



February 29, 2016

## **PTC Therapeutics Reports Fourth Quarter and Full Year 2015 Financial Results and Provides Corporate Update**

- **Refuse to File letter received from FDA regarding Translarna™ (ataluren) for nmDMD -**
- **Successful first year ex-U.S. launch with 2015 Translarna net sales of \$33.7M -**
- **Translarna European regulatory submissions for DMD and CF under review by the CHMP -**
- **Strong balance sheet with \$339M in cash at year-end -**

SOUTH PLAINFIELD, N.J., Feb. 29, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the fourth quarter and full year ending December 31, 2015.

"We are shocked and disappointed to have received a Refuse to File (RTF) letter from the FDA regarding our NDA for Translarna, and we are engaging in dialogue with the FDA to determine a path forward," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Despite this recent setback, we achieved a number of key milestones in 2015 as we continue to build PTC into a global, commercial biotechnology company. Translarna had a landmark first year launch in nonsense mutation Duchenne muscular dystrophy and we are currently under regulatory review in the EU to remove the condition to the existing marketing authorization as well as expand our label to include nonsense mutation cystic fibrosis. Our Phase 3 ACT CF clinical trial is fully enrolled and our clinical stage SMA and cancer stem cell assets continue to progress. I am proud of what we accomplished in 2015. Resilience has been one of our key values since I founded PTC in 1998 and our team continues to work diligently to bring Translarna to patients globally."

### **Key Fourth Quarter, Full Year 2015 and other Corporate Highlights:**

- 1 **Refuse to File letter received from the FDA regarding Translarna for nonsense mutation Duchenne muscular dystrophy (nmDMD).** The letter from the U.S. Food and Drug Administration (FDA) received on February 22, 2016 stated that the new drug application (NDA) for Translarna was not sufficiently complete to permit a substantive review. Specifically, PTC was notified in the letter that, in the view of the FDA, both the Phase 2b and ACT DMD trials were negative and do not provide substantial evidence of effectiveness. The FDA also characterized certain of the company's adjustments to the ACT DMD study as post hoc and therefore not supportive of effectiveness. In addition, the FDA noted that the NDA did not contain adequate information regarding the abuse potential of Translarna, a requirement for new molecules that cross the blood-brain barrier. PTC is engaging in dialogue with the FDA to discuss and clarify the matters set forth in the letter and to determine the best path forward.
- 1 **Successful first year Translarna launch with 2015 revenues of \$33.7M.** PTC has established a strong global commercial footprint launching the first approved therapy in Duchenne muscular dystrophy (DMD), with sales generated in 23 countries including most recently Argentina, the Czech Republic, Hungary, Portugal and Singapore. PTC is targeting to expand access to Translarna to over 35 countries by the end of 2016. Market access discussions regarding funding on a country-by-country basis are ongoing. In the UK, PTC has had constructive discussions with the National Health Services (NHS) England regarding a managed access agreement for Translarna with a decision from the National Institute for Health and Care Excellence (NICE) expected in the coming months. In Germany, PTC has had multiple discussions with the German Federal Association of the Statutory Health Insurances (GKV-SV) over the last several months to come to agreement on pricing and reimbursement. Recently, these discussions transitioned into an arbitration process, which did not lead to an acceptable agreement. As a result, PTC expects to delist Translarna from the German pharmacy ordering system. Under these circumstances, patients and healthcare professionals may be able to access Translarna through a reimbursed importation pathway possible under German law, thus minimizing any access issues for existing and new German patients.
- 1 **Actively pursuing regulatory approvals for Translarna in DMD globally.** In early January 2016, PTC submitted the ACT DMD Phase 3 results to the European Medicines Agency (EMA) in fulfillment of the principal condition of the EMA marketing authorization for the treatment of nmDMD in ambulatory patients aged five and over. The submission to the EMA, called a type II variation, seeks to remove the condition to the existing marketing authorization. PTC anticipates the Committee for Medicinal Products for Human Use (CHMP) will issue its recommendation regarding this request in mid-2016. Translarna also received regulatory approvals in both Israel and South Korea in 2015. In September 2015, Health Canada initiated an expedited review of Translarna for potential approval for nmDMD in the

first half of 2016.

- | **ACT CF Phase 3 clinical trial on track for completion by year-end 2016 with top-line results expected early 2017.** In November 2015, PTC announced that it had completed enrollment for ACT CF, the company's second Phase 3 clinical trial of Translarna for patients with nonsense mutation cystic fibrosis (nmCF). ACT CF is a 48-week placebo-controlled Phase 3 clinical trial designed to evaluate the effect of Translarna in patients six years of age or older with nmCF not receiving chronic inhaled aminoglycosides. During the third quarter, PTC submitted a variation to its marketing authorization requesting EMA approval of Translarna for the treatment of nmCF based on the company's previous Phase 3 study. PTC anticipates the CHMP will issue its recommendation regarding this submission in mid-2016.
- | **As part of 10 by '20 strategy, four additional indications in development for Translarna.** Given its mechanism of action, Translarna has the potential to address numerous genetic disorders caused by a nonsense mutation to address significant unmet need across a spectrum of many rare diseases. In addition to its advanced DMD and CF programs, PTC is pursuing indications in mucopolysaccharidosis type I (MPS I), aniridia, and two genetically defined epilepsy disorders, Dravet syndrome and CDKL5. PTC's goal is to investigate Translarna's activity in a minimum of ten indications beyond DMD and CF by 2020 in order to deliver on its commitment to patients and maximize the potential of Translarna as both a product and a pipeline.
- | **Internally developed pipeline continues to progress.** In January 2016, clinical development of the spinal muscular atrophy (SMA) program, a collaboration with Roche and the SMA Foundation, resumed with a second compound, RG7916, beginning a Phase 1 study in healthy volunteers. In addition, PTC's cancer stem cell program in oncology continues to advance with Phase 1 data expected in 2016. PTC's discovery group is focused on the advancement of novel programs for rare and neglected disorders including next generation nonsense read-through, Huntington's disease and familial dysautonomia.
- | **Maintained strong balance sheet with approximately \$339 million in cash and cash equivalents.** PTC completed a successful \$150 million offering of 3.00% convertible senior notes due 2022 in August 2015, raising net proceeds of approximately \$145 million. PTC finished 2015 with approximately \$339 million in cash and cash equivalents.

#### **Upcoming Events:**

PTC will participate in the following upcoming conferences:

- | Cowen and Company's 36<sup>th</sup> Annual Health Care Conference on March 8 at 11:20 a.m. (ET) in Boston, MA
- | Barclay's Global Healthcare Conference on March 15 at 1:35 p.m. (ET) in Miami, FL
- | Deutsche Bank's 41<sup>st</sup> Annual Healthcare Conference on May 4-5 in Boston, MA

The presentations will be webcast live on the Events and Presentations page under the investor relations section of PTC's website at [www.ptcbio.com](http://www.ptcbio.com) and will be archived for two weeks following the presentation. PTC's current investor presentation is available at the same website location.

#### **Fourth Quarter and Full Year 2015 Financial Highlights:**

- | Translarna net product sales were \$12.7 million for the fourth quarter of 2015, representing 30% sequential growth versus \$9.8 million in the third quarter of 2015. For the full year 2015, Translarna generated \$33.7 million in net product sales compared to \$0.7 million in the prior year.
- | Total revenues for the fourth quarter of 2015 were \$12.7 million compared to \$12.7 million in the same period of 2014. Total revenues for 2015 were \$36.8 million compared to \$25.2 million for the same period of 2014. The change in total revenue was a result of the expanded commercial launch of Translarna during 2015, which received marketing authorization from the EMA in August 2014, offset by lower grant revenue.
- | Non-GAAP R&D expenses were \$31.4 million for the fourth quarter of 2015, excluding \$3.7 million in non-cash, stock-based compensation expense, compared to \$23.7 million for the fourth quarter of 2014, excluding \$3.2 million in non-cash, stock-based compensation expense. GAAP R&D expenses were \$35.0 million for the fourth quarter of 2015 compared to \$26.9 million for the fourth quarter of 2014. For the full year 2015, non-GAAP R&D expenses were \$105.7 million, excluding \$16.1 million in non-cash, stock-based compensation expense, compared to \$70.1 million for 2014, excluding \$9.7 million in non-cash, stock-based compensation expense. For the full year 2015, GAAP R&D expenses were \$121.8 million compared to \$79.8 million in the prior year period. The increase in R&D expense for the fourth quarter and year ended December 31, 2015, as compared to the prior year periods was primarily due to expansion of our clinical development activities including late-stage studies and extension programs in both Duchenne muscular dystrophy and cystic fibrosis.
- | Non-GAAP SG&A expenses were \$21.7 million for the fourth quarter of 2015, excluding \$4.2 million in non-cash, stock-based compensation expense, compared to \$14.5 million for the fourth quarter of 2014, excluding \$3.5 million

in non-cash, stock-based compensation expense. GAAP SG&A expenses were \$25.9 million for the fourth quarter of 2015 compared to \$18.0 million for the fourth quarter of 2014. For the full year 2015, non-GAAP SG&A expenses were \$64.2 million, excluding \$17.8 million in non-cash, stock-based compensation expense, compared to \$35.2 million for 2014, excluding \$9.6 million in non-cash, stock-based compensation expense. GAAP full-year 2015 SG&A expenses were \$82.1 million compared to \$44.8 million in 2014. The increase in SG&A expense for the fourth quarter and year ended December 31, 2015, as compared to the prior year periods primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna for DMD.

- | Net loss for the fourth quarter of 2015 was \$50.9 million compared to a net loss of \$27.3 million for the same period in 2014. Net loss for the full year 2015 was \$170.4 million compared to \$93.8 million for the same period in 2014.
- | Cash, cash equivalents, and marketable securities totaled approximately \$339 million at December 31, 2015 compared to approximately \$315 million at December 31, 2014. This increase includes net proceeds of approximately \$145 million from a \$150 million convertible debt offering completed in the third quarter of 2015.
- | Shares issued and outstanding as of December 31, 2015 were 34.3 million, which includes 0.3 million shares of unvested restricted stock.

## 2016 Guidance:

- | Total ex-U.S. Translarna nmDMD revenues for 2016 are anticipated to be between \$65 and \$85 million. This guidance assumes current exchange rates and the continued commercial expansion for Translarna in nmDMD outside of the U.S.
- | Operating expenses for 2016 are currently under review as a result of the Refuse to File letter recently received from the FDA.

## Non-GAAP Financial Measures

In this press release, PTC's financial results and financial guidance are provided in accordance with accounting principles generally accepted in the United States (GAAP) and using certain non-GAAP financial measures. In particular, non-GAAP financial results exclude stock-based compensation expense. These results are provided as a complement to results reported in GAAP because management believes these non-GAAP financial measures are the best indication of the company's business.

**PTC Therapeutics, Inc.**  
**Consolidated Statements of Operations**  
(In thousands, except per share data)

	Three Months Ended		Twelve Months Ended	
	December 31,		December 31,	
	2015	2014	2015	2014
Revenues:				
Net product revenue	\$12,694	\$636	\$33,696	\$717
Collaboration and grant revenue	40	12,022	3,070	24,528
Total revenues	<u>12,734</u>	<u>12,658</u>	<u>36,766</u>	<u>25,245</u>
Operating expenses:				
Research and development (1)	35,048	26,871	121,816	79,838
Selling, general and administrative (1)	25,887	18,017	82,080	44,820
Total operating expenses	<u>60,935</u>	<u>44,888</u>	<u>203,896</u>	<u>124,658</u>
Loss from operations	(48,201)	(32,230)	(167,130)	(99,413)
Interest (expense)/income, net	(2,537)	406	(2,367)	1,180
Other (expense)/income, net	42	(138)	(465)	(213)
Loss before income tax expense	<u>(50,696)</u>	<u>(31,962)</u>	<u>(169,962)</u>	<u>(98,446)</u>
Income tax (expense)/benefit	(252)	4,693	(485)	4,693
Net loss	<u>(\$50,948)</u>	<u>(\$27,269)</u>	<u>(\$170,447)</u>	<u>(\$93,753)</u>
Weighted-average shares outstanding (in shares):				
Basic and diluted	<u>33,915,316</u>	<u>32,274,729</u>	<u>33,626,248</u>	<u>31,565,310</u>
Net loss per share - basic and diluted (in dollars per share)	<u>(\$1.50)</u>	<u>(\$0.84)</u>	<u>(\$5.07)</u>	<u>(\$2.97)</u>

(1) Non-cash share-based compensation expense

included in operating expenses are as follows:

Research and development	\$3,686	\$3,221	\$16,138	\$9,739
Selling, general and administrative	4,163	3,483	17,841	9,571
Total share-based compensation expense	\$7,849	\$6,704	\$33,979	\$19,310

**PTC Therapeutics, Inc.**  
**Summary Consolidated Balance Sheet**  
(In thousands, except share amounts)

	December 31, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	\$338,925	\$315,241
<b>Total assets</b>	<b>\$368,041</b>	<b>\$333,219</b>
Total debt	\$94,608	—
Total deferred revenue	139	3,354
<b>Total liabilities</b>	<b>\$142,040</b>	<b>\$34,752</b>
Total stockholders' equity (33,916,559 and 32,898,392 common shares issued and outstanding at December 31, 2015 and December 31, 2014, respectively)	226,001	298,467
<b>Total liabilities and stockholders' equity</b>	<b>\$368,041</b>	<b>\$333,219</b>

### Today's Conference Call and Webcast Reminder

Today's call will take place at 4:30 p.m. (ET) and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 51896300. The webcast will be available on the investor relations section of the PTC website at [www.ptcbio.com](http://www.ptcbio.com). A webcast replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for two weeks.

### About PTC Therapeutics, Inc.

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)

### About Translarna

Translarna, discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as dystrophin in Duchenne muscular dystrophy. Translarna is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. Translarna is an investigational new drug in the United States. The development of Translarna has been supported by grants from Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation); Muscular Dystrophy Association; FDA's Office of Orphan Products Development; National Center for Research Resources; National Heart, Lung, and Blood Institute; and Parent Project Muscular Dystrophy.

### For More Information Please Contact:

Investors:

Emily Hill

+1 (908) 912-9327

[ehill@ptcbio.com](mailto:ehill@ptcbio.com)

Media:

Justine O'Malley  
+1 (908) 912-9551  
[jomalley@ptcbio.com](mailto:jomalley@ptcbio.com)

### **Forward Looking Statements:**

All statements, other than those of historical fact, contained in this press release, are forward-looking statements, including the information provided under the heading "2016 Guidance" and 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, EMA and other regulatory bodies outside of the US or EEA and related regulatory reviews; PTC's ability to maintain its current marketing authorizations or obtain and maintain 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 NDA for Translarna for the treatment of nmDMD; the potential for PTC to reach a final agreement with NICE and NHS England on a managed access agreement for reimbursement for Translarna for the treatment of nmDMD, which is subject to positive outcome from the specialized appraisal process by NICE and actual reimbursement decisions by NHS England; the price of Translarna for the treatment of nmDMD in territories where PTC is or may be authorized to market Translarna, including in Germany; 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 of Translarna for the treatment of nmCF and other indications, as well its Phase 1 RG7916 and Phase 2 MOONFISH studies under its SMA collaboration with Roche and the SMA Foundation, and its Phase 1 study under its cancer stem cell program, 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 Quarterly Report on Form 10-Q or 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.

To view the original version on PR Newswire, visit:<http://www.prnewswire.com/news-releases/ptc-therapeutics-reports-fourth-quarter-and-full-year-2015-financial-results-and-provides-corporate-update-300227891.html>

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