

**UNITED STATES  
SECURITIES AND EXCHANGE COMMISSION  
WASHINGTON, D.C. 20549**

**FORM 8-K**

**CURRENT REPORT**

**Pursuant to Section 13 or 15(d) of the Securities Exchange Act of 1934**

Date of Report (Date of earliest event reported): **April 22, 2016**

**PTC THERAPEUTICS, INC.**

(Exact Name of Company as Specified in Charter)

**Delaware**  
(State or Other Jurisdiction  
of Incorporation)

**001-35969**  
(Commission  
File Number)

**04-3416587**  
(IRS Employer  
Identification No.)

**100 Corporate Court**  
**South Plainfield, NJ**  
(Address of Principal Executive Offices)

**07080**  
(Zip Code)

Company's telephone number, including area code: **(908) 222-7000**

**Not applicable**

(Former Name or Former Address, if Changed Since Last Report)

Check the appropriate box below if the Form 8-K filing is intended to simultaneously satisfy the filing obligation of the registrant under any of the following provisions ( see General Instruction A.2. below):

- Written communications pursuant to Rule 425 under the Securities Act (17 CFR 230.425)
- Soliciting material pursuant to Rule 14a-12 under the Exchange Act (17 CFR 240.14a-12)
- Pre-commencement communications pursuant to Rule 14d-2(b) under the Exchange Act (17 CFR 240.14d-2(b))
- Pre-commencement communications pursuant to Rule 13e-4(c) under the Exchange Act (17 CFR 240.13e-4(c))

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**Item 5.02 Departure of Directors or Certain Officers; Election of Directors; Appointment of Certain Officers; Compensatory Arrangements of Certain Officers.**

On April 26, 2016, PTC Therapeutics, Inc. (the "Company") announced that effective as of April 22, 2016, Dr. Spiegel stepped down from his role of full-time Chief Medical Officer and transitioned to a consultant role for the Company for an initial period of two years at a rate of \$2,500 per day, for a minimum of \$300,000 per year, payable monthly. Dr. Spiegel's consulting agreement replaces his employment agreement with the Company. The foregoing description of the consulting agreement, which is filed herewith as Exhibit 10.1 and is incorporated herein by reference, is qualified in its entirety by reference to its terms.

**Item 7.01 Regulation FD Disclosure.**

On April 26, the Company issued a press release announcing Dr. Spiegel's transition and the promotion of Dr. Tuyen Ong, the Company's Senior Vice President, Head of Clinical and Translational Research, to the role of Chief Medical Officer. A copy of the press release that discusses these matters is filed as Exhibit 99.1 and is incorporated by reference in Item 7.01 of this Current Report on Form 8-K.

The information contained in Item 7.01 of this Current Report on Form 8-K, including Exhibit 99.1, is furnished pursuant to, and shall not be deemed to be "filed" for the purposes of, Section 18 of the Securities Exchange Act of 1934, as amended, or otherwise subject to the liabilities of that section. The information contained in Item 7.01 of this Current Report shall not be incorporated by reference into any registration statement or any other document filed pursuant to the Securities Act of 1933, as amended, except as otherwise expressly stated in such filing.

**Item 9.01. Financial Statements and Exhibits.**

(d) Exhibits:

- 10.1 Consulting Services Agreement
- 99.1 Press Release dated April 26, 2016

**SIGNATURES**

Pursuant to the requirements of the Securities Exchange Act of 1934, the registrant has duly caused this report to be signed on its behalf by the undersigned hereunto duly authorized.

PTC THERAPEUTICS, INC.

Date: April 27, 2016

By: /s/ Shane Kovacs  
Shane Kovacs  
Chief Financial Officer

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**EXHIBIT INDEX**

| <u>Exhibit No.</u> | <u>Description</u>                 |
|--------------------|------------------------------------|
| 10.1               | Consulting Services Agreement      |
| 99.1               | Press Release dated April 26, 2016 |

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## PTC SERVICES AGREEMENT

This Agreement is made as of April 22, 2016 (the "Effective Date") between PTC Therapeutics, Inc. ("PTC") and Spiegel Consulting LLC (the "Consultant").

WHEREAS, Robert J. Spiegel M.D., FACP ("Dr. Spiegel"), Consultant's principal, has served as an employee of PTC until the Effective Date, under the Employment Agreement dated as of January 23, 2014 between PTC and the Consultant (the "Employment Agreement");

WHEREAS, the parties mutually desire to transition Dr. Spiegel to a consultant role for PTC as of the Effective Date, in accordance with Section 2.e. of the Employment Agreement, and so the Employment Agreement is being terminated by separate document effective as of the Effective Date of this Agreement; and

WHEREAS, the purpose of this Agreement is to confirm the parties' understanding with respect to (i) Consultant's rendering consulting services to PTC in exchange for the consideration as further described in this Agreement, and (ii) Consultant's obligations to protect information and property which is confidential and proprietary to PTC.

NOW THEREFORE, PTC and Consultant agree as follows:

### SERVICES

Consultant shall perform the following services for PTC (the "Services"), ensuring that Dr. Spiegel will be available and perform services on a schedule equivalent to 2.5 full-time days per week (adjustable subject to the good-faith agreement of the parties), to fulfill all obligations of Consultant hereunder:

Serve as an advisor/consultant for PTC, performing tasks reasonably requested by PTC in connection with his area of expertise, including without limitation:

- Represent PTC in meetings with health and regulatory authorities, scientific meetings, and KOL interactions.
- Support PTC's DMD & CF regulatory filing and launch activities.
- Work with PTC's Med Affairs, Commercial, and Business Development groups to identify high impact activities.
- Continue to 'mentor' and support Tuyen Ong as CMO
- Continue to Chair PTC's PTC596 Team and serve as PTC's internal oncology expert.
- Provide input on and assist with execution of PTC's clinical and regulatory strategy related to Translarna™ (ataluren).

Consultant shall also provide such other services as the parties may mutually agree.

Consultant represents that Dr. Spiegel is capable of properly performing the Services.

### COMPENSATION

As Consultant's total cash compensation for Consultant's Services hereunder, PTC shall pay Consultant \$25,000 per month (adjustable pro rata if the days per week are adjusted per the "SERVICES" section above), payable in advance on the date hereof and on each successive monthly anniversary thereof. In the event Consultant works more than 10 full-time equivalent days per month, then additional compensation shall be paid at the rate of \$2,500 per day (pro-rated for partial days) above 10. In addition, the options to purchase common stock of PTC issued to Dr. Spiegel prior to the Effective Date based on his prior services as an employee of PTC shall be treated as set forth on Schedule A hereto. Consultant shall remain eligible for further option grants as and when determined by the Company.

In addition to the foregoing amount, PTC shall pay Consultant for (i) all reasonable and necessary travel expenses (other than ordinary commuting expenses) incurred by Consultant at the prior request of PTC in providing the Services, and (ii) other expenses pre-approved in writing by an authorized PTC representative that are necessary to performance of the Services. Payment for such expenses shall be made to Consultant within 45 days of receipt by PTC of invoices and receipts substantiating such expenses and detailing the services to which they relate, and PTC shall then have no further obligation to Consultant for such expenses.

Consultant shall keep or cause to be kept full, timely and accurate records in reasonable form and detail and to which PTC and its designated employees, agents or representatives shall have access at any reasonable time for auditing purposes. When requested by PTC, Consultant shall be required to report on the status of its work on the Services in a manner satisfactory to PTC.

### NO CONFLICTS

Throughout the term of this Agreement the parties hereto agree that the type of services to be rendered hereunder by Consultant are not exclusive to PTC. Consultant, during the term of this Agreement, may accept from others, concurrent consulting work provided that such consulting work does not in any way interfere with the Services Consultant is required to perform under this Agreement and is not a breach of any of Consultant's obligations under this Agreement. Notwithstanding the foregoing, Consultant represents that (i) Dr. Spiegel has not, and will not during the term of this Agreement, enter into any agreement or relationship that would interfere with or prevent his performance under this Agreement, (ii) by entering into and performing this Agreement, Dr. Spiegel is not and will not be violating any agreement or arrangement with any third parties, and (iii) that during the term of this Agreement Dr. Spiegel will not solicit

or divert business, customers or employees of PTC on behalf of Consultant, himself or any other business.

## **CONFIDENTIALITY**

(a) In providing consulting services to PTC pursuant to this Agreement, Consultant may acquire information that pertains to PTC's products, processes, equipment, programs, development efforts, therapeutic targets, compounds, assays, know-how, or plans ("Proprietary Information"). Consultant agrees not to disclose any Proprietary Information to third parties or to use any Proprietary Information for any purpose other than performance of the Services.

(b) Proprietary Information subject to the foregoing paragraph does not include information Consultant can demonstrate: (i) is or later becomes available to the public through no breach of this Agreement by Consultant; (ii) is obtained by Consultant from a third party who Consultant had a reasonable basis to believe had the legal right to disclose the information to Consultant; (iii) is already in the possession of Consultant on the date this Agreement becomes effective; or (iv) is required to be disclosed by law, government regulation, or court order; provided, however, that Consultant shall use its best efforts to provide PTC with notice and an opportunity to oppose or limit such disclosure.

(c) Without limiting the foregoing, Consultant agrees that it has only disclosed and will only disclose confidential information to employees and principals of Consultant who have a need to know such confidential information for the sole purpose of providing Services to PTC under this Agreement. Prior to such disclosure, Consultant shall advise and require such employees and principals to be bound by the terms of this Agreement. Consultant shall be liable for any breach of this Agreement by its employees or agents.

(d) Consultant to PTC shall not disclose to PTC any confidential information of other parties without the prior written consent of PTC.

(e) Consultant agrees to promptly return, upon request by PTC, all materials in Consultant's possession that were either (i) supplied by PTC or its representatives in conjunction with the Services or (ii) generated by Consultant in the performance of the Services and contain or reference Proprietary Information. This obligation of return of materials is in addition to, and shall not be construed to limit, any deliverables with respect to the Services.

## **INTELLECTUAL PROPERTY**

(a) Consultant hereby assigns to PTC any right, title, and interest Consultant may have in any know-how, invention, discovery, improvement, or other intellectual property which Consultant develops during the course of and as a direct result of performing the Services. Any intellectual property assignable to PTC pursuant to the preceding sentence is hereinafter referred to as "PTC Intellectual Property". Upon the request of PTC, Consultant shall (i) provide such documentation relating to any PTC Intellectual Property, and (ii) execute such further assignments, documents, and other instruments, as may be necessary to assign PTC Intellectual Property to PTC and to assist PTC in applying for, obtaining and enforcing patents or other rights in the United States and in any foreign country with respect to any PTC Intellectual Property. Consultant will ensure that Dr. Spiegel executes and delivers the Inducement Letter in the form attached as Exhibit A hereto, and will provide a signed copy thereof to PTC. PTC will bear the cost of preparation of all patent or other applications and assignments, and the cost of obtaining and enforcing all patents and other rights to PTC Intellectual Property. Consultant hereby designates PTC as agent, and grants to PTC a power of attorney with full power of substitution, for the purpose of effecting the foregoing assignments. Consultant agrees not to publish any PTC Intellectual Property without the prior written consent of PTC.

## **INDEMNIFICATION**

The Indemnification Agreement dated as of January 23, 2014 between PTC and Dr. Spiegel shall continue in full force and effect during the Term of this Agreement, with respect to all activities in connection with this Agreement.

## **TERM AND TERMINATION**

(a) Unless terminated earlier pursuant to the following paragraph, this Agreement shall terminate two years from the Effective Date (the "Term") or such lesser period as PTC and the Consultant may mutually agree. The parties may extend the Term by mutual written agreement.

(b) PTC may terminate this Agreement for Cause and Consultant may terminate this Agreement for Good Reason, each as defined in the Employment Agreement (but with the references in such definition deemed to apply to this Agreement in lieu of the Employment Agreement), in each cause on written notice to the other party.

(c) Termination of this Agreement under this section shall not affect (i) PTC's obligation to pay for services previously performed by Consultant or expenses reasonably incurred by Consultant for which Consultant is entitled to reimbursement under this Agreement and PTC's continuing obligation to Consultant under the Indemnification section of this Agreement, or (ii) Consultant's continuing obligations to PTC under the Confidentiality, Intellectual Property, Term and Termination, and Miscellaneous sections of this Agreement. However, Consultant shall not be entitled to any continuing compensation of any nature following termination.

## **MISCELLANEOUS**

(a) Failure of any party to insist, in one or more instances, on performance by the other in strict accordance with the terms and conditions of this Agreement shall not be deemed a waiver or relinquishment of any right granted hereunder or of the future performance of any such term or condition or of any other term or condition of this Agreement, unless such waiver is contained in a writing signed by or on behalf of the waiving party.

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(b) This Agreement shall be governed by and construed and enforced in accordance with the laws of the State of New Jersey, without application of the conflicts of law provisions thereof.

(c) PTC may assign its rights and obligations hereunder (i) to any person or entity who succeeds to all or substantially all of PTC's business or that aspect of PTC's business in which Consultant is principally involved, or (ii) to any affiliate wholly-owned by or under common control with PTC. Consultant's rights and obligations under this Agreement are of a personal nature and therefore may not be assigned without the prior written consent of PTC.

(d) This Agreement shall inure to the benefit of and be binding upon the respective heirs, executors, successors, representatives, and authorized assigns of the parties, as the case may be.

(e) The relationship created by this Agreement shall be that of third party contractor, and, as such, the Consultant shall be responsible for the payment of all taxes including, but not limited to, social security and income tax relating to the rendering of the Services and compensation paid to the Consultant pursuant to the terms of this Agreement. Consultant shall not be entitled to any rights, benefits, or privileges of PTC employees, nor shall Consultant have any authority to bind or act as agent for PTC or its employees for any purpose. Consultant shall be liable for any breach of this Agreement by its employees, principals, or agents.

(f) Consultant acknowledges that any breach or threatened breach of the terms or conditions of this Agreement will result in substantial, continuing and irreparable injury to PTC. Therefore, Consultant agrees that, in addition to any other remedy that may be available to PTC, PTC shall be entitled to injunctive or other equitable relief by a court of appropriate jurisdiction in the event of any breach or threatened breach of this Agreement, and Consultant waives any requirements relating to the posting of bond or other surety in connection with such injunctive or equitable relief.

(g) Notice or payments given by one party to the other hereunder shall be in writing and deemed to have been properly given or paid if deposited with the United States Postal Service, registered or certified mail, addressed as follows:

PTC:

PTC Therapeutics, Inc.  
Attention: Legal Department  
100 Corporate Court  
Middlesex Business Center  
South Plainfield NJ 07080

with an email copy to: legal@ptcbio.com

Consultant:

Spiegel Consulting

Robert J. Spiegel  
400 Elm St.  
Westfield, CT 07090

(i) Except as expressly set forth herein, this Agreement replaces all previous agreements and the discussions relating to the subject matters hereof and constitutes the entire agreement between PTC and Consultant with respect to the subject matters of this Agreement; provided, however, that any stock options provided to Consultant's employees and/or principals as compensation for the Services shall be governed exclusively by the terms of a separate stock option certificate and the applicable PTC equity plan documents. This Agreement may not be modified in any respect by any verbal statement, representation, or agreement made by any employee, officer, or representative of PTC, or by any written documents unless it is signed by an officer of PTC and by an authorized representative of Consultant.

Notwithstanding the foregoing, and notwithstanding the termination of the Employment Agreement, Dr. Spiegel shall continue to comply with his post-employment covenants to PTC under the Employment Agreement, including those set forth in Sections 5 and 6 thereof, and any failure to do so shall be a basis for termination of this Agreement by PTC at its election.

j) If any term or provision of this Agreement is deemed invalid, contrary to, or prohibited under applicable laws or regulations of any jurisdiction, the remainder of this Agreement shall remain in effect, and the relevant term or provision shall be limited to the maximum permissible extent.

IN WITNESS WHEREOF, the parties have executed this Agreement effective the date first stated above.

**Spiegel Consulting LLC**

/s/ Robert J. Spiegel

\_\_\_\_\_  
Robert J. Spiegel  
SSN/Tax ID:

**PTC THERAPEUTICS, INC.**

/s/ Stuart Peltz

\_\_\_\_\_  
Stuart Peltz  
CEO

Reference is hereby made to that certain PTC Services Agreement (the “**Agreement**”), dated as of April 22, 2016, between PTC Therapeutics, Inc., (“PTC”), and Spiegel Consulting LLC (“SC”), pursuant to which SC agreed to cause Dr. Robert J. Spiegel (“**me**,” “**my**” and “**I**”) to provide certain the Services (as defined in the Agreement).

A. I am familiar with all of the terms, covenants and conditions of the Agreement and I hereby consent to the execution thereof. I shall perform and comply with all of the terms, covenants, conditions and obligations of the Agreement in so far as they relate to me, even if the employment between me and SC should hereafter expire, terminate or be suspended. I hereby confirm all grants, representations, warranties and agreements made by SC under the Agreement, and I hereby assign to SC any right, title, and interest I may have in any know-how, invention, discovery, improvement, or other intellectual property which I may develop during the course of and as a direct result of performing the Services.

B. Unless I am deemed substituted for SC as a direct party to the Agreement pursuant to Paragraph C below, I shall look solely to SC and not to PTC for the payment of compensation for my services and for the discharge of all other obligations of my employer with respect to my services under the Agreement.

C. If SC or its successors in interest should be dissolved or should otherwise cease to exist, I shall, at PTC’s election, be substituted for SC for the balance of the term of the Agreement upon the terms, covenants and conditions set forth therein.

D. If PTC shall serve SC with any notices, demands or instruments relating to the Agreement, or to the rendition of my services thereunder, service upon SC shall also constitute service upon me.

E. For purposes of any and all workers’ compensation statutes, laws or regulations (“**Workers’ Compensation**”), I acknowledge that an employment relationship exists between SC and me.

IN WITNESS WHEREOF, I have executed this inducement letter as of the date first written above.

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Dr. Robert J. Spiegel

#### **Schedule A**

##### **COMPREHENSIVE LIST AND TREATMENT OF STOCK OPTIONS**

#### **A. Comprehensive List of Stock Options of Dr. Spiegel (collectively, the “Stock Options”):**

(i) stock options to purchase 250 shares granted prior to May 15, 2013 with an exercise price of \$490.80 per share, comprised of:

- (x) 250 which have vested but remained unexercised;
- (y) 0 which have vested and been exercised; and
- (z) 0 which remain unvested.

(ii) stock options to purchase 25,000 shares granted on May 15, 2013 with an exercise price of \$10.85 per share, comprised of:

- (x) 18,750 which have vested but remained unexercised;
- (y) 0 which have vested and been exercised; and
- (z) 6,250 which remain unvested.

(iii) stock options to purchase 150,000 shares granted on February 27, 2014 with an exercise price of \$33.97 per share, comprised of:

- (x) 75,000 which have vested but remained unexercised;
- (y) 0 which have vested and been exercised; and
- (z) 75,000 which remain unvested.

(iv) stock options to purchase 10,000 shares granted on January 2, 2015 with an exercise price of \$51.00 per share, comprised of:

- (x) 10,000 which have vested but remained unexercised;
- (y) 0 which have vested and been exercised; and
- (z) 0 which remain unvested.

(v) stock options to purchase 10,000 shares granted on January 4, 2016 with an exercise price of \$30.86 per share, comprised of:

- (x) 0 which have vested but remained unexercised;
- (y) 0 which have vested and been exercised; and
- (z) 10,000 which remain unvested.

#### **B. Treatment of Stock Options:**

(i) *continued vesting:*

From the Effective Date through and including the date this Agreement is terminated in accordance with its terms, PTC shall allow the Stock Options which are currently unvested to continue to vest per the regular schedule based on his continuity of services under this Agreement.

**(ii) *exercise period:***

During the term and until three (3) months immediately following termination of this Agreement, consistent with and subject to the terms of the applicable stock option agreement/certificate and related documentation, Dr. Spiegel shall have the right to exercise the vested portion of the Stock Options; provided, however, that in no event may Dr. Spiegel exercise any of the vested Stock Options beyond the date that is ten (10) years from the initial grant date of any such options as set forth in the applicable option agreement or certificate.

**(iii) *change to Non-Qualified Options:***

As a result of Dr. Spiegel's change in status, all vested Incentive Stock Options shall be deemed to constitute Non-Qualified Stock Options beginning on the date that is three (3) months following his last day of employment under the Employment Agreement (not including the period of consulting under this Agreement) with PTC.

**(iv) *withholding obligations:***

When and if Dr. Spiegel exercises the vested portion of any Stock Options, PTC shall require him to satisfy any applicable income and employment tax withholding obligations prior to PTC's issuance of any shares with respect to the options.

**(v) *certification of insider trading policy:***

Consultant will read and execute PTC's current "Consultant Certification Regarding Insider Trading Policy".



## PTC Therapeutics Appoints Dr. Tuyen Ong as Chief Medical Officer

**SOUTH PLAINFIELD, N.J., April 26, 2016** — PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Tuyen Ong, M.D. has been promoted to Chief Medical Officer. Dr. Ong joined PTC in 2014 as the Senior Vice President, Head of Clinical Development and Translational Research. He succeeds Robert J. Spiegel M.D., FACP, who has served as PTC's Chief Medical Officer since 2011. Dr. Spiegel will remain active in a consulting role, continuing to support PTC's development programs and its global clinical and regulatory activities.

"Tuyen has been a valuable addition to the PTC team, bringing a wealth of industry experience and drug development expertise," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "He will continue to play an important role in the development of Translarna for rare genetic disorders as well as the advancement of our company pipeline. I thank Bob for his significant contributions to PTC over the last five years. He has worked closely with Tuyen and the entire clinical team in advance of this transition to lay the groundwork to support our future clinical and regulatory milestones."

Prior to joining PTC in 2014, Dr. Ong served as Vice President of Global Clinical Development and Operations at Valeant Pharmaceuticals (previously Bausch and Lomb). Dr. Ong played a key role in Bausch and Lomb's filing of new drug applications and its transformation into a global competitor in the specialty care sector. Previously, Dr. Ong worked at Pfizer Inc., developing drugs for diseases with high unmet medical need in various disease areas including respiratory, gastrointestinal, hepatology, and ophthalmology. Dr. Ong holds a medical degree from the University of London and an MBA from New York University Stern School of Business.

### About PTC Therapeutics

PTC is a global biopharmaceutical company focused on the discovery, development and commercialization of orally administered, proprietary small molecule drugs targeting an area of RNA biology we refer to as post-transcriptional control. Post-transcriptional control processes are the regulatory events that occur in cells during and after a messenger RNA, or mRNA, molecule is copied from DNA through the transcription process. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders and oncology. PTC has discovered all of its compounds currently under development using its proprietary technologies. PTC plans to continue to develop these compounds both on its own and through selective collaboration arrangements with leading pharmaceutical and biotechnology companies. For more information on the company, please visit our website [www.ptcbio.com](http://www.ptcbio.com)

### FOR MORE INFORMATION:

#### INVESTORS:

Jane Baj  
+1 (908) 912-9167  
[jbaj@ptcbio.com](mailto:jbaj@ptcbio.com)

#### MEDIA:

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### Forward Looking Statements:

All statements, other than those of historical fact, contained in this press release, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; the timing and outcome of PTC's regulatory strategy and process, including as it relates to PTC's submissions with the FDA, European Medicines Agency (EMA) and other regulatory bodies outside of the US or European Economic Area (EEA) and related regulatory reviews; PTC's ability to maintain its current marketing authorizations or obtain and maintain

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additional marketing authorizations; PTC's ability to work with the FDA to resolve the matters set forth in the Refuse to File letter PTC received in connection with its new drug application (NDA) for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD); the clinical utility and potential advantages of Translarna; the timing and scope of PTC's commercial and early access program launches; the rate and degree of market acceptance of Translarna; PTC's estimates regarding the potential market opportunity for Translarna, including the size of eligible patient populations and PTC's ability to identify such patients; the timing, results and conduct of PTC's clinical trials and studies, including statements regarding the timing of initiation, evaluation, enrollment and completion of the trials and studies and the period during which the results of the trials and studies will become available; PTC's strategy, future operations, future financial position, future revenues or projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "plan," "guidance," "anticipate," "believe," "estimate," "expect," "intend," "may," "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including whether PTC is required to perform additional clinical and non-clinical trials at significant cost and whether such trials, if successful, may enable FDA review of a NDA submission; whether the FDA, the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD or PTC's other clinical trials; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna; whether patients and healthcare professionals may be able to access Translarna through alternative means if pricing and reimbursement negotiations in the applicable territory do not have a positive outcome, including whether Translarna may be accessed through a reimbursed importation pathway provided under German law and whether such pathway will minimize any access issues for German patients while maintaining a sustainable price; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), the period during which the outcome of regulatory reviews will become available, adverse decisions by regulatory authorities, other delay or deceleration of the



regulatory process, and PTC's ability to meet existing or future regulatory standards with respect to Translarna; the scope of regulatory approvals or authorizations for Translarna (if any), including labeling and other matters that could affect the availability or commercial potential of Translarna; PTC's ability to maintain the marketing authorization of Translarna for the treatment of nmDMD in the EEA, which is subject to annual review and renewal by the EMA following its reassessment of the risk benefit balance of the authorization; PTC's ability to obtain full marketing authorization in the EEA or obtain or maintain marketing authorizations in territories outside the EEA; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; PTC's ability to fulfill any additional obligations, including with respect to further trials or studies relating to cost-effectiveness, obtaining licenses or satisfying requirements for labor and business practices, in the territories in which it may obtain regulatory approval, including the United States, EEA and other territories; the initiation, conduct and availability of data from clinical trials and studies; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; the outcome of ongoing or future clinical trials or studies; PTC's ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; the sufficiency of PTC's cash resources and PTC's ability to obtain adequate financing in the future for PTC's foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Annual Report on Form 10-K as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that Translarna will receive full regulatory approval in any territory or maintain its current marketing authorization in the EEA, or prove to be commercially successful in general, or specifically with respect to the treatment of nmDMD.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does not undertake or plan to update or revise any such forward-looking statements to reflect actual results or changes in plans, prospects, assumptions, estimates or projections, or other circumstances occurring after the date of this press release except as required by law.

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