



November 2, 2016

## PTC Therapeutics Reports Third Quarter 2016 Financial Results and Provides Corporate Update

- **Record quarter with Translarna™ net sales of \$22M; 125% year-over-year growth -**
- **YTD Translarna net sales of \$56M; on track for \$65M to \$85M full year guidance -**
- **Successful pricing and reimbursement negotiations concluded in Italy and Romania -**
- **CHMP opinion regarding Translarna's EMA marketing renewal expected by year-end -**
- **SMA program advancing with Phase 2 SUNFISH study initiated in Type 2/3 patients -**

SOUTH PLAINFIELD, N.J., Nov. 2, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ending September 30, 2016.

"For the past 18 years, we have focused significant effort towards developing Translarna for those affected by Duchenne," stated Stuart W. Peltz, Ph.D. Chief Executive Officer, PTC Therapeutics, Inc. "Over 400 Duchenne patients have participated in our clinical trials dating back to 2005 and the vast majority continue to remain on Translarna including approximately 130 boys in the United States. With this high compliance rate, we believe these actions speak to the benefit Translarna is providing to patients in the U.S. and around the world. In the E.U., regulatory discussions continue and we anticipate an opinion from the CHMP before the end of the year. With respect to the U.S., we will continue to escalate our appeal so that we may have the opportunity to have the Translarna NDA submission reviewed by the FDA. Our goal remains to deliver this novel therapy to nonsense mutation Duchenne patients globally."

### Key Third Quarter and Other Corporate Highlights:

- ▮ **Translarna net sales of \$22M in the third quarter represents 125% year-over-year growth.** Commercial access to Translarna continued to expand during the quarter with a significant number of new patients coming from England. While pricing and reimbursement discussions with a number of European countries are ongoing, PTC has now successfully concluded pricing and reimbursement negotiations in both Italy and Romania. PTC anticipates growth from both additional penetration into existing markets as well as expanding access in new geographies across Central and Eastern Europe, the Middle East and Latin America.
- ▮ **EMA regulatory discussions regarding Translarna's marketing authorization are ongoing.** PTC continues to interact with the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP) in connection with the company's ongoing request for annual renewal of its marketing authorization for Translarna. Following the company's CHMP meeting in October, the committee requested additional information regarding the risk-benefit profile of Translarna, its efficacy and the design of a potential trial that would provide comprehensive clinical data. While we have provided information in response to the CHMP's requests and expect to continue to engage in further interactions, recent dialogue has introduced a higher degree of uncertainty as to the outcome. PTC anticipates that an opinion regarding its marketing authorization renewal request will be adopted by the CHMP before the end of 2016. The current marketing authorization remains valid while the EMA assessment is ongoing and until a decision is made by the European Commission.
- ▮ **Appeal process of FDA's Refuse to File regarding Translarna for DMD continues.** As PTC previously stated, the RTF appeal could be an iterative process requiring multiple rounds before a conclusion. Having had its appeal denied, the company intends to continue the appeal to higher levels of the FDA. PTC continues to believe that the FDA can only accomplish a proper assessment of the data and analyses based on the results of the company's extensive clinical research and experience in more than 400 boys in the context of a full and fair review. This would include an advisory committee meeting that allows DMD clinical experts and representatives of the patient community to express their views on Translarna for the treatment of nmDMD. PTC intends to provide an update following a final conclusion of the appeal process or, alternatively, following a determination by the company to pursue an alternate regulatory strategy for advancing the potential approval of Translarna for the treatment of nmDMD in the U.S.
- ▮ **ACT CF Phase 3 clinical trial results expected late first quarter 2017.** PTC's confirmatory Phase 3 ACT CF clinical trial, a 48-week placebo-controlled trial designed to evaluate the efficacy of Translarna in patients six years of age or older with nonsense mutation cystic fibrosis, is ongoing. Following feedback from the FDA and the company's CHMP rapporteurs during the third quarter, PTC modified the protocol for ACT CF. In-line with clinical trials for other approved CF therapies, the primary endpoint of lung function as measured by FEV1 was updated from relative change to absolute change in percent predicted FEV1. Pulmonary exacerbations will be an important secondary endpoint.

- | **SMA program advancing with Phase 2 SUNFISH trial initiated in Type 2/3 patients.** PTC's joint development program in Spinal Muscular Atrophy (SMA) with Roche and the SMA Foundation initiated a Phase 2 study in pediatric and adult Type 2/3 SMA patients. SUNFISH, is a two-part study investigating the safety, tolerability and efficacy of RG7916, an oral small molecule survival motor neuron 2 (SMN2) splicing modifier. The first part of the study will evaluate safety and tolerability through escalating doses of RG7916. After dose selection, the study will transition into the pivotal second part evaluating the efficacy of RG7916. Initiation of the pivotal second part of the study is expected to begin in 2017 and will trigger a \$20 million milestone payment to PTC from Roche. A similarly designed two-part study to evaluate RG7916 in Type 1 SMA patients is expected to begin in the coming months.
- | **PTC596 cancer stem cell program to advance in clinical development in 2017.** Phase 1 safety data from PTC's clinical oncology program were recently presented at the European Society for Medical Oncology Congress. The ongoing Phase 1 dose-escalation study is evaluating the safety, tolerability and pharmacokinetics of PTC596 in patients with advanced solid tumors as a monotherapy. Preliminary results demonstrate that PTC596 is generally well tolerated at doses associated with exposures that achieved or exceeded efficacious target plasma concentrations in preclinical studies. Escalating doses are being evaluated for safety, pharmacodynamics, and to determine a target dose for subsequent studies. PTC596 is a novel, oral investigational drug that reduces the levels of BMI1, a protein that is required for cancer stem cell survival. Based on its proposed mechanism of action, PTC596 is expected to be most efficacious when used as part of combination therapy. Continued clinical development of PTC596 is being planned for 2017.

### Third Quarter Financial Highlights:

- | Translarna net product sales were \$22.0 million for the third quarter of 2016, representing a 125% increase versus \$9.8 million in the third quarter of 2015. Net sales increased in conjunction with continued expansion of commercial access to Translarna for nonsense mutation DMD boys outside of the U.S.
- | Total revenues for the third quarter of 2016 were \$23.0 million, an increase of \$13.2 million or 135% compared to \$9.8 million in the same period of 2015. The change in total revenue was primarily a result of the expanded commercial launch of Translarna.
- | GAAP R&D expenses were relatively flat at \$31.4 million for the third quarter of 2016 compared to \$30.6 million for the same period in 2015. Non-GAAP R&D expenses were \$27.1 million for the third quarter of 2016, excluding \$4.3 million in non-cash, stock-based compensation expense, compared to \$26.8 million for the same period in 2015, excluding \$3.8 million in non-cash, stock-based compensation expense.
- | GAAP SG&A expenses were \$23.7 million for the third quarter of 2016 compared to \$21.4 million for the same period in 2015. Non-GAAP SG&A expenses were \$19.0 million for the third quarter of 2016, excluding \$4.6 million in non-cash, stock-based compensation expense, compared to \$17.1 million for the same period in 2015, excluding \$4.2 million in non-cash, stock-based compensation expense. The increase in SG&A expense for the third quarter 2016 as compared to the prior year period primarily resulted from additional costs associated with commercial activities in support of Translarna across Europe and other regions.
- | Net interest expense for the third quarter of 2016 was \$2.1 million compared to net interest expense of \$0.9 million in the same period in 2015. The increase in interest expense is primarily a result of the \$150 million convertible debt offering completed during mid-third quarter 2015. The debt was recorded on PTC's balance sheet at a discount, which will be amortized over the life of the bond.
- | Net loss for the third quarter of 2016 was \$35.2 million, a decrease of \$8.1 million compared to a net loss of \$43.2 million for the same period in 2015.
- | Cash, cash equivalents, and marketable securities totaled approximately \$248.3 million at September 30, 2016 compared to approximately \$338.9 million at December 31, 2015.
- | Shares issued and outstanding as of September 30, 2016 were 34.3 million, which includes 0.2 million shares of unvested restricted stock.

### 2016 Guidance:

- | PTC expects to achieve total ex-US nmDMD Translarna net sales in the middle of our guidance of \$65 to \$85 million for 2016.
- | Operating expenses for the full year 2016 are now anticipated to be between \$180 million and \$190 million, excluding expected non-cash stock-based compensation expense of approximately \$35 million, for total operating expenses of approximately \$215 million to \$225 million. These expenses are in support of our ongoing clinical trials for Translarna in nmDMD and nmCF, commercial launch activities for Translarna outside of the U.S., and the continued research and clinical development of other product pipeline candidates.
- | PTC expects to end 2016 with cash and cash equivalents of approximately \$220 million.

### 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 and restructuring expenses

relating to the reorganization of operations intended to improve efficiency and better align costs and employment structure with the Company's strategic plans. These results are provided as a complement to results reported in GAAP, because management uses these non-GAAP financial measures when assessing and identifying operational trends. In management's opinion, these non-GAAP measures are useful to investors and other users of our financial statements by providing greater transparency into the operating performance at PTC and the company's future outlook.

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

	<b>Three Months Ended September 30,</b>		<b>Nine Months Ended September 30,</b>	
	<b>2016</b>	<b>2015</b>	<b>2016</b>	<b>2015</b>
Revenues:				
Net product revenue	\$ 22,013	\$ 9,772	\$ 56,328	\$ 21,002
Collaboration and grant revenue	973	4	1,186	3,030
Total revenues	22,986	9,776	57,514	24,032
Operating expenses:				
Research and development (1)	31,396	30,640	91,622	86,768
Selling, general and administrative (2)	23,654	21,368	72,958	56,193
Total operating expenses	55,050	52,008	164,580	142,961
Loss from operations	(32,064)	(42,232)	(107,066)	(118,929)
Interest (expense) income, net	(2,133)	(852)	(6,149)	170
Other expense, net	(786)	(51)	(1,893)	(507)
Loss before income tax expense	(34,983)	(43,135)	(115,108)	(119,266)
Income tax expense	(184)	(88)	(206)	(233)
Net loss attributable to common stockholders	<u>\$ (35,167)</u>	<u>\$ (43,223)</u>	<u>\$ (115,314)</u>	<u>\$ (119,499)</u>
Weighted-average shares outstanding:				
Basic and diluted (in shares)	34,088,741	33,908,853	34,002,952	33,528,833
Net loss per share—basic and diluted (in dollars per share)	<u>\$ (1.03)</u>	<u>\$ (1.27)</u>	<u>\$ (3.39)</u>	<u>\$ (3.56)</u>
<b>(1) Research and development expense reconciliation</b>				
GAAP research and development	\$ 31,396	\$ 30,640	\$ 91,622	\$ 86,768
Less: share-based compensation	4,319	3,828	12,734	12,452
Less: one-time restructuring cost	5	—	845	—
<b>Non-GAAP research and development expense</b>	<u>\$ 27,072</u>	<u>\$ 26,812</u>	<u>\$ 78,043</u>	<u>\$ 74,316</u>
<b>(2) Selling, general and administrative expense reconciliation</b>				
GAAP selling, general and administrative	\$ 23,654	\$ 21,368	\$ 72,958	\$ 56,193
Less: share-based compensation	4,640	4,226	13,876	13,678
Less: one-time restructuring cost	28	—	1,661	—
<b>Non-GAAP selling, general and administrative expense</b>	<u>\$ 18,986</u>	<u>\$ 17,142</u>	<u>\$ 57,421</u>	<u>\$ 42,515</u>

**PTC Therapeutics, Inc.**  
**Summary Consolidated Balance Sheets**  
(In thousands, except per share data)

	<b>September 30, 2016</b>	<b>December 31, 2015</b>
Cash, cash equivalents and marketable securities	\$ 248,338	\$ 338,925
<b>Total assets</b>	<u><b>\$ 288,177</b></u>	<u><b>\$ 365,281</b></u>
Total debt	\$ 96,559	\$ 91,848
Total deferred revenue	910	139
<b>Total liabilities</b>	<u><b>\$ 148,000</b></u>	<u><b>\$ 139,280</b></u>
Total stockholders' equity (34,165,519 and 33,916,559 common shares issued and outstanding at September 30, 2016 and December 31, 2015, respectively)	140,177	226,001
<b>Total liabilities and stockholders' equity</b>	<u><b>\$ 288,177</b></u>	<u><b>\$ 365,281</b></u>

### **Today's Conference Call and Webcast Reminder**

Today's call will take place at 4:45 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 3969239. 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)

### **For More Information, Please Contact:**

#### **Investors:**

Emily Hill  
+1 (908) 912-9327  
[ehill@ptcbio.com](mailto:ehill@ptcbio.com)

#### **Media:**

Jane Baj  
+1 (908) 912-9167  
[jbaj@ptcbio.com](mailto:jbaj@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 (i) when the EMA's CHMP will issue an opinion with respect to the renewal of the marketing authorization for Translarna for the treatment of nmDMD and, when issued, whether such opinion will be positive, (ii) the nature of any conditions or restrictions that may be placed on any renewal of the marketing authorization by the European Commission (if such marketing authorization is renewed), (iii) PTC's ability to design an acceptable new clinical trial in nmDMD with input from the EMA, (iv) PTC's ability to resolve the matters set forth in the requests for information received to date from the CHMP, and (v) the timing and outcome of future interactions PTC has with the FDA with respect to Translarna for the treatment of nmDMD, including PTC's ability to resolve the matters set forth in the Refuse to File letter with the FDA or otherwise advance Translarna for the treatment of nmDMD in the United States (whether pursuant to continued appeal under the formal dispute resolution process or otherwise); 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 as 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; potential advancement of PTC's joint development program in SMA, in particular as related to the timing of initiation, evaluation, enrollment and completion of the Phase 2 clinical studies of RG7916 in SMA patients and whether and when a milestone payment to PTC from Roche may be triggered; PTC's ability to continue to supply Translarna to patients across Europe and in other territories; 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 "guidance," "plan," "target," "anticipate," "believe," "estimate," "expect," "intend," "may," "potential," "project," "possible," "potential," "will," "would," "could," "should," "continue," "goal," 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: PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the EEA, including whether the EMA determines that the benefit-risk balance of Translarna authorization supports renewal of the company's marketing authorization in the European Economic Area, or EEA, and whether the European Commission determines to renew such authorization; the nature and scope of any new nmDMD trial that PTC may design with the input of the EMA and PTC's

ability to enroll, fund and conduct such trial; the 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; the EMA's determinations with respect to PTC's variation submission which seeks to add Translarna for the treatment of nonsense mutation cystic fibrosis to PTC's marketing authorization in the EEA; 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; the outcome of ongoing or future clinical trials or studies in Translarna, including ACT CF and the Phase 2 study of Translarna for nmDMD in pediatric patients; the eligible patient base and commercial potential of Translarna and PTC's other product candidates; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD, including its ability to establish and maintain arrangements with manufacturers, suppliers, distributors and production and collaboration partners on favorable terms; 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; 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 (or other delay or deceleration of the regulatory process), and PTC's ability to meet existing or future regulatory standards with respect to Translarna; 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 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 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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