

The Company did not recognize tax expense during 2013 or 2012. The reconciliation between federal income taxes at the statutory rate and the Company's income tax expense for the year is as follows:

	<b>YEAR ENDED DECEMBER 31</b>	
	<b>2013</b>	<b>2012</b>
U.S. tax benefit at statutory rate	\$ (2,709,375)	\$ (2,129,409)
Meals and entertainment	3,721	3,711
Incentive stock option	115,020	13,329
Research and development credit	(436,879)	
Deferred tax valuation allowances	3,027,513	2,112,369
Income tax expense	\$	\$

F-35

Table of Contents**BELLICUM PHARMACEUTICALS, INC.****Notes to the Financial Statements**

December 31, 2013 and 2012

Deferred income taxes reflect the net tax effect of temporary differences between the carrying amounts of assets and liabilities for financial reporting purposes, and the amounts used for income tax purposes. Significant components of the Company's deferred taxes as of December 31, 2013 and 2012 are as follows:

	<b>2013</b>	<b>2012</b>
Deferred tax liabilities:		
Depreciation	\$ (194,887)	\$ (229,075)
Prepaid costs	(123,486)	(331,408)
Tenant improvement allowance	(111,419)	(139,567)
Deferred rent		(4,095)
Total deferred tax liabilities	(429,792)	(704,145)
Deferred tax assets:		
Net operating loss carry forward	9,141,814	6,896,802
Non-qualified stock options	71,761	53,979
Tenant improvement liability	128,528	160,998
Deferred contract manufacturing costs	99,030	17,725
Research and development credit	826,161	389,282
Other	4,652	
Total deferred tax assets	10,271,946	7,518,786
Valuation allowance	(9,842,154)	(6,814,641)
Total deferred tax	\$	\$
Net current deferred tax liability	\$ (64,000)	\$ (273,000)
Net non-current deferred tax asset	64,000	273,000
Total deferred tax	\$	\$

As of December 31, 2013, the Company had gross federal income tax net operating loss carry forwards of \$26,887,686 and federal research tax credits of \$826,161. The net operating loss carry forwards will expire beginning in 2024, if not utilized. In assessing the realization of deferred tax assets, management considers whether it is more likely than not that some portion or all of the deferred tax assets will be realized. The ultimate realization of deferred tax assets is dependent upon the Company attaining future taxable income during periods in which those temporary differences become deductible. Due to the uncertainty surrounding the realization of the benefits of its deferred assets, including net operating loss carry forwards, the Company has provided a 100% valuation allowance on its deferred tax assets at December 31, 2013 and 2012. The Internal Revenue Code Section 382 limits net operating loss and tax credit carry forwards when an ownership change of more than 50% of the value of the stock in a loss corporation occurs. Accordingly, the ability to utilize remaining net operating loss and tax credit carry forwards may be significantly restricted.

#### **NOTE 14 REVERSE STOCK SPLIT**

On December 4, 2014 and December 5, 2014, respectively, our board of directors and our stockholders approved an amendment to our amended and restated certificate of incorporation to effect a reverse split of shares of our common stock on a 1-for-1.7 basis (the Reverse Stock Split ). The par value and the authorized shares of the common stock were not adjusted as a result of the Reverse Stock Split. All issued and outstanding common stock, options for common stock, warrants for common stock, and per share amounts contained in the financial statements have been retroactively adjusted to reflect this Reverse Stock Split for all periods presented.



**Table of Contents**

**BELLICUM PHARMACEUTICALS, INC.**

**Notes to the Financial Statements**

December 31, 2013 and 2012

**NOTE 15 SUBSEQUENT EVENTS**

The Company evaluated subsequent events through the date the accompanying financial statements were available to be issued, which was October 17, 2014.

On January 15, 2014, the Company issued 1,582,706 shares of Series B for cash proceeds of approximately \$7.3 million, or \$4.625 per share.

In March and July of 2014, the line of credit was amended to allow for additional advances up to \$500,000 with the following terms: 12-month interest only draw-down period, followed by a 24-month straight-line amortization of principal and interest at the prime rate plus 2.75%. In August of 2014, \$81,548 was drawn on the line of credit.

On March 25, 2014, the Company issued 393,529 shares of common stock for \$200,700, or \$0.51 per share, in conjunction with the exercise of warrants which were set to expire in March of 2014.

In May of 2014, the NIH awarded the Company an additional \$332,608 for the grant year from April 2014 through March 2015.

On August 22, 2014, the Company issued 10,091,743 shares of Series C convertible preferred stock (Series C) at a purchase price of \$5.45 per share and warrants to purchase up to 6,559,598 shares of Series C with an exercise price of \$6.00 per share which are convertible into 3,858,549 common shares. The warrants have a five year term, but are subject to earlier termination in the event of a Qualified IPO (defined in the warrants) or upon a merger or sale of the Company. The Company received gross proceeds from the transaction of approximately \$55.0 million.

The holders of Series C have rights that are senior to the rights of the holders of all other classes of shares in the event of a liquidation of the Company. In connection with the issuance of Series C, the redemption rights of the holders of Series A and Series B were terminated, the liquidation preferences of Series A and Series B were subordinated to Series C, and accruing dividends on the shares of Series B stopped accruing.

On October 3, 2014, the Company entered into an omnibus amendment agreement with ARIAD, which amended the Amended ARIAD License to expand the license to cover a broader scope of dimerizers and licensed products for use and exploitation in any field of use other than *in vivo* administration of genetic material directly into a human being using viral vectors for the purpose of producing proteins or other macromolecules that are expressed or secreted for therapeutic or prophylactic purposes. In connection with the agreement, the Company agreed to pay ARIAD \$50,000,000 for expanded use of the license and termination of all obligations to make milestone and royalty payments to ARIAD in the future.

In connection with the amendment, the Company issued a promissory note to ARIAD for a principal amount of \$35,000,000. The principal does not accrue interest unless the Company is in default, in which case it accrues at a rate of 10% per annum. The Company made an initial principal payment of \$15,000,000 in connection with the execution

of the amendment. The Company is required to pay \$20,000,000 in a lump sum installment on or before June 30, 2015 and \$15,000,000 in a second lump sum installment on or before June 30, 2016 (or earlier under certain specified circumstances). Additionally, in connection with the second installment, ARIAD agreed to return to the Company all shares of common stock of the Company that ARIAD currently holds.

F-37

**Table of Contents**

**7,350,000 Shares**

**Common Stock**

**PROSPECTUS**

*Joint Book-Running Managers*

**Jefferies**

**Citigroup**

**Piper Jaffray**

*Co-Manager*

**Trout Capital**

December 17, 2014