PTC THERAPEUTICS, INC.
Form S-1/A
February 10, 2014

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As filed with the Securities and Exchange Commission on February 10, 2014

Registration No. 333-193677

UNITED STATES
SECURITIES AND EXCHANGE COMMISSION
Washington, D.C. 20549

AMENDMENT NO. 1
TO
FORM S-1
REGISTRATION STATEMENT
UNDER
THE SECURITIES ACT OF 1933

PTC THERAPEUTICS, INC.
(Exact name of registrant as specified in its charter)

Delaware	**2834**	**04-3416587**
(State or other jurisdiction of incorporation or organization)	(Primary Standard Industrial Classification Code Number)	(I.R.S. Employer Identification No.)

100 Corporate Court
South Plainfield, New Jersey 07080
(908) 222-7000
(Address, including zip code, and telephone number, including area code, of registrant's principal executive offices)

Stuart W. Peltz, Ph.D.
Chief Executive Officer
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South Plainfield, New Jersey 07080
(908) 222-7000
(Name, address, including zip code, and telephone number, including area code, of agent for service)

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Approximate date of commencement of proposed sale to the public:
As soon as practicable after this Registration Statement is declared effective.

If any of the securities being registered on this form are to be offered on a delayed or continuous basis pursuant to Rule 415 under the Securities Act of 1933, check the following box. o

If this form is filed to register additional securities for an offering pursuant to Rule 462(b) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o

If this form is a post-effective amendment filed pursuant to Rule 462(c) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o

If this form is a post-effective amendment filed pursuant to Rule 462(d) under the Securities Act, check the following box and list the Securities Act registration statement number of the earlier effective registration statement for the same offering. o

Indicate by check mark whether the registrant is a large accelerated filer, an accelerated filer, a non-accelerated filer, or a smaller reporting company. See the definitions of "large accelerated filer," "accelerated filer" and "smaller reporting company" in Rule 12b-2 of the Exchange Act.

Large accelerated filer o	Accelerated filer o	Non-accelerated filer ý (Do not check if a smaller reporting company)	Smaller reporting company o

CALCULATION OF REGISTRATION FEE

Title of Each Class of Securities To Be Registered	**Proposed Maximum**	**Amount of Registration**

	Aggregate Offering Price(1)	Fee(2)(3)
Common Stock, $0.001 par value per share	$86,250,000	$11,109

(1) Estimated solely for the purpose of calculating the registration fee pursuant to Rule 457(o) under the Securities Act of 1933, as amended.

(2) Calculated pursuant to Rule 457(o) based on a bona fide estimate of the proposed maximum aggregate offering price.

(3) A registration fee of $9,660 was previously paid in connection with the Registration Statement, and an additional amount of $1,449 is being paid herewith.

The Registrant hereby amends this Registration Statement on such date or dates as may be necessary to delay its effective date until the Registrant shall file a further amendment which specifically states that this Registration Statement shall thereafter become effective in accordance with Section 8(a) of the Securities Act of 1933 or until the Registration Statement shall become effective on such date as the Commission, acting pursuant to said Section 8(a), may determine.

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The information in this prospectus is not complete and may be changed. We may not sell these securities until the registration statement filed with the Securities and Exchange Commission is effective. This prospectus is not an offer to sell these securities and it is not soliciting an offer to buy these securities in any state where the offer or sale is not permitted.

Subject to completion, dated February 10, 2014

Prospectus

$75,000,000

Common stock

This is a public offering of common stock by PTC Therapeutics, Inc. We are selling $75,000,000 of shares of our common stock.

Our common stock trades on The NASDAQ Global Select Market under the trading symbol "PTCT". On February 7, 2014, the last sale price of our common stock as reported on The NASDAQ Global Select Market was $22.76 per share.

We are an "emerging growth company" and have elected to rely on certain reduced public company disclosure requirements. See "Prospectus summary Implications of being an emerging growth company."

Investing in our common stock involves a high degree of risk. See "Risk factors" beginning on page 11.

	Per share	Total
Public offering price	$	$
Underwriting discounts and commissions(1)	$	$
Proceeds to PTC Therapeutics, Inc., before expenses	$	$

(1) The underwriters will receive compensation in addition to the underwriting discount. See "Underwriting" on page 177.

Celgene European Investment Company LLC, or CEIC, one of our existing investors, has indicated an interest in purchasing up to approximately $3.2 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, CEIC may determine to purchase fewer shares than it has indicated an interest in purchasing or not to purchase any shares in this offering. In addition, the underwriters could determine to sell fewer shares to CEIC than it has indicated an interest in purchasing or not to sell any shares to CEIC. The underwriters will receive the same underwriting discount on any shares purchased by CEIC as they will on any other shares sold to the public in this offering.

We have granted the underwriters an option for a period of 30 days to purchase up to $11,250,000 of additional shares of our common stock to cover any over-allotments. The underwriters can exercise this right at any time within 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 passed on the adequacy or accuracy of this prospectus. Any representation to the contrary is a criminal offense.

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

J.P. Morgan **Credit Suisse**

Deutsche Bank Securities **Cowen and Company**

Wedbush PacGrow Life Sciences **Emerging Growth Equities, Ltd.**

The date of this prospectus is , 2014

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We have not authorized anyone to provide you with information other than that contained in this prospectus or in any free writing prospectus prepared by or on behalf of us or to which we have referred you. We take no responsibility for, and can provide no assurance as to the reliability of, any other information that others may give to you. We are offering to sell, and seeking offers to buy, shares of our common stock only in jurisdictions where offers and sales are permitted. The information contained in this prospectus is accurate only as of the date of this prospectus, regardless of the time of delivery of this prospectus or any sale of our common stock. Our business, financial conditions, results of operations and prospects may have changed since that date.

No action is being taken in any jurisdiction outside the United States to permit a public offering of our common stock or possession or distribution of this prospectus in that jurisdiction. Persons who come into possession of this prospectus in jurisdictions outside the United States are required to inform themselves about and to observe any restrictions as to this offering and the distribution of this prospectus applicable to that jurisdiction.

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Prospectus summary

This summary highlights information contained elsewhere in this prospectus. This summary does not contain all of the information you should consider before investing in our common stock. You should read this entire prospectus carefully, especially the "Risk factors" section and our financial statements and the related notes appearing elsewhere in this prospectus, before making an investment decision.

Our company overview

We are a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small-molecule drugs that target post-transcriptional control processes. While our discovery programs are directed at targets in multiple therapeutic areas, we are focusing particularly on the development and commercialization of treatments for orphan and ultra-orphan disorders. Our lead product candidate is ataluren for the treatment of patients with genetic disorders that arise from a type of genetic mutation known as a nonsense mutation. We hold worldwide commercialization rights to ataluren for all indications in all territories. Ataluren is in late stage clinical development for the treatment of Duchenne muscular dystrophy caused by nonsense mutations, or nmDMD, and cystic fibrosis caused by nonsense mutations, or nmCF. There are currently no marketed therapies approved to treat the underlying cause of nmDMD or nmCF. The European Medicines Agency, or EMA, has designated ataluren as an orphan medicinal product, and the U.S. Food and Drug Administration, or FDA, has granted orphan drug designation to ataluren for the treatment of both nmDMD and nmCF.

We have initiated a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmDMD. We refer to this trial as the Ataluren Confirmatory Trial in DMD, or ACT DMD. We dosed the first patient in this trial in 2013 and expect to complete enrollment in mid-2014. In October 2012, we submitted a marketing authorization application, or MAA, to the EMA for conditional approval of ataluren for the treatment of nmDMD. In January 2014, EMA's Committee for Medicinal Products for Human Use, or CHMP, adopted a negative opinion recommending the refusal of the granting of the conditional marketing authorization for ataluren for the treatment of nmDMD. We have requested a re-examination of the CHMP opinion. We are also planning a Phase 3 clinical trial of ataluren for the treatment of nmCF. We plan to begin dosing patients in this trial in the first half of 2014.

We have completed a Phase 2b clinical trial of ataluren for the treatment of nmDMD and a Phase 3 clinical trial of ataluren for the treatment of nmCF. We did not achieve the primary efficacy endpoint in either trial with the pre-specified level of statistical significance. However, we believe that the collective data from these trials, including retrospective and subgroup analyses that we have performed, provide strong support for concluding that ataluren was active and showed clinically meaningful improvements over placebo in these trials. In addition, we believe that our experience in these completed clinical trials has allowed us to enhance the designs of our confirmatory Phase 3 clinical trials and improve our likelihood of success in these trials. Accordingly, we initiated our confirmatory Phase 3 ACT DMD clinical trial and are planning a confirmatory Phase 3 clinical trial of ataluren for the treatment of nmCF. Ataluren has been generally well tolerated in all of our clinical trials to date. We also plan to pursue additional indications for ataluren beyond nmDMD and nmCF and expect to initiate a proof-of-concept study for a third indication in 2014.

We continue to advance the development of our spinal muscular atrophy collaboration with F. Hoffman-La Roche Ltd and Hoffman-La Roche Inc., which we refer to collectively as Roche, and the Spinal Muscular Atrophy Foundation, or SMA Foundation. A development candidate for the program was selected in August 2013, and a Phase 1 clinical program was initiated in healthy volunteers in January 2014. Each of these events triggered a milestone payment to us from Roche.

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The letters "PTC" in our corporate name are an acronym for post-transcriptional control processes, which are the regulatory events that occur in cells after a messenger RNA, or mRNA, molecule is copied, or transcribed, from DNA. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. Nonsense mutations create a premature stop signal in the translation of the genetic code contained in mRNA and prevent the production of full-length, functional proteins. The absence or overproduction of specific proteins can cause disease. We apply proprietary technologies and our extensive knowledge of post-transcriptional control processes in our drug discovery and development activities. We discovered ataluren by applying our technologies to identify molecules that promote or enhance the suppression of nonsense mutations.

In addition, we have a pipeline of product candidates that are in preclinical development. Our preclinical and discovery programs are focused on the development of new treatments for multiple therapeutic areas, including neuromuscular disease, oncology and infectious disease. We have discovered all of our compounds currently under development using our proprietary technologies. We plan to develop these compounds both on our own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies.

Ataluren

Ataluren is a novel, orally administered small-molecule compound that targets nonsense mutations. We are developing ataluren for the treatment of genetic disorders in which a nonsense mutation is the cause of the disease. Genetic tests are available for many genetic disorders, including Duchenne muscular dystrophy and cystic fibrosis, to determine if the underlying cause is a nonsense mutation.

We believe that ataluren interacts with the ribosome, which is the component of the cell that decodes the mRNA molecule and manufactures proteins, to enable the ribosome to read through premature nonsense stop signals on mRNA and allow the cell to produce a full-length, functional protein. We believe that a drug with a mechanism of action that allows the ribosome to read through premature stop signals without affecting the normal termination of protein synthesis may be able to overcome the effects of nonsense mutations.

Ataluren is administered orally as granules mixed with permitted liquids or semi-solid foods, such as milk, water, applesauce or yogurt. We designed this formulation because children comprise a significant portion of the patient population for ataluren and often have difficulty swallowing pills or capsules. Ataluren is manufactured in reliable and reproducible synthetic processes from readily available starting materials.

Ataluren for nmDMD

Muscular dystrophies are genetic disorders involving progressive muscle wasting and weakness. Duchenne muscular dystrophy is the most common and one of the most severe types of muscular dystrophy. Duchenne muscular dystrophy occurs when a mutation in the dystrophin gene prevents the cell from making a functional dystrophin protein. Based on information from the American Journal of Medical Genetics, we estimate that a nonsense mutation is the cause of Duchenne muscular dystrophy in approximately 13% of patients, or approximately 2,000 patients in the United States and 2,500 patients in the European Union. There is currently no marketed therapy approved for the treatment of the underlying cause of Duchenne muscular dystrophy. Currently available treatments for Duchenne muscular dystrophy are only palliative.

We have initiated our confirmatory Phase 3 ACT DMD clinical trial to evaluate the efficacy and safety of ataluren in patients with nmDMD. This is a multicenter, randomized, double-blind, placebo controlled

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Phase 3 clinical trial. We dosed the first patient in this trial in April 2013, with enrollment expected to be completed in mid-2014. We plan to conduct this trial in approximately 220 patients at investigational sites worldwide.

The primary objective of this trial is to evaluate the effect of ataluren on ambulation as measured by mean change in distance walked during a 6-minute walk test, which we refer to as 6-minute walk distance. Based on our estimates regarding patient enrollment, we expect to complete this trial and have initial, top-line data available in mid-2015.

The trial protocol specifies the following key inclusion criteria for patients enrolling in this trial:

- the patient must be seven through 16 years of age;
- at baseline, the patient must walk no more than 80% of predicted 6-minute walk distance compared to healthy boys matched for age and height, but have the ability to walk at least 150 meters during the 6-minute walk test; and
- the patient must have used systemic corticosteroids for a minimum of six months prior to the start of treatment.

The study population and outcome measures that we are using in our confirmatory Phase 3 ACT DMD clinical trial are based on, and reflect our analysis of the results of, our completed Phase 2b clinical trial of ataluren for the treatment of nmDMD, including data regarding disease progression, referred to as natural history data, and a post-hoc, retrospective subgroup analysis of patients who would meet the enrollment criteria for our confirmatory Phase 3 ACT DMD clinical trial. This retrospective subgroup analysis showed a much larger treatment effect in mean change in 6-minute walk distance between ataluren and placebo in this subgroup than in the overall population included in the Phase 2b clinical trial. In light of this natural history data and retrospective subgroup analysis, our confirmatory Phase 3 ACT DMD clinical trial is focusing on patients in the decline phase of the disease based on age and baseline 6-minute walk distance. The intent of focusing on patients in the decline phase of the disease is to enhance the demonstration of ataluren's effect to slow decline in walking ability. In addition, we believe that by only enrolling patients who are treated with systemic corticosteroids, the variability of 6-minute walk distance results will be reduced.

In October 2012, we submitted an MAA to the EMA for conditional approval of ataluren for the treatment of nmDMD. During the review process, the EMA informed us of major objections that would preclude a recommendation for marketing authorization unless adequately addressed. These major objections related to, among other things, the EMA's views regarding insufficient evidence of efficacy based on our single Phase 2b clinical trial, resulting in a negative risk-benefit balance for purposes of conditional approval, and uncertainties about the effective dose. The EMA also questioned whether our confirmatory Phase 3 ACT DMD clinical trial could be completed if the EMA granted conditional approval. In December 2013, the EMA convened a scientific advisory group, or SAG, meeting as part of the regulatory review process followed by the oral explanation meeting with the CHMP. We believe that both the SAG and oral explanation meetings allowed us and independent experts in the DMD field to provide information to the SAG and CHMP members about important aspects of our clinical data and trial design.

In January 2014, the CHMP adopted a negative opinion recommending the refusal of the granting of the conditional marketing authorization for ataluren for the treatment of nmDMD. The CHMP stated that a principal reason for the negative opinion was that the prior Phase 2b clinical trial had failed to demonstrate in the primary analysis that patients taking ataluren could walk a greater distance in six minutes than patients taking placebo, the primary endpoint. Additionally, the CHMP noted that other

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measures of efficacy provided only limited supportive evidence of the beneficial effects of ataluren. The CHMP acknowledged in communication to us that the post hoc analyses that we presented to the CHMP were performed in line with the most current knowledge about the natural history of the disease and that our definition of the subgroups in the analyses were both clinically and scientifically justified. However, the CHMP concluded that we did not provide sufficiently compelling evidence of efficacy to justify conditional approval. In addition, the CHMP considered that we had not provided sufficient data to determine how ataluren works in the body and how its effects change with dose. Finally, the CHMP expressed concern that the conduct of the confirmatory Phase 3 ACT DMD trial might be affected by the availability of an authorized product and therefore potentially jeopardize the feasibility of completing the trial. Therefore, despite divergent minority positions, the CHMP concluded a favorable risk-benefit balance could not be established at the time of their meeting and adopted a negative opinion. We have requested a re-examination of the CHMP opinion. Based upon the timelines for a re-examination process, we believe that our confirmatory Phase 3 ACT DMD clinical trial will be substantially enrolled at the time the CHMP would consider a revision of their initial opinion as part of the re-examination process.

We continue to believe that completion of our confirmatory Phase 3 ACT DMD clinical trial and submission of data to the regulatory authorities is the more likely path to obtain marketing approval of ataluren. There is substantial risk that the EMA will not grant us conditional approval upon re-examination of the original CHMP negative opinion. If granted, EMA conditional approval would permit us to market ataluren in the European Union for treatment of nmDMD prior to completion of our confirmatory Phase 3 ACT DMD clinical trial. We plan to complete our confirmatory Phase 3 ACT DMD clinical trial before applying for marketing approval from the FDA. In designing our confirmatory Phase 3 ACT DMD clinical trial for the treatment of nmDMD, we have sought to reflect the views expressed by both the EMA and the FDA in our discussions with these regulatory authorities. We expect that these trial results, if favorable, could serve as the basis for full approval by the EMA and the FDA of ataluren for the treatment of nmDMD. If the trial results are favorable, and based on our estimates of patient enrollment and data availability, we expect to be able to submit applications for full marketing approval of ataluren for the treatment of nmDMD in both the European Union and the United States in 2016.

Ataluren for nmCF

Cystic fibrosis is among the most common life-threatening genetic disorders worldwide. Cystic fibrosis is caused by defects in a single gene known as the cystic fibrosis transmembrane conductance regulator, or CFTR. Based on information from the Cystic Fibrosis Foundation, we estimate that nonsense mutations are the cause of cystic fibrosis in approximately 10% of patients, or approximately 3,000 patients in the United States and approximately 3,700 to 4,200 patients in the European Union. There is currently no marketed therapy approved to correct defective CFTR production and function in patients with nmCF. For nmCF patients, available treatments do not address the underlying cause of the disease and are designed only to alleviate the symptoms of the disease.

We are planning a multicenter, randomized, double-blind, placebo controlled Phase 3 clinical trial to evaluate the efficacy and safety of ataluren in approximately 210 patients with cystic fibrosis caused by a nonsense mutation as confirmed by gene sequencing. We expect that the primary objective of this trial will be to evaluate the effect of ataluren on pulmonary function as measured by relative change in percent of predicted forced expiratory volume in one second, or FEV_1. FEV_1 is a measure of the volume of air that has been exhaled at the end of the first second of forced expiration. Percent of predicted FEV_1, or %-predicted FEV_1, is based on a comparison to healthy individuals matched for age, height and gender. Based on our

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estimates regarding initiation of the trial and patient enrollment, we expect to complete this trial and have initial, top-line data available in 2016.

We expect to require that patients in this trial be at least six years of age and have %-predicted FEV_1 within a specified range, sweat chloride in excess of a specified level as evidence of the severity of the disease and documentation of a nonsense mutation in at least one copy of the CFTR gene. We expect to exclude patients from the trial if, among other reasons, they are receiving chronic inhaled aminoglycoside antibiotics.

We selected the enrollment criteria for our confirmatory Phase 3 clinical trial in part based on a subgroup analysis of patients not receiving inhaled aminoglycoside antibiotics in our completed Phase 3 clinical trial for the treatment of nmCF. We believe that the inhaled antibiotic tobramycin interfered with ataluren's mechanism of action. For the subgroup of patients not receiving chronic inhaled aminoglycoside antibiotics, there was a substantial difference in mean relative changes from baseline in %-predicted FEV_1 at the end of the trial favoring ataluren in comparison with placebo. In contrast, patients that received chronic inhaled aminoglycoside antibiotics and ataluren did not exhibit a difference compared to patients that received chronic inhaled aminoglycoside antibiotics and placebo.

We have received scientific advice from the EMA regarding the possibility of submitting an MAA for conditional approval of ataluren for the treatment of nmCF and the protocol design of a post-approval confirmatory trial. There is substantial risk that the EMA will not grant us conditional approval of ataluren for the treatment of nmCF. We had interactions with the FDA in 2013 regarding the clinical development design which would have the potential to support an NDA, but we did not achieve a consensus between the EMA and FDA views. While we have incorporated feedback from the FDA into our proposed trial design, we believe that certain key recommendations from the FDA are not appropriate. Two of the key recommendations that we are in disagreement with are the designation of FEV1, CF pulmonary exacerbations and body mass index as three co-primary endpoints for the trial and a suggested three-year trial duration. We plan to make FEV1 the primary endpoint with CF pulmonary exacerbations and body mass index key secondary endpoints, which is consistent with other clinical trials currently ongoing in cystic fibrosis and FDA's earlier recommendation. Additionally, we believe that extending the study duration to three years would result in a number of complications that would ultimately limit the robustness of the data and conclusions that could be drawn from the results. Based on these interactions, we nonetheless intend to proceed with our confirmatory Phase 3 clinical trial of ataluren in nmCF in the first half of 2014 consistent with feedback from the EMA on our trial design.

Our strategy

Our goal is to become a leading biopharmaceutical company focused on discovering, developing and commercializing small-molecule therapeutics that target post-transcriptional control processes and address disorders, particularly in the orphan and ultra-orphan areas, with high unmet medical needs. To achieve our goal, we are pursuing the following strategies:

- Complete clinical development and seek marketing approvals for ataluren for the treatment of nmDMD and nmCF.

- Commercialize ataluren through our own focused, specialized sales force initially in the European Union and the United States and, eventually, in other key territories.

- Explore additional, strategically attractive indications for ataluren based on the large number of genetic disorders caused by nonsense mutations.

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Advance the development of our preclinical product candidates and discover and develop additional small molecules that alter post-transcriptional control processes in a broad range of indications.

Seek third party grants and support and selectively establish strategic alliances.

Risks associated with our business

Our business is subject to a number of risks of which you should be aware before making an investment decision. These risks are discussed more fully in the "Risk factors" section of this prospectus immediately following this prospectus summary. These risks include, among others, the following:

We currently depend heavily on the success of ataluren. Our ability to generate product revenues, which may not occur for several years, if ever, will depend heavily on the successful development and commercialization of ataluren for either or both of nmDMD and nmCF. There is substantial risk that the EMA will not grant us conditional approval of ataluren for the treatment of either nmDMD or nmCF.

Clinical trials of ataluren or any of our other product candidates may not be successful. For example, we did not achieve the primary efficacy endpoint with the pre-specified level of statistical significance in a Phase 2b clinical trial of ataluren for the treatment of nmDMD that we completed in 2009 or in a Phase 3 clinical trial of ataluren for the treatment of nmCF that we completed in 2011. If we are unable to obtain required marketing approvals for, commercialize, obtain and maintain patent protection for or gain market acceptance by physicians, patients and third-party payors of ataluren or any of our other product candidates, or experience significant delays in doing so, our business will be materially harmed and our ability to generate revenue will be materially impaired.

Our scientific approach focusing on the discovery and development of product candidates that target post-transcriptional control processes is unproven and may not result in the development of commercially viable drugs that safely and effectively treat genetic disorders or other diseases.

Our current and any future collaborations with third parties for the development and commercialization of our product candidates may not be successful.

We have a limited operating history. We currently have no commercial products and we have not received marketing approval for any product candidate.

We have incurred significant operating losses since inception and may need substantial additional funding. We expect to incur significant expenses and increasing operating losses for at least the next several years. As of September 30, 2013, we had an accumulated deficit of $310.9 million.

Our corporate information

Our executive offices are located at 100 Corporate Court, South Plainfield, New Jersey 07080, and our telephone number is (908) 222-7000. Our website address is *www.ptcbio.com*. The information contained on, or that can be accessed through, our website is not a part of this prospectus. We have included our website address in this prospectus solely as an inactive textual reference.

In this prospectus, unless otherwise stated or the context otherwise requires, references to "PTC," "PTC Therapeutics," "we," "us," "our" and similar references refer to PTC Therapeutics, Inc. and, where appropriate, its subsidiaries. The trademarks, trade names and service marks appearing in this prospectus are the property of their respective owners.

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Implications of being an emerging growth company

As a company with less than $1 billion in revenue during our last fiscal year, we qualify as an "emerging growth company" as defined in the Jumpstart Our Business Startups Act of 2012, or the JOBS Act, and we may remain an emerging growth company until December 31, 2018, subject to satisfaction of certain conditions. For so long as we remain an emerging growth company, we are permitted and intend to rely on exemptions from certain disclosure and other requirements that are applicable to other public companies that are not emerging growth companies. In particular, in this prospectus, we have provided only two years of audited financial statements and have not included all of the executive compensation related information that would be required if we were not an emerging growth company. Accordingly, the information contained herein may be different than the information you receive from other public companies in which you hold stock.

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The offering

Common stock offered by us	$75,000,000 of shares of our common stock.
Common stock to be outstanding after this offering	28,208,762 shares
Over-allotment option	The underwriters have an option for a period of 30 days to purchase up to $11,250,000 of additional shares of our common stock to cover over-allotments.
Use of proceeds	We intend to use the net proceeds from this offering to fund the clinical development of and seek marketing approval for ataluren for the treatment of nmDMD, to fund the clinical development of and seek marketing approval for ataluren for the treatment of nmCF, to fund pre-approval commercial efforts for ataluren, to fund research and development of ataluren for additional indications and for our earlier stage programs, and for working capital and other general corporate purposes. See "Use of proceeds" for more information.
Risk factors	You should read the "Risk factors" section of this prospectus for a discussion of factors to consider carefully before deciding to invest in shares of our common stock.
NASDAQ Global Select Market symbol	"PTCT"

The number of shares of our common stock to be outstanding after this offering is based on 24,913,508 shares of our common stock outstanding as of January 28, 2014 and assumes the issuance and sale of $75,000,000 of shares of our common stock at an assumed public offering price of $22.76 per share, which is the last sale price of our common stock, as reported on the NASDAQ Global Select Market on February 7, 2014. The number of shares of our common stock to be outstanding after the closing of this offering excludes:

- 3,025,394 shares of our common stock issuable upon the exercise of stock options outstanding as of January 28, 2014, at a weighted-average exercise price of $22.33 per share;
- 15,160 shares of our common stock issuable upon the exercise of warrants outstanding as of January 28, 2014, at a weighted-average exercise price of $199.32 per share; and
- 163,661 shares of our common stock available for future issuance, as of January 28, 2014, under our 2013 long term incentive plan.

Unless otherwise indicated, all information in this prospectus assumes:

- no exercise of the outstanding stock options or warrants described above; and
- no exercise by the underwriters of their option to purchase up to $11,250,000 of additional shares of our common stock to cover over-allotments.

Celgene European Investment Company LLC, or CEIC, one of our existing investors, has indicated an interest in purchasing up to approximately $3.2 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, CEIC may determine to purchase fewer shares than it has indicated an interest in purchasing or not to purchase any shares in this offering. In addition, the underwriters could determine to sell fewer shares to CEIC than it has indicated an interest in purchasing or not to sell any shares to CEIC. The underwriters will receive the same underwriting discount on any shares purchased by CEIC as they will on any other shares sold to the public in this offering.

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Summary financial data

You should read the following summary financial data together with our financial statements and the related notes appearing elsewhere in this prospectus and the "Selected financial data" and "Management's discussion and analysis of financial condition and results of operations" sections of this prospectus. We have derived the statements of operations data for the years ended December 31, 2011 and 2012 from our audited financial statements included in this prospectus. We have derived the statements of operations data for the nine months ended September 30, 2012 and 2013 and the balance sheet data as of September 30, 2013 from our unaudited financial statements included in this prospectus. The unaudited financial data include, in the opinion of our management, all adjustments, consisting of normal recurring adjustments, that are necessary for a fair statement of our financial position and results of operations for these periods. Our historical results for any prior period are not necessarily indicative of results to be expected in any future period, and our results for any interim period are not necessarily indicative of results to be expected for a full fiscal year.

Statement of operations data	**Year ended December 31,**		**Nine months ended September 30,**	
(in thousands, except share and per share data)	**2011**	**2012**	**2012**	**2013**
Revenues:				
Collaboration revenue	$ 98,961	$ 28,779	$ 22,861	$ 27,395
Grant revenue	6,451	5,167	4,445	2,890
Total revenues	105,412	33,946	27,306	30,285
Operating expenses:				
Research and development	58,677	46,139	36,689	39,855
General and administrative	16,153	14,615	11,391	17,735
Total operating expenses	74,830	60,754	48,080	57,590
Income (loss) from operations	30,582	(26,808)	(20,774)	(27,305)
Interest income (expense), net	(2,444)	(1,210)	(1,007)	(6,250)
Loss on extinguishment of debt				(130)
Other income, net	461	1,783	1,818	(3)
Income (loss) before tax benefit	28,599	(26,235)	(19,963)	(33,688)
Income tax benefit	2,306			
Net income (loss)	30,905	(26,235)	(19,963)	(33,688)
Deemed dividend				(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization		159,954	159,954	3,391
Less beneficial conversion charge		(378)	(378)	
Net income (loss) attributable to common stockholders	$ 30,905	$ 133,341	$ 139,613	$ (48,546)
Net income (loss) per share(1)				
Basic	$ 23.95	$ 219.76	$ 182.41	$ (5.40)
Diluted	$ 4.55	$ 42.50	$ 39.41	$ (5.40)
Weighted-average shares outstanding:				
Basic	1,089	3,328	2,937	8,995,167

Diluted	5,729	17,205	13,593	8,995,167

(1) See Note 8 to our audited financial statements appearing at the end of this prospectus regarding the calculation of net income per share.

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Balance sheet data	**September 30, 2013**	
(in thousands)	**Actual**	**As adjusted(1)**
Cash, cash equivalents and marketable securities		