INTERCEPT PHARMACEUTICALS INC
Form 424B4
June 19, 2013

Filed Pursuant to Rule 424(b)(4)
File No. 333-189194

P R O S P E C T U S

1,730,000 Shares

Common Stock

We are offering 1,730,000 shares of our common stock.

Our common stock is listed on The NASDAQ Global Market under the symbol ICPT. As of June 18, 2013, the last reported sale price of our common stock on The NASDAQ Global Market was $33.01 per share.

Investing in our common stock involves risks that are described in the Risk Factors section beginning on page 12 of this prospectus.

We are an emerging growth company and are subject to reduced public company reporting requirements. See Prospectus Summary Implications of Being an Emerging Growth Company.

	Per Share	Total
Public offering price	$33.0100	$57,107,300
Underwriting discount[(1)]	$1.9806	$3,426,438
Proceeds, before expenses, to us	$31.0294	$53,680,862

(1) The underwriters will receive compensation in addition to the underwriting discount. See Underwriting on page 35 of this prospectus for a description of the compensation payable to the underwriters.

The underwriters may also exercise their option to purchase up to an additional 259,500 shares of our common stock from us, at the public offering price, less the underwriting discount, for 30 days after the date of this prospectus.

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.

The shares will be ready for delivery on or about June 24, 2013.

BofA Merrill Lynch **Citigroup**

BMO Capital Markets

Needham & Company **Wedbush PacGrow Life Sciences** **Janney Montgomery Scott**

The date of this prospectus is June 18, 2013.

TABLE OF CONTENTS

TABLE OF CONTENTS

You should rely only on the information contained or otherwise incorporated by reference in this prospectus and any free writing prospectus prepared by or on behalf of us or to which we have referred you. We have not authorized anyone to provide you with information that is different. We are offering to sell, and seeking offers to buy, shares of common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus, any free writing prospectus, or any document incorporated by reference herein is accurate only as of its date, regardless of the time of delivery of this prospectus or of any sale of common stock. To the extent there is a conflict between the information contained in this prospectus and the information contained in any document incorporated by reference herein filed prior to the date of this prospectus, you should rely on the information in this prospectus; provided that if any statement in one of these documents is inconsistent with a statement in another document having a later date for example, a document incorporated by reference in the prospectus the statement in the document having the later date modifies or supersedes the earlier statement.

It is important for you to read and consider all information contained in this prospectus, including the documents incorporated by reference herein, in making your investment decision. You should also read and consider the information in the documents to which we have referred you in the sections entitled Where You Can Find More Information and Incorporation of Documents by Reference in this prospectus.

For investors outside of the United States: neither we nor any of the underwriters have done anything that would permit this offering or possession or distribution of this prospectus in any jurisdiction where action for that purpose is required, other than in the United States. You are required to inform yourselves about and to observe any restrictions relating to this offering and the distribution of this prospectus.

We further note that the representations, warranties and covenants made by us in any agreement that is filed as an exhibit to the registration statement of which this prospectus is a part or to any document that is incorporated by reference herein were made solely for the benefit of the parties to such agreement, including, in some cases, for the purpose of allocating risk among the parties to such agreements, and should not be deemed to be a representation,

warranty or covenant to you. Moreover, such representations, warranties or covenants were accurate only as of the date when made. Accordingly, such representations, warranties and covenants should not be relied on as accurately representing the current state of our affairs.

TABLE OF CONTENTS

PROSPECTUS SUMMARY

This summary provides an overview of selected information contained elsewhere in this prospectus or incorporated by reference into this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2012 and our other filings with the Securities and Exchange Commission listed in the section of this prospectus entitled Incorporation of Documents by Reference and does not contain all of the information you should consider before investing in our common stock. You should carefully read this prospectus, the registration statement of which this prospectus is a part and the information incorporated by reference herein in their entirety before investing in our common stock, including the information discussed under Risk Factors in this prospectus and in our Annual Report on Form 10-K for the year ended December 31, 2012 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013 incorporated by reference herein, along with our consolidated financial statements and notes thereto that are incorporated by reference herein. Unless otherwise indicated herein, the terms we, our, us, or the Company refer to Intercept Pharmaceuticals, Inc. and its wholly-owned subsidiary.

Overview

We are a biopharmaceutical company focused on the development and commercialization of novel therapeutics to treat chronic liver diseases utilizing our expertise in bile acid chemistry. Our product candidates have the potential to treat orphan and more prevalent liver diseases for which there currently are limited therapeutic solutions.

Our Lead Product Candidate

Our lead product candidate, obeticholic acid, or OCA, is a bile acid analog, a chemical substance that has a structure based on a naturally occurring human bile acid. OCA is a first-in-class product candidate that selectively binds to and induces activity in the farnesoid X receptor, or FXR, which we believe has broad liver-protective properties. We are developing OCA initially for primary biliary cirrhosis, or PBC, as a second line treatment for patients who have an inadequate response to or who are unable to tolerate standard of care therapy and therefore need additional treatment. PBC is a chronic autoimmune liver disease that, if inadequately treated, may eventually lead to cirrhosis, liver failure and death. We are conducting a Phase 3 clinical trial of OCA in PBC, which we call the POISE trial, that we anticipate will serve as the basis for seeking regulatory approval in the United States and Europe. In December 2012, we completed enrollment of the POISE trial approximately three months ahead of schedule with 217 patients, exceeding the originally targeted number of patients by approximately 20% and thereby improving the statistical power of the trial from 90% to 95%. We currently expect results from the POISE trial to be available in the second quarter of 2014. OCA has received orphan drug designation in the United States and Europe for the treatment of PBC.

We own worldwide rights to OCA outside of Japan and China, where we have exclusively licensed the compound to Dainippon Sumitomo Pharma, or DSP, and granted it an option to exclusively license OCA in certain other Asian countries. Patents covering the composition of matter for OCA expire in 2022, before any patent term adjustments or patent term extensions. Our current plan is to commercialize OCA in the United States and Europe ourselves for the treatment of PBC by targeting a limited and focused group of specialist physicians.

The liver performs many essential functions that are crucial for survival, including the regulation of bile acid metabolism. Bile acids are natural detergent-like emulsifying agents that are released from the gallbladder into the intestine when food is ingested, and are essential for the absorption of dietary cholesterol and other nutrients. In the past decade, we have learned that bile acids are also complex signaling molecules that integrate metabolic and

immune pathways involved in the healthy functioning of various tissues and organs. The biological effects of bile acids are mediated through dedicated receptors such as FXR, a nuclear receptor that regulates bile acid synthesis and clearance from the liver, thereby preventing excessive bile acid build-up in the liver, which may be toxic. In addition, bile acid activation of FXR induces anti-fibrotic, anti-inflammatory and other mechanisms that are necessary for the normal regeneration of the liver. Based on the discovery of similar FXR-mediated protective mechanisms in other organs exposed to bile acids, we believe that FXR may also be a potential target for the treatment of a number of intestinal, kidney and other diseases.

1

TABLE OF CONTENTS

PBC is a rare liver disease that primarily results from autoimmune destruction of the bile ducts that transport bile acids out of the liver. The disease causes a toxic build-up of bile acids in the liver, resulting in progressive liver damage marked by chronic inflammation and fibrosis, or scarring. In response to the bile acid mediated toxicity seen in PBC, liver cells release alkaline phosphatase, or ALP, a liver enzyme that is a key biomarker of the disease pathology. Elevated blood levels of ALP are used as the primary means of diagnosis of PBC and are closely monitored in patients as the most important indicator of treatment response and prognosis.

The only approved drug for the treatment of PBC is ursodeoxycholic acid, which is available generically as ursodiol. Ursodiol is a naturally occurring bile acid found in small quantities in humans, and is the least detergent of the various types of bile acids that make up the bile pool. Its primary mechanism of action at therapeutic doses is to dilute more detergent bile acids, but it has no known pharmacological effects mediated by FXR or other bile acid receptors. Although ursodiol is the established standard of care for the treatment of PBC, studies have shown that up to 50% of PBC patients fail to respond adequately to treatment, meaning that they continue to be at significant risk of progressing to liver failure even with treatment. The outlook and treatment options for end-stage PBC patients who fail to respond to ursodiol are limited, and include liver transplant, which is associated with significant complications and costs. Patients typically need to take approximately one gram of ursodiol daily in divided doses, which we believe presents a compliance challenge for some patients. Given this issue, coupled with ursodiol s limited efficacy in up to 50% of PBC patients, we believe that there is a significant unmet need for a novel second line therapy in PBC. We believe that OCA has the potential to provide significant benefits in the treatment of PBC, including efficacy, pharmacological activity and ease of use.

According to industry data, there are approximately 300,000 people with PBC in developed countries, of whom we believe approximately 60,000 have been diagnosed and are on ursodiol therapy. Based on this estimate, we believe there are up to 30,000 PBC patients who may currently be eligible for treatment with OCA. With increasing identification of PBC through routine liver function testing in primary care, we believe that there may be significantly more patients who will potentially be eligible for, and be interested in, receiving a new therapy if it becomes available on the market.

We have previously completed two randomized, placebo-controlled Phase 2 trials of OCA in PBC patients, one with OCA in combination with ursodiol and one with OCA as monotherapy. The results demonstrated that, over a 12-week period, single daily doses of OCA at the lowest dose of 10 milligrams (mg) met the primary endpoint in both Phase 2 trials, producing statistically significant reductions in ALP levels of greater than 20%. We consider reductions in ALP levels of greater than 10% to be a clinically meaningful improvement. Pruritus, or itching, a very common symptom in PBC patients, was the most common adverse event reported in our Phase 2 trials, with severity increasing with dose.

Our Phase 3 POISE trial has been designed to study the safety and efficacy of OCA in PBC patients with an inadequate therapeutic response to ursodiol or who are unable to tolerate ursodiol. The primary endpoint of the 12-month double-blind portion of the POISE trial is the achievement of both an ALP level of less than 1.67 times upper limit normal, or ULN, with a minimum 15% reduction in ALP level from baseline, and a normal bilirubin level, as compared to placebo. Patients with ALP and bilirubin levels within these thresholds have been shown in long-term studies to be at significantly lower risk of progressing to liver transplant and death.

We are advancing a once daily 10 mg dose of OCA in the POISE trial as our potential approvable dose. We recently completed an intention to treat analysis for the 10 mg dose groups in our two Phase 2 trials that was limited to those patients who would have met the POISE trial entry criteria. This analysis demonstrated that after 12 weeks of treatment, approximately 40% to 45% of OCA-treated patients would have met the POISE trial primary endpoint, as compared to 5% to 9% of the placebo-treated patients. In addition, 80% of OCA-treated patients across our Phase 2 trials had a reduction in ALP levels of at least 10%, as compared to 13% of placebo-treated patients.

If the POISE trial is successful, we intend to submit a New Drug Application, or NDA, to the U.S. Food and Drug Administration, or FDA, for approval of OCA for the treatment of PBC in the United States and a Marketing Authorization Application, or MAA, to the European Medicines Agency, or EMA, for approval in

TABLE OF CONTENTS

Europe, in the fourth quarter of 2014. Based on written scientific advice from the EMA, we believe that the EMA will accept our current clinical program as the basis for considering approval of OCA for PBC. With respect to the FDA, we intend to request an accelerated approval of OCA by seeking acceptance from the FDA of the POISE trial primary endpoint as a surrogate endpoint that is reasonably likely to predict clinical benefit. If the FDA grants an accelerated approval of OCA, we will be required to conduct one or more additional clinical trials post-approval to verify and confirm the clinical benefit predicted by achievement of the surrogate endpoint. This clinical outcomes trial must satisfy the FDA s definition of an adequate and well-controlled trial, would have to be substantially underway at the time of the NDA submission and would be completed after accelerated approval. Although the FDA has not confirmed our use of a surrogate endpoint in the POISE trial for regulatory approval, we are in discussions with the FDA about the design of the clinical outcomes trial and plan to initiate it by the end of 2013.

A number of published clinical studies have demonstrated that lower levels of ALP, both independently or in conjunction with normal bilirubin levels, correlate with a significant reduction in adverse clinical outcomes such as liver transplant and death. We believe that one of the key factors in the FDA s potential acceptance of our POISE trial primary endpoint as a basis for approval will be the result of additional analysis of the already available PBC clinical outcomes data. We believe that the Global PBC Study Group that we are sponsoring, which is anticipated to involve a dataset of more than 4,000 PBC patients from 15 academic centers in eight countries, and the UK-based PBC research cohort, involving a dataset of over 2,300 PBC patients from every hospital in the UK, represent the largest PBC clinical datasets assembled to analyze the correlation of biochemical therapeutic response with clinical outcomes in PBC patients. We further believe that the analyses already available confirm the results recently published, or made available to us, by four different members of the Global PBC Study Group (the University of Toronto, Mayo Clinic, University of Paris and Erasmus MC (Rotterdam)). These groups have all independently corroborated that the achievement of an ALP level of less than 1.67 times ULN, together with a normal bilirubin level, correlate with a statistically significant reduction of risk of adverse clinical outcomes such as liver transplant and death.

Additional Pipeline Opportunities Beyond OCA in PBC

In addition to PBC, we are pursuing other indications in our OCA development program, including portal hypertension, nonalcoholic steatohepatitis, or NASH, and bile acid diarrhea. The pipeline chart below shows the current stage of development of OCA for these indications, as well as the preclinical programs for our other product candidates.

* An agonist is a substance that binds to a receptor of a cell and triggers a response by that cell.

TABLE OF CONTENTS

We are currently conducting an open label Phase 2a trial of OCA in patients with portal hypertension, studying once-daily doses of 10mg and 25mg, and we presented results from the 10 mg dose group of this trial at the annual meeting of the American Association for the Study of Liver Diseases in November 2012. There are currently no approved therapies for the treatment of portal hypertension, although beta blockers are commonly used to treat patients.

In addition, OCA is currently being tested in a Phase 2b trial for the treatment of NASH, sponsored by the U.S. National Institute of Diabetes and Digestive and Kidney Diseases, or NIDDK, in collaboration with us. In November 2012, the NIDDK completed enrollment, achieving the target of 280 patients for this trial. Based on the interim analysis that was completed in June 2012, the NIDDK decided to continue this Phase 2b trial and we anticipate that final results will be available in the fourth quarter of 2014. In addition, our collaborator, DSP, has initiated a second Phase 2 NASH trial in Japan, with a targeted enrollment of 200 patients, that is anticipated to be completed in the first half of 2016. There are currently no approved therapies for the treatment of NASH.

In addition, investigators at the Imperial College of London initiated enrollment in July 2012 in an open label Phase 2a trial of OCA as a treatment for bile acid diarrhea, which we refer to as the OBADIAH trial, and presented initial results in patients with primary bile acid diarrhea at the 2013 Digestive Diseases Week annual meeting in May 2013. We expect final results from this trial will be available in the fourth quarter of 2013.

By virtue of our patent portfolio and the proprietary know-how of our employees and our collaborators at the University of Perugia, we believe that we hold a leading position in the bile acid chemistry therapeutic field. Through a longstanding collaboration with Professor Roberto Pellicciari, Ph.D., one of our co-founders, and certain scientists in the medicinal chemistry group at the University of Perugia, we have gained the capability to rationally design compounds that bind selectively and potently to FXR and other bile acid receptors. Starting with OCA, which was invented by Professor Pellicciari and, together with its underlying patents, was assigned to us under our agreements with him and the University of Perugia, our collaboration has resulted in a pipeline of bile acid analogs in addition to OCA, which target both FXR and a second dedicated bile acid receptor called TGR5, a target of interest for the treatment of type 2 diabetes and associated metabolic diseases. We intend to continue developing these and other product candidates as we advance our pipeline, in some cases subject to the procurement of additional funding or through strategic collaborations.

Recent Developments

Analysis of Data from Global Primary Biliary Cirrhosis Study Group

In April 2013, the Global PBC Study Group presented an analysis of data from over 2,100 PBC patients, among whom 981 patients would have met one of the entry criteria for our ongoing Phase 3 POISE trial of having an ALP level exceeding 1.67 times ULN and/or an abnormal bilirubin level. The data (Figure 1) show that after one year of ursodiol therapy, 58.7% of this subgroup of PBC patients (n=576/981) had an inadequate therapeutic response to ursodiol as defined by having failed to meet an endpoint identical to the primary endpoint in our ongoing POISE trial. In the ursodiol non-responder group, 30.0% of patients went on to require a liver transplant or die (n=173/576) as compared to 12.6% of patients in the ursodiol responder group (n=51/405), reflecting a 2.4-fold higher event rate for the ursodiol non-responders (p=4.5x10E-10). We believe that the analysis of this subgroup of patients from the Global PBC Study Group further substantiates the primary endpoint used in POISE as being strongly predictive of adverse clinical outcomes such as liver transplant and death in PBC patients.

In order to exclude deaths due to causes other than PBC-associated liver failure, the Global PBC Study Group analyzed younger subgroups of patients who were under 65 years old (n=789) and under 60 years old (n=666) at the time they initiated ursodiol therapy and also would have met the POISE trial entry criteria. In the under 65 subgroup (Figure 2), after one year of ursodiol therapy, 60.5% of patients (n=477/789) would have failed to meet the POISE endpoint and 28.9% of these patients went on to require a liver transplant or die (n=138/477) as compared to 8.7% of patients in the ursodiol responder group (n=27/312), reflecting a 3.3-fold higher event rate for the ursodiol non-responders (p=1x10E-7). In the under 60 subgroup (Figure 3), after one year of ursodiol therapy, 61.3% of patients (n=408/666) would have failed to meet the POISE endpoint

TABLE OF CONTENTS

and 26.2% of these patients went on to require a liver transplant or die (n=107/408) as compared to 7.4% of patients in the ursodiol responder group (n=19/258), reflecting a 3.6-fold higher event rate for the ursodiol non-responders (p=1x10E-7).

The event rate amongst the responders in the under 65 and under 60 subgroups was, respectively, 30.9% and 41.3% lower than the event rate of the responder group in the overall patient cohort that included older patients. We believe that this difference is likely due to the greater exclusion of mortality unrelated to PBC in the younger patient subgroups, resulting in even greater differentiation of the responder and non-responder groups.

The following figures show the results of the analyses conducted by the Global PBC Study Group as described above:

Figure 1 All Ursodiol-Treated Patients Meeting POISE Entry Criteria (p=4.5x10E-10)

Figure 2 Ursodiol-Treated Patients Meeting POISE Entry Criteria Under 65 Years of Age (p=1x10E-7)

TABLE OF CONTENTS

Figure 3 Ursodiol-Treated Patients Meeting POISE Entry Criteria Under 60 Years of Age (p=1x10E-7)

Initial Results from Ongoing Phase 2a Trial in Chronic Bile Acid Diarrhea

In May 2013, we announced initial results from OBADIAH, an ongoing Phase 2a trial of OCA as a treatment for primary bile acid diarrhea, or PBAD, presented at the Digestive Diseases Week conference. The initial results from the OBADIAH trial demonstrate that treatment with OCA is associated with statistically significant increased levels of fibroblast growth factor 19, or FGF19, and improvement in clinical symptoms in patients with PBAD. This disease, also known as idiopathic bile acid malabsorption, is a common chronic diarrheal condition due to excessive bile acid production and loss. PBAD is estimated to affect approximately one percent of the population and about one-third of patients diagnosed with diarrhea-predominant irritable bowel syndrome. Patients with PBAD have low levels of FGF19, a hormone released in the ileum in response to FXR activation and regulates bile acid production by the liver. As a result, excess bile acids spill into the gut and produce diarrhea by overstimulating intestinal secretions. All three of our previously completed Phase 2 trials in other indications demonstrated that OCA markedly and dose dependently stimulates the release of FGF19.

The primary outcome measure of the open-label OBADIAH trial is to assess changes in FGF19 levels over a two-week period in ten patients with PBAD and in two other groups, one consisting of patients with secondary bile acid diarrhea due to Crohn's disease and the other consisting of IBS-D patients who have normal FGF19 levels. Secondary outcome measures include clinical symptom scores, biochemical response and tolerability. Data from ten PBAD patients, the first group studied in OBADIAH, indicate that a 25 mg daily oral dose of OCA resulted in a statistically significant increase in median fasting FGF19 from 133 to 237 pg/mL, with most patients achieving a greater than 60% increase (p=0.007). In addition, clinical improvements were seen in all patients with reductions in median stool frequency from 23 to 14 per week (p=0.03) and an improvement in the median Bristol Stool Form Scale assessing stool type from 5.15 to 4.34 (p=0.05). Notably, during the two-week follow-up period after stopping OCA therapy, stool frequency returned to pre-treatment baseline values. OCA was well tolerated in all patients.

TABLE OF CONTENTS

Our Strategy

Our strategy is to develop and commercialize novel therapeutics for patients with chronic liver and other diseases, beginning with OCA for the second line treatment of PBC and other follow-on indications that we believe are underserved by existing therapies. The key elements of our strategy are to:

complete the development of OCA for its lead indication, PBC;
obtain regulatory approval of OCA for the treatment of PBC in the United States, Europe and other countries;
commercialize OCA in the United States, Europe and other countries, initially for the treatment of PBC;
continue to develop OCA in other orphan and more prevalent liver and other diseases; and
advance the earlier stage product candidates in our pipeline.

We may enter into strategic collaborations to implement our strategy.

Risks Relating to Our Business

We are a development stage biopharmaceutical company, and our business and ability to execute our business strategy are subject to a number of risks of which you should be aware before you decide to buy our common stock. In particular, you should consider the following risks, which are discussed more fully in the section entitled Risk Factors in this prospectus and in our Annual Report on Form 10-K for the year ended December 31, 2012 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013 incorporated by reference herein.

we have never been profitable, have no products approved for commercial sale and to date have not generated any revenue from product sales;
we will require substantial additional funding beyond this contemplated offering to complete the development and commercialization of OCA and to continue to advance the development of our other product candidates, and such funding may not be available on acceptable terms or at all;
OCA and/or our other product candidates may not receive regulatory approval in a timely manner or at all;
the FDA may not agree to our proposed surrogate endpoint for accelerated approval of OCA for the treatment of PBC, in which case we would need to complete an additional Phase 3 trial in order to seek approval in the United States instead of being able to seek approval based on a clinical outcomes trial to be completed after accelerated approval;
we may be subject to delays in our clinical trials, which could result in increased costs and delays or limit our ability to obtain regulatory approval for our product candidates;
because the results of earlier studies and clinical trials of our product candidates may not be predictive of future clinical trial results, our product candidates may not have favorable results in future clinical trials, which would delay or limit their future development;
we are in a highly competitive industry and face competition from existing and new treatments that may be more effective and less costly than our products;
we have never commercialized any of our product candidates and our products, even if approved, may not be accepted by healthcare providers or healthcare payors;
the failure of our collaborators to perform their obligations under our collaboration agreements may delay or otherwise harm the development and commercialization of our product candidates; and
we may be unable to maintain and protect our intellectual property assets, which could impair the advancement of our pipeline and commercial opportunities.

TABLE OF CONTENTS

Implications of Being an Emerging Growth Company

We qualify as an emerging growth company as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act. As an emerging growth company, we currently take advantage of reduced disclosure and other requirements that are otherwise applicable generally to public companies. These provisions include:

reduced disclosure about our executive compensation arrangements;
no non-binding advisory votes on executive compensation or golden parachute arrangements; and
exemption from the auditor attestation requirement in the assessment of our internal control over financial reporting.

We may take advantage of these exemptions until such time that we are no longer an emerging growth company. We would cease to be an emerging growth company on the date that is the earliest of (i) the last day of the fiscal year in which we have total annual gross revenues of $1 billion or more; (ii) December 31, 2017; (iii) the date on which we have issued more than $1 billion in nonconvertible debt during the previous three years; or (iv) the date on which we are deemed to be a large accelerated filer under the rules of the Securities and Exchange Commission. Accordingly, the information contained or incorporated by reference herein may be different than the information you receive from other public companies in which you hold stock.

Corporate Information

We were incorporated in the State of Delaware on September 4, 2002. Our principal executive offices are located at 18 Desbrosses Street, New York, NY 10013, and our telephone number is (646) 747-1000. We also have an office in San Diego, CA. Our website address is *www.interceptpharma.com*. We have included our website address in this prospectus solely as an inactive textual reference, and the information contained on, or that can be accessed through, our website is not part of this prospectus.

All brand names or trademarks appearing in this prospectus and the documents incorporated by reference are the property of their respective holders. We own or have rights to trademarks or trade names that we use in connection with the operation of our business, including our corporate names, logos and website names.

TABLE OF CONTENTS

THE OFFERING

Common stock offered by us	1,730,000 shares
Common stock to be outstanding after this offering	18,600,802 shares

Option to purchase additional shares

We have granted the underwriters an option for a period of up to 30 days to purchase up to 259,500 additional shares of common stock at the offering price.

Use of proceeds

We estimate that the net proceeds from this offering will be approximately $53.3 million, or approximately $61.4 million if the underwriters exercise their option to purchase additional shares in full, after deducting underwriting discounts and commissions and estimated offering expenses payable by us. We intend to use substantially all of the net proceeds from this offering to fund (i) the development of OCA for additional indications beyond PBC; (ii) the continuation of the long-term safety extension portion of our POISE trial and the proposed Phase 3 clinical outcomes trial after the anticipated FDA and EMA filings; (iii) certain pre-commercialization and potential commercial launch activities of OCA for PBC; and (iv) general corporate purposes, including general and administrative expenses, capital expenditures, working capital and prosecution and maintenance of our intellectual property.

See Use of Proceeds for a more complete description of the intended use of proceeds from this offering.

Risk factors

You should read the Risk Factors section of this prospectus, our Annual Report on Form 10-K for the year ended December 31, 2012 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013, incorporated by reference herein, for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.

NASDAQ Global Market symbol	ICPT

The number of shares of common stock to be outstanding after this offering is based on an aggregate of 16,870,802 shares outstanding as of May 31, 2013. The number of shares of our common stock outstanding immediately after this offering excludes:

1,830,547 shares of common stock issuable upon exercise of outstanding options as of May 31, 2013, at a weighted average exercise price of $16.46 per share, of which 1,083,457 shares were vested as of such date;

restricted stock units for 164,710 shares of our common stock that were unvested as of May 31, 2013;

960,418 shares of common stock issuable upon the exercise of warrants outstanding as of May 31, 2013, at a weighted average exercise price of $9.79 per share; and

531,003 shares of our common stock reserved for future issuance under our 2012 Equity Incentive Plan, or the 2012 Plan, plus any future increases in the number of shares of common stock reserved for issuance under the 2012 Plan pursuant to evergreen provisions.

Except as otherwise indicated, all information in this prospectus assumes no exercise by the underwriters of their option to purchase additional shares of our common stock.

TABLE OF CONTENTS

SUMMARY CONSOLIDATED FINANCIAL DATA

The summary consolidated financial data presented below for the years ended December 31, 2010, 2011 and 2012 are derived from our audited consolidated financial statements incorporated by reference in this prospectus from our Annual Report on Form 10-K for the year ended December 31, 2012. The summary consolidated financial data presented below for the three months ended March 31, 2012 and 2013, and for the period from inception (September 4, 2002) to March 31, 2013 (required to be included since we are a development stage company), are derived from our unaudited financial statements incorporated by reference in this prospectus from our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013. The unaudited consolidated financial statements have been prepared on the same basis as our audited consolidated financial statements and include, in the opinion of management, all adjustments necessary for a fair presentation of the financial information set forth in those statements.

Our historical results are not necessarily indicative of future operating results. You should read this summary consolidated financial data in conjunction with the sections entitled Risk Factors, Capitalization and Management s Discussion and Analysis of Financial Condition and Results of Operations and our consolidated financial statements and related notes, all included elsewhere or incorporated by reference in this prospectus. For more details on how you can obtain the documents incorporated by reference in this prospectus, see Where You Can Find More Information and Incorporation of Documents by Reference.

	Years Ended December 31,			Three Months Ended March 31,		Period From September 4, 2002 (Inception) Through March 31, 2013
	2010	2011	2012	2012	2013	
	(in thousands, except share and per share amounts)					
				(unaudited)		(unaudited)
Statement of Operations Data:						
Licensing revenue	$	$1,805	$2,446	$759	$405	$4,657
Costs and expenses:						
Research and development	12,710	11,426	16,183	3,060	4,832	76,267
General and administrative	3,644	4,209	5,177	1,059	2,397	31,995
Total operating expenses	16,354	15,635	21,360	4,119	7,229	108,262
Other income (expense):						
Revaluation of warrants	672	1,045	(24,626)	678	(3,683)	(26,758)
Other income (expense), net	594	48	(104)	2	296	1,970
	1,266	1,093	(24,730)	680	(3,387)	(24,788)
Net loss	(15,088)	(12,737)	(43,643)	(2,680)	(10,211)	(128,393)
Dividends on preferred stock, not declared	(2,901)	(3,000)	(2,630)	(750)		(10,944)
Net loss attributable to common stockholders	$(17,989)	$(15,737)	$(46,273)	$(3,430)	$(10,211)	$(139,338)
Net loss per common share, basic and diluted	$(5.40)	$(4.73)	$(7.36)	$(1.03)	$(0.62)	

Weighted average shares outstanding, basic and diluted	3,329,666	3,329,666	6,283,238	3,329,266	16,558,297

10

TABLE OF CONTENTS

The following summary unaudited balance sheet data as of March 31, 2013 is presented:

on an actual basis; and

on a pro forma basis to give effect to our sale of 1,730,000 shares of common stock in this offering at the offering price of $33.01 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

The summary unaudited pro forma balance sheet is for informational purposes only and does not purport to indicate balance sheet information as of any future date.

	March 31, 2013	
	Actual	Pro Forma
	(Unaudited) (In thousands)	
Balance Sheet Data:		
Cash, cash equivalents and investment securities	$ 104,220	$ 157,551
Total assets	106,196	159,527
Working capital	96,159	149,490
Warrant liability, total	30,413	30,413
Deferred revenue, total	11,757	11,757
Total liabilities	45,396	45,396
Common stock	17	18
Additional paid-in capital	189,423	242,752
Accumulated deficit during the development stage	(128,393)	(128,393)
Total stockholders' equity	60,800	114,131

TABLE OF CONTENTS

RISK FACTORS

A purchase of shares of our common stock is an investment in our securities and involves a high degree of risk. You should carefully consider the risks and uncertainties and all other information contained in or incorporated by reference in this prospectus, including the risks and uncertainties discussed under Risk Factors in our Annual Report on Form 10-K for the year ended December 31, 2012 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013. All of these risk factors are incorporated by reference herein in their entirety. If any of these risks actually occur, our business, financial condition and results of operations would likely suffer. In that case, the market price of our common stock could decline, and you may lose part or all of your investment in our company. Additional risks of which we are not presently aware or that we currently believe are immaterial may also harm our business and results of operations.

Risks Relating to This Offering and Ownership of our Common Stock

We have a significant stockholder, which will limit your ability to influence corporate matters and may give rise to conflicts of interest.

Genextra S.p.A., together with its affiliates, whom we refer to collectively as Genextra, is our largest stockholder. As of May 31, 2013, Genextra owned 7,187,217 shares of our common stock and warrants to purchase an additional 865,381 shares of our common stock. The shares of common stock owned by Genextra represented approximately 42.6% of our outstanding common stock as of May 31, 2013. Following this offering, we anticipate that the shares of common stock owned by Genextra will represent 38.6% of our outstanding common stock. Accordingly, Genextra exerts and will continue to exert significant influence over us and any action requiring the approval of the holders of our common stock, including the election of directors and amendments to our organizational documents, such as increases in our authorized shares of common stock, and approval of significant corporate transactions. This concentration of voting power makes it less likely that any other holder of common stock or directors of our business will be able to affect the way we are managed and could delay or prevent an acquisition of us on terms that other stockholders may desire. In addition, if Genextra obtains a majority of our common stock, Genextra would be able to control all matters submitted to our stockholders for approval, as well as our management and affairs. For example, Genextra would be able to control the election of directors, amendments to our organizational documents and approval of any merger, consolidation, sale of all or substantially all of our assets or other business combination or reorganization. In addition, if Genextra obtains a majority of our common stock, we would be deemed a controlled company for purposes of NASDAQ listing requirements. Under NASDAQ rules, a controlled company may elect not to comply with certain NASDAQ corporate governance requirements, including (i) the requirement that a majority of our board of directors consist of independent directors, (ii) the requirement that the compensation of our officers be determined or recommended to the board by a compensation committee that is composed entirely of independent directors, and (iii) the requirement that director nominees be selected or recommended to the board by a majority of independent directors or a nominating committee that is composed entirely of independent directors.

Furthermore, the interests of Genextra may not always coincide with your interests or the interests of other stockholders and Genextra may act in a manner that advances its best interests and not necessarily those of other stockholders, including seeking a premium value for its common stock, and might affect the prevailing market price for our common stock. Our board of directors, which consists of seven directors, including two affiliated with Genextra, has the power to set the number of directors on our board from time to time.

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

We intend to use substantially all of the net proceeds from this offering to fund (i) the development of OCA for additional indications beyond PBC; (ii) the continuation of the long-term safety extension portion of our POISE trial and the proposed Phase 3 clinical outcomes trial after the anticipated FDA and EMA filings; (iii) certain pre-commercialization and potential commercial launch activities of OCA for PBC; and (iv) for general corporate purposes, including general and administrative expenses, capital expenditures, working capital and prosecution and maintenance of our intellectual property. Although we currently intend to use the

TABLE OF CONTENTS

net proceeds from this offering in such a manner, we will have broad discretion in the application of the net proceeds. Our failure to apply these funds effectively could affect our ability to continue to develop and commercialize our product candidates.

Purchasers in this offering will experience immediate and substantial dilution in the book value of their investment.

The public offering price is substantially higher than the net tangible book value per share of our common stock based on the total value of our tangible assets less our total liabilities immediately following this offering. Therefore, if you purchase shares of our common stock in this offering, you will experience immediate and substantial dilution of $26.80 per share in the price you pay for shares of our common stock as compared to its pro forma net tangible book value giving effect to this offering. To the extent outstanding options or warrants to purchase shares of common stock that are in-the-money are exercised, there will be further dilution. For further information on this calculation, see Dilution elsewhere in this prospectus.

A significant portion of our total outstanding shares of common stock is restricted from resale but may be sold into the market in the future. The sale of these shares could cause the market price of our common stock to drop significantly, even if our business is doing well.

Sales of a substantial number of shares of our common stock in the public market could occur in the future. These sales, or the perception in the market that the holders of a large number of shares of common stock intend to sell shares, could reduce the market price of our common stock and could impair our ability to raise capital through the sale of additional equity securities. We are unable to predict the effect that sales may have on the prevailing market price of our common stock.

As of May 31, 2013, we had 16,870,802 shares of common stock outstanding. Of these shares, an aggregate of 9,747,711 shares of our common stock, or 57.8% of our outstanding shares, were held by our officers, directors, beneficial owners of 5% or more of our securities (other than FMR LLC and its affiliates) and their respective affiliates, which may be sold subject to Rule 144. Following this offering, it is anticipated that these shares will represent approximately 52.4% of our outstanding shares. In addition, all of our directors and officers and certain holders of more than 5% of our common stock are subject to lock-up agreements with the underwriters of this offering that restrict the stockholders ability to transfer shares of our common stock for at least 90 days from the date of this prospectus. The lock-up agreements limit the number of shares of common stock that may be sold immediately following the public offering. Subject to limitations, approximately 9,747,711 shares will become eligible for sale upon expiration of the lock-up period, as calculated and described in more detail in the section entitled Shares Eligible for Future Sale. In addition, shares issued or issuable upon exercise of options and warrants vested as of the expiration of the lock-up period will be eligible for sale at that time. Sales of stock by these stockholders could have a material adverse effect on the trading price of our common stock.

In addition, as of May 31, 2013, holders of an aggregate of 11,075,680 shares of our common stock, including shares underlying warrants of such holders, have rights, subject to certain conditions and the lock-up described above, to require us to file registration statements covering their shares or to include their shares in registration statements that we may file for ourselves or other stockholders. We also have registered 2,712,103 shares of common stock, a portion of which we have issued and a portion of which we may issue under our equity compensation plans. Once issued and vested, these shares of common stock can be freely sold in the public market. Any sales of securities by these

stockholders, option holders and warrant holders could have a material adverse effect on the trading price of our common stock.

A significant portion of our total outstanding shares of common stock isrestricted from resale but may be sold into th

TABLE OF CONTENTS

CAUTIONARY NOTE REGARDING FORWARD-LOOKING STATEMENTS

This prospectus and the documents incorporated by reference into it contain, in addition to historical information, certain information, assumptions and discussions that may constitute forward-looking statements that involve substantial risks and uncertainties. All statements other than statements of historical facts contained in this prospectus, including statements regarding our strategy, future operations, future financial position, future revenue, projected costs, prospects, plans, objectives of management and expected market growth are forward-looking statements. These statements involve known and unknown risks, uncertainties and other important factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements.

The words anticipate, believe, estimate, expect, intend, may, plan, predict, project, target, poten should, continue, and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words. These forward-looking statements include, among other things, statements about:

the accuracy of our estimates regarding expenses, future revenues and capital requirements;

the initiation, cost, timing, progress and results of our development activities, preclinical studies and clinical trials;

the timing of and our ability to obtain and maintain regulatory approval of obeticholic acid, or OCA, and any other product candidates we may develop, and any related restrictions, limitations, and/or warnings in the label of any approved product candidates;

our plans to research, develop and commercialize our future product candidates;

our collaborators election to pursue research, development and commercialization activities;

our ability to attract collaborators with development, regulatory and commercialization expertise;

our ability to obtain and maintain intellectual property protection for our product candidates;

our ability to successfully commercialize our product candidates;

the size and growth of the markets for our product candidates and our ability to serve those markets;

the rate and degree of market acceptance of any future products;

the success of competing drugs that are or become available;

regulatory developments in the United States and other countries;

the performance of our third-party suppliers and manufacturers;

our ability to obtain additional financing;

our use of the proceeds from this offering and our recently completed initial public offering;

our expectations regarding the time during which we will be an emerging growth company under the JOBS Act;

the accuracy of our estimates regarding expenses, future revenues, capital requirements and the need for additional financing; and

our ability to attract and retain key scientific or management personnel.

These forward-looking statements are only predictions and we may not actually achieve the plans, intentions or expectations disclosed in our forward-looking statements, so you should not place undue reliance on our forward-looking statements. Actual results or events could differ materially from the plans, intentions and expectations disclosed in the forward-looking statements we make. We have based these forward-looking statements largely on our current expectations and projections about future events and trends that we believe may affect our business, financial condition and operating results. We have included important factors in the cautionary statements included in this prospectus and other documents incorporated by reference herein,

TABLE OF CONTENTS

particularly under the heading Risk Factors, that could cause actual future results or events to differ materially from the forward-looking statements that we make. Our forward-looking statements do not reflect the potential impact of any future acquisitions, mergers, dispositions, joint ventures or investments we may make.

The forward-looking statements in this prospectus represent our views as of the date of this prospectus. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, we do not assume any obligation to update any forward-looking statements whether as a result of new information, future events or otherwise, except as required by applicable law. You should, therefore, not rely on these forward-looking statements as representing our views as of any date subsequent to the date of this prospectus.

Industry and Market Data

This prospectus and the documents incorporated by reference herein contain estimates and other statistical data made by independent parties and by us relating to market size and growth and other data about our industry. We obtained such industry and market data from our own research as well as from industry and general publications, surveys and studies conducted by third parties. This data involves a number of assumptions and limitations and contains projections and estimates of the future performance of the industries in which we operate that are subject to a high degree of uncertainty. We caution you not to give undue weight to such projections, assumptions and estimates. Further, industry and general publications, studies and surveys generally state that they have been obtained from sources believed to be reliable, although they do not guarantee the accuracy or completeness of such information. While we believe that these publications, studies and surveys are reliable, we have not independently verified the data contained in them. In addition, while we believe that the results and estimates from our internal research are reliable, such results and estimates have not been verified by any independent source.

TABLE OF CONTENTS

USE OF PROCEEDS

We estimate that our net proceeds from the sale of 1,730,000 shares of common stock in this offering will be approximately $53.3 million after deducting underwriting discounts and commissions and estimated offering expenses payable by us. If the option to purchase additional shares is exercised in full, we estimate that our net proceeds will be approximately $61.4 million.

We currently estimate that we will use the net proceeds from this offering, as follows:

- to fund the development of OCA for additional indications beyond PBC;
- to fund the continuation of the long-term safety extension portion of our POISE trial and the proposed Phase 3 clinical outcomes trial after the anticipated FDA and EMA filings;
- to fund certain pre-commercialization and potential commercial launch activities of OCA for PBC; and
- for general corporate purposes, including general and administrative expenses, capital expenditures, working capital and prosecution and maintenance of our intellectual property.

We have not determined the amounts we plan to spend on any of the items listed above or the timing of these expenditures. Our expected use of the net proceeds from this offering represents our current intentions based upon our present plans and business conditions. The amounts and timing of our actual use of net proceeds will vary depending on numerous factors, including the relative success and cost of our research, preclinical and clinical development programs, the pre-commercialization activities we may engage in for OCA in PBC and the timing of such activities, the amount and timing of additional revenues, if any, received from our collaborations with DSP and Servier, whether we are able to enter into future collaborations, and any unforeseen cash needs. 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 other clinical trials or preclinical activities if the net proceeds from this offering and the other sources of cash are less than expected. We have no current understandings, agreements or commitments for any material acquisitions or licenses of any products, businesses or technologies.

Based upon our current operating plan and initiatives described above, we believe that our planned use of the net proceeds from this offering and our existing cash, cash equivalents and short-term investments will enable us to fund our operating expenses and capital expenditure requirements into early 2016. This estimate reflects our planned use of the net proceeds from this offering described above in addition to the planned expenditures in our existing operating plan relating to, among other items, our ongoing POISE trial and long-term safety extension of the POISE trial; nonclinical studies and clinical trials and consulting expenditures to support our planned regulatory submissions for OCA in PBC; anticipated pre-commercial activities for OCA in PBC; and IND-enabling studies of INT-767. We have based our estimates on assumptions that may prove to be wrong, and we may use our available capital resources sooner than we currently expect. Because of the numerous risks and uncertainties associated with the development and commercialization of our product candidates, we are unable to estimate the amounts of increased capital outlays and operating expenditures necessary to complete the development of our product candidates.

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

PRICE RANGE OF OUR COMMON STOCK

Our common stock has been listed on the NASDAQ Global Market since October 11, 2012 under the symbol ICPT. Prior to that date, there was no public market for our common stock. Shares sold in our initial public offering on October 10, 2012 were priced at $15.00 per share.

On June 18, 2013, the closing price for our common stock as reported on the NASDAQ Global Market was $33.01 per share. The following table sets forth the ranges of high and low sales prices per share of our common stock as reported on the NASDAQ Global Market for the period indicated. Such quotations represent inter-dealer prices without retail markup, markdown or commission and may not necessarily represent actual transactions.

Year Ended December 31, 2012	High	Low
Fourth quarter (from October 11, 2012)	$ 35.99	$ 17.96

Year Ended December 31, 2013	High	Low
First quarter	$ 42.67	$ 33.45
Second quarter (through June 18, 2013)	$ 37.93	$ 30.38

Stockholders

As of June 17, 2013, there were 38 stockholders of record, which excludes stockholders whose shares were held in nominee or street name by brokers. The actual number of common stockholders is greater than the number of record holders, and includes stockholders who are beneficial owners, but whose shares are held in street name by brokers and other nominees. This number of holders of record also does not include stockholders whose shares may be held in trust by other entities.

TABLE OF CONTENTS

CAPITALIZATION

The following table sets forth our cash and cash equivalents and investment securities as well as capitalization as of March 31, 2013:

on an actual basis; and

on a pro forma basis to give effect to our sale of 1,730,000 shares of common stock in this offering at the offering price of $33.01 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us.

You should read this table together with Selected Financial Data included elsewhere in this prospectus, and our financial statements and related notes and the Management s Discussion and Analysis of Financial Condition and Results of Operations in our Annual Report on Form 10-K for the year ended December 31, 2012 and Quarterly Report on Form 10-Q for the quarter ended March 31, 2013, all of which are incorporated by reference in this prospectus.

	March 31, 2013	
	Actual	Pro Forma
	(Unaudited)	
	(In thousands)	
Cash, cash equivalents and investment securities	$104,220	$157,551
Warrant liability, total	30,413	30,413
Stockholders' equity		
Common stock, $0.001 par value; 25,000,000 shares authorized, 16,633,964 shares issued and outstanding, actual; 18,363,964 shares issued and outstanding, as adjusted	17	18
Additional paid-in capital	189,423	242,752
Accumulated other comprehensive loss, net	(245)	(245)
Accumulated deficit during the development stage	(128,393)	(128,393)
Total stockholders' equity	60,800	114,131
Total capitalization	$91,213	$144,544

The number of shares of common stock to be outstanding after this offering is based on 16,633,964 shares of common stock outstanding on March 31, 2013. The table above does not include:

1,645,909 shares of common stock issuable upon exercise of outstanding options as of March 31, 2013, at a weighted average exercise price of $12.81 per share, of which 1,184,273 shares were vested as of such date;

restricted stock units for 176,188 shares of our common stock that were unvested as of March 31, 2013;

1,042,985 shares of common stock issuable upon the exercise of warrants outstanding as of March 31, 2013, at a weighted average exercise price of $9.76 per share; and

881,354 shares of our common stock reserved for future issuance under our 2012 Equity Incentive Plan, or the 2012 Plan, plus any future increases in the number of shares of common stock reserved for issuance under the 2012 Plan pursuant to evergreen provisions.

TABLE OF CONTENTS

DILUTION

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

As of March 31, 2013, our historical net tangible book value was $60.8 million, or $3.66 per share of common stock. Historical net tangible book value per share represents the amount of our total tangible assets less total liabilities, divided by 16,633,964, the number of shares of common stock outstanding on March 31, 2013.

After giving effect to the sale of 1,730,000 shares of our common stock in this offering at the offering price of $33.01 per share, after deducting underwriting discounts and commissions and estimated offering expenses payable by us, our pro forma net tangible book value as of March 31, 2013 would have been $114.1 million, or $6.21 per share. This amount represents an immediate increase in pro forma net tangible book value of $2.56 per share to our existing stockholders and an immediate dilution in pro forma net tangible book value of approximately $26.80 per share to new investors purchasing shares of our common stock in this offering. We determine dilution by subtracting the pro forma net tangible book value per share after the offering from the amount of cash that a new investor paid for a share of common stock.

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

Offering price per share		$ 33.01
Historical net tangible book value per share as of March 31, 2013	$ 3.66	
Increase in net tangible book value per share attributable to new investors		2.56
Pro forma net tangible book value per share after the offering		6.21
Dilution per share to new investors		$ 26.80

If the underwriters exercise their option to purchase additional shares in full, the pro forma net tangible book value per share after giving effect to the offering would be $6.56 per share. This represents an immediate increase in pro forma net tangible book value of $2.91 per share to existing stockholders and an immediate dilution in net tangible book value of $26.45 per share to new investors purchasing shares of our common stock in this offering.

The table above does not include:

1,645,909 shares of common stock issuable upon exercise of outstanding options as of March 31, 2013, at a weighted average exercise price of $12.81 per share, of which 1,184,273 shares were vested as of such date;

restricted stock units for 176,188 shares of our common stock that were unvested as of March 31, 2013;

1,042,985 shares of common stock issuable upon the exercise of warrants outstanding as of March 31, 2013, at a weighted average exercise price of $9.76 per share; and

881,354 shares of our common stock reserved for future issuance under our 2012 Equity Incentive Plan, or the 2012 Plan, plus any future increases in the number of shares of common stock reserved for issuance under the 2012 Plan pursuant to evergreen provisions.

To the extent that outstanding options or warrants are exercised, you will experience further dilution. In addition, we may choose to raise additional capital 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 that additional capital is raised through the sale of equity or convertible debt securities, the issuance of these securities may result in further dilution to our

stockholders.

TABLE OF CONTENTS

SELECTED FINANCIAL DATA

The following table sets forth our selected financial data for the periods and as of the dates indicated. You should read the following selected financial data in conjunction with our audited and unaudited financial statements and the related notes thereto and the Management s Discussion and Analysis of Financial Condition and Results of Operations section of our Annual Report on Form 10-K for the year ended December 31, 2012 and our Quarterly Report on Form 10-Q for the quarter ended March 31, 2013 incorporated by reference herein.

The statement of operations data for the years ended December 31, 2010, 2011 and 2012, and the balance sheet data as of December 31, 2010, 2011 and 2012, are derived from our audited financial statements incorporated by reference in this prospectus. The statement of operations data for the three months ended March 31, 2012 and 2013, and for the period from inception (September 4, 2002) to March 31, 2013 (required to be included since we are a development stage company) and the balance sheet data as of March 31, 2013, are derived from our unaudited financial statements and the related notes from our unaudited financial statements incorporated by reference in this prospectus. Our interim unaudited financial statements have been prepared in accordance with generally accepted accounting principles in the United States of America, or GAAP, on the same basis as the annual audited financial statements and, in the opinion of management, reflect all adjustments, consisting only of normal recurring adjustments, necessary to present a fair statement of our financial position as of March 31, 2013 and the results of our operations for the three months ended March 31, 2012 and 2013 and for the period from inception (September 4, 2002) to March 31, 2013.

Our historical results are not necessarily indicative of the results that may be expected in the future and interim results are not necessarily indicative of results to be expected for any other period or the full year. For more details on how you can obtain the documents incorporated by reference in this prospectus, see Where You Can Find More Information and Incorporation of Documents by Reference.

	Years Ended December 31,			Three Months Ended March 31,		Period From September 4, 2002 (Inception)
	2010	2011	2012	2012	2013	Through March 31, 2013
	(in thousands, except share and per share amounts)					
				(unaudited)		(unaudited)
Statement of Operations Data:						
Licensing revenue	$	$1,805	$2,446	$759	$405	$4,657
Costs and expenses:						
Research and development	12,710	11,426	16,183	3,060	4,832	76,267
General and administrative	3,644	4,209	5,177	1,059	2,397	31,995
Total operating expenses	16,354	15,635	21,360	4,119	7,229	108,262
Other income (expense):						
Revaluation of warrants	672	1,045	(24,626)	678	(3,683)	(26,758)
Other income (expense), net	594	48	(104)	2	296	1,970
	1,266	1,093	(24,730)	680	(3,387)	(24,788)

Net loss	(15,088)	(12,737)	(43,643)	(2,680)	(10,211)	(128,393)
Dividends on preferred stock, not declared	(2,901)	(3,000)	(2,630)	(750)		(10,944)
Net loss attributable to common stockholders	$(17,989)	$(15,737)	$(46,273)	$(3,430)	$(10,211)	$(139,338)
Net loss per common share, basic and diluted	$(5.40)	$(4.73)	$(7.36)	$(1.03)	$(0.62)	
Weighted average shares outstanding, basic and diluted	3,329,666	3,329,666	6,283,238	3,329,266	16,558,297	

TABLE OF CONTENTS

	December 31,			March 31,
	2010	2011	2012	2013
	(in thousands)			(unaudited)
Balance Sheet Data:				
Cash, cash equivalents and investment securities	$15,424	$17,707	$110,194	$104,220
Total assets	17,118	19,470	112,179	106,196
Working capital	13,890	14,872	98,814	96,159
Accounts payable, accrued expenses and other liabilities	1,587	1,504	3,746	3,226
Warrant liability, total	6,881	5,836	30,359	30,413
Deferred revenue, total		14,608	12,162	11,757
Common and preferred stock	31	31	17	17
Additional paid-in capital	70,268	72,134	184,100	189,423
Accumulated deficit during development stage	(61,803)	(74,540)	(118,183)	(128,393)
Total stockholder's equity (deficit)	8,318			