May 5, 2016

PTC Therapeutics Reports First Quarter 2016 Financial Results and Provides Corporate Update

- Translarna™ net sales of $18.9M representing 49% sequential growth -
- Full-year net sales on track to meet guidance of $65M to $85M -
- Proof of concept achieved in second SMA compound, and RG7916 and clinical study in SMA patients planned for 2H of 2016 -

SOUTH PLAINFIELD, N.J., May 5, 2016 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2016.

"We are working hard to make Translarna available to all Duchene muscular dystrophy patients globally who may benefit," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "To that end, we are in dialogue with the FDA about the Refuse to File letter we received earlier this year. In Europe, we are also continuing discussions with the CHMP surrounding our regulatory submissions for DMD and CF. We are seeing growth in Translarna global sales and continue to expand access for DMD patients outside of the U.S. We were pleased with the positive recommendation from NICE, and anticipate access for patients in England once the market access agreement with NHS England is finalized. We are also excited that the second SMA compound in our collaboration is advancing in the clinic."

Key First Quarter and other Corporate Highlights:

- **Actively pursuing regulatory approvals for Translarna in DMD globally.** PTC has engaged in dialogue with the U.S. Food and Drug Administration (FDA) to discuss the matters described in the Agency's Refuse to File letter regarding Translarna for nonsense mutation Duchenne muscular dystrophy (nmDMD) and to seek a potential path forward to bring Translarna to patients in the U.S. Given the sensitivity around these discussions as well as their iterative nature, PTC will provide an update once it has clarified the regulatory strategy for Translarna in the U.S.

  In the first quarter of 2016, PTC submitted the ACT DMD Phase 3 results to the European Medicines Agency (EMA) in support of the marketing authorization for Translarna for the treatment of nmDMD in ambulatory patients aged five and over, and separately, submitted a request for renewal of its current conditional marketing authorization. PTC is in the process of responding to requests for supplementary information from the Committee for Medicinal Products for Human Use (CHMP) to assist in determining whether the results of ACT DMD support a positive benefit-risk assessment for Translarna. PTC anticipates that the CHMP will issue its recommendation in mid-2016.

  In Canada, PTC plans to submit a New Drug Submission for Translarna to Health Canada incorporating the results of the company's Phase 3 ACT DMD study in the second half of 2016.

  PTC recently initiated a Phase 1 pharmacokinetics study to assess the effects of Translarna in Japanese healthy volunteers. This study is the first step in pursuing regulatory approval for Translarna for nmDMD in Japan, one of the world's largest pharmaceutical markets.

- **Translarna revenue of $18.9M in first quarter, which represents 49% growth over prior quarter.** PTC has established a strong global commercial footprint launching the first approved therapy in nmDMD. By the end of 2016, PTC anticipates expanding commercial access to Translarna in over 35 countries across Europe, the Middle East, Latin America and Asia Pacific.

- **Translarna received recommendation in draft guidance from NICE.** In the UK, the National Institute for Health and Care Excellence (NICE) has recommended Translarna for ambulatory patients aged five years and older with nmDMD in connection with a Managed Access Agreement (MAA) with NHS England. The provision of patient access is subject to the completion of the MAA with NHS England and finalization of the NICE draft guidance. NICE recently extended the timeframe for finalization of its guidance.

- **German patients accessing Translarna through alternate distribution channel.** In Germany, PTC has delisted

Translarna from the pharmacy ordering system. Since delisting in April, initial orders for German patients have already been successfully fulfilled via a foreign importation pathway. This pathway allows patients with high unmet medical needs to receive reimbursed access to treatments not directly available in Germany.

- **ACT CF Phase 3 clinical trial on track for completion by year-end 2016 with top-line results expected early 2017**. During the third quarter of 2015, PTC submitted a variation to its marketing authorization requesting EMA approval of Translarna for the treatment of nonsense mutation cystic fibrosis (nmCF) based on the company's previous Phase 3 study. The company anticipates the CHMP will issue its opinion regarding this submission in mid-2016. PTC's confirmatory Phase 3 ACT CF trial is currently ongoing, and there is substantial risk that the results from this trial will be required for approval.

- **SMA program progressing with proof of concept achieved in Phase 1 study of RG7916 and clinical study in SMA patients planned for second half of 2016.** Clinical development of the spinal muscular atrophy (SMA) program, a collaboration with Roche and the SMA Foundation, continued with the completion of a Phase 1 study of RG7916 in healthy volunteers. Preliminary results indicate that RG7916 was well tolerated and treatment resulted in increases of full length SMN2 mRNA. A clinical study of RG7916 in SMA patients is expected to begin in the second half of 2016.

First Quarter Financial Highlights:

- Translarna net product sales were $18.9 million for the first quarter of 2016, representing 49% growth over $12.7 million reported in the fourth quarter of 2015; and significant growth versus $5.1 million in the first quarter of 2015. Translarna net product sales were positively impacted by a significant order from Brazil, where purchasing is often fulfilled in six-month orders, partially offset by a lower sales price for existing stock in Germany as a result of a mandatory discount imposed by the German Federal Association of the Statutory Health Insurances (GKV-SV) prior to delisting. The effect of larger but less frequent orders from Brazil may result in fluctuations in quarterly sales reporting during the course of the year.
- Total revenues for the first quarter of 2016 were $18.9 million compared to $7.5 million in the same period of 2015. The change in total revenue was a result of the expanded commercial launch of Translarna, partially offset by lower grant revenue.
- Non-GAAP R&D expenses were $27.1 million for the first quarter of 2016, excluding $4.3 million in non-cash, stock-based compensation expense, compared to $23.3 million for the same period in 2015, excluding $4.6 million in non-cash, stock-based compensation expense. GAAP R&D expenses were $31.4 million for the first quarter of 2016 compared to $27.9 million for the same period in 2015. The increase in R&D expense for the first quarter of 2016 as compared to the prior year period was primarily due to additional costs associated with our ongoing clinical trials.
- Non-GAAP SG&A expenses were $21.3 million for the first quarter of 2016, excluding $4.6 million in non-cash, stock-based compensation expense, compared to $12.5 million for the same period in 2015, excluding $5.1 million in non-cash, stock-based compensation expense. GAAP SG&A expenses were $25.9 million for the first quarter of 2016 compared to $17.6 million for the same period in 2015. The increase in SG&A expense for the first 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 first quarter of 2016 was $2.0 million compared to net interest income of $0.5 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 the 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 first quarter of 2016 was $41.2 million compared to a net loss of $37.9 million for the same period in 2015.
- During the quarter, PTC announced a workforce reduction of approximately 18% of its employees and contractors, which will result in a one-time charge of approximately $2.6 million. PTC incurred $1.9 million of this charge in the first quarter.
- Cash, cash equivalents, and marketable securities totaled approximately $299 million at March 31, 2016 compared to approximately $339 million at December 31, 2015.
- Shares issued and outstanding as of March 31, 2016 were 34.3 million, which includes 0.4 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 the full year 2016 are anticipated to be between $185 million and $195 million, excluding expected non-cash stock-based compensation expense of approximately $40 million, for total operating expenses of approximately $225 million to $235 million. These expenses will be primarily in support of our ongoing clinical trials for Translarna in nmDMD and nmCF, commercial launch activities for Translarna outside of the US, and the continued

research and clinical development of other product pipeline candidates.
- PTC expects to end 2016 with cash and cash equivalents of approximately $200 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. 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.

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

	Three Months Ended March 31,	
	2016	**2015**
Revenues:		
Net product revenue	$18,878	$5,069
Collaboration and grant revenue	17	2,413
Total revenues	18,895	7,482
Operating expenses:		
Research and development (1)	31,399	27,938
Selling, general and administrative (1)	25,938	17,615
Total operating expenses	57,337	45,553
Loss from operations	(38,442)	(38,071)
Interest (expense)/income, net	(1,956)	524
Other expense, net	(721)	(368)
Loss before income tax expense	(41,119)	(37,915)
Income tax expense	(114)	—
Net loss	($41,233)	($37,915)
Weighted-average shares outstanding (in shares):		
Basic and diluted	33,919,169	33,067,752
Net loss per share - basic and diluted (in dollars per share)	($1.22)	($1.15)
(1) Non-cash share-based compensation expense included in operating expenses are as follows:		
Research and development	$4,328	$4,667
Selling, general and administrative	4,587	5,081
Total share-based compensation expense	$8,915	$9,748

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

	March 31, 2016	**December 31, 2015**
Cash, cash equivalents and marketable securities	$298,712	$338,925
Total assets	$335,232	$365,281

Total debt	$93,366	$91,848
Total deferred revenue	396	139
Total liabilities	$139,231	$139,280
Total stockholders' equity (33,919,684 and 33,916,559 common shares issued and outstanding at March 31, 2016 and December 31, 2015, respectively)	196,001	226,001
Total liabilities and stockholders' equity	$335,232	$365,281

Upcoming Events:

PTC will participate in the following upcoming conference:

- Bank of America 2016 Healthcare Conference, **May 12 at 9:20 a.m. in Las Vegas, NV**

The presentation will be webcast live on the Events and Presentations page under the investor relations section of PTC's website at 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.

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 93568683. The webcast will be available on the investor relations section of the PTC website at 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

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.

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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

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 European Economic Area, or EEA, and related regulatory reviews; PTC's ability to maintain its current marketing authorizations, including in the EEA, or obtain and maintain additional marketing authorizations; PTC's ability to resolve the matters set forth in the Refuse to File letter PTC received from the FDA in connection with its new drug application, or NDA, for Translarna for the treatment of nmDMD; the potential for PTC to reach a final agreement with NHS England on a managed access agreement for reimbursement for Translarna for the treatment of nmDMD, which is subject to final positive guidance from NICE; 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 as its 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; 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 risk-benefit balance of Translarna supports continuation of our marketing authorization in the EEA on a conditional basis, a full basis, or at all; whether other regulators agree with PTC's interpretation of the results of ACT DMD; 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; PTC's ability to commercialize and commercially manufacture Translarna in general and specifically as a treatment for nmDMD; the outcome of pricing and reimbursement negotiations in those territories in which PTC is authorized to sell Translarna, including whether final guidance from NICE recommends Translarna for the treatment of nmDMD and the acceptability of final terms of any market access agreement between PTC and NHS England; 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 be utilized by German patients while maintaining a sustainable price for Translarna; 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; 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 or 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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