



May 4, 2015

## PTC Therapeutics Reports First Quarter 2015 Financial Results and Provides Corporate Update

### **-Translarna™ launch accelerates with 82 DMD patients now on commercial therapy -First patient dosed in BMI1 cancer stem cell Phase 1 study-**

SOUTH PLAINFIELD, N.J., May 4, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2015.

"We are off to a strong start this year. In our first full quarter since the commercial launch, we have seen strong support for access to Translarna, the first treatment for nonsense mutation Duchene Muscular Dystrophy. We now have 82 patients on commercial therapy, nearly double the number on treatment since our last earnings call," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Our goal is to bring Translarna to patients across the globe as quickly as possible and we are pleased to have grown our commercial footprint to approximately 25 countries to date. We are also actively establishing our US infrastructure, in preparation for our anticipated US launch."

### **Key First Quarter 2015 Corporate Highlights**

- | **Translarna commercial launch continues to accelerate.** PTC's lead product, Translarna™ (ataluren), received marketing authorization in the EU in August 2014 for the treatment of nonsense mutation Duchene muscular dystrophy, representing the first-ever treatment approved for the underlying cause of the disease. As of April 30<sup>th</sup>, PTC has 82 DMD patients on commercial therapy through either reimbursed early access programs or commercial sales.
- | **US commercial launch preparations are underway.** In December 2014, PTC began submitting a rolling New Drug Application to the FDA for the approval of Translarna in nonsense mutation DMD. Top-line data from the company's ongoing Phase 3 ACT DMD trial is expected in the fourth quarter of this year which should form the basis for finalizing the NDA submission. Concurrently, PTC has begun building out its US commercial team and infrastructure in preparation for a potential US launch in the first half of 2016. Most recently, PTC announced the hiring of Eric Pauwels as head of commercial operations in the Americas.
- | **Confirmatory Phase 3 ACT CF trial enrollment is on track.** PTC initiated its global confirmatory Phase 3 ACT CF trial in nonsense mutation cystic fibrosis patients in June 2014. The trial is being conducted at approximately 90 clinical sites globally and is expected to enroll approximately 208 patients by the end of 2015. PTC intends to file for approval of Translarna for the treatment of nonsense mutation cystic fibrosis in the EU in the second half of 2015.
- | **Translarna pipeline will expand with a proof-of-concept study in aniridia.** PTC plans to initiate a Phase 2 proof-of-concept study in nonsense mutation aniridia by year end 2015. Aniridia is a rare genetic disorder that results in disruption in the development of the eye. Pre-clinical data were presented at the Association for Research in Vision and Ophthalmology (ARVO) on May 3<sup>rd</sup>. PTC is also initiating a proof-of-concept Phase 2 study of Translarna in nonsense mutation MPS I in addition to its ongoing Phase 3 trials in nonsense mutation DMD and nonsense mutation CF. Because of its mechanism of action, Translarna has the potential to address numerous disorders caused by a nonsense mutation. PTC continues to evaluate additional indications to fully capture Translarna's potential.
- | **SMA Phase 1 results highlighted at the annual meeting of the American Academy of Neurology.** On April 22<sup>nd</sup> at the AAN emerging science session, clinical data from the Phase 1 SMA study demonstrated a significant and dose-dependent increase in the production of full length SMN2 mRNA. After a single dose, an increase of approximately 80% was observed in the levels of full length SMN2 mRNA expression. Since the SMN2 gene in healthy volunteers is the same as in SMA patients, these results demonstrate proof of mechanism based on the expected pharmacodynamic effect. The Phase 1 clinical study was completed in the spring of 2014 and was well tolerated at all dose levels studied.
- | **New preclinical and clinical developments in the SMA collaboration.** Dosing of the first cohort in the Phase 2 MOONFISH study successfully completed. RG7800 was well tolerated and preliminary review of the blinded data indicates substantial increases in full length SMN2 mRNA. Long term preclinical animal data have recently shown an eye finding at concentrations above those explored in patients. This finding was not observed in humans. As a precautionary measure, the collaboration partners decided to temporarily suspend dosing of additional patients to evaluate this finding and confirm next steps for MOONFISH.
- | **Initiated cancer stem cell program Phase 1 study.** An open-label Phase 1 clinical study for this program targeting BMI1, a protein linked to drug resistant cancers, began in April. In the fourth quarter of 2014, PTC completed IND-enabling studies for PTC596 and filed an investigational new drug application with the FDA.

## Upcoming Events:

PTC will participate in the following conferences in the second quarter:

- ┆ Deutsche Bank 40<sup>th</sup> Annual Healthcare conference May 6<sup>th</sup> in Boston, MA
- ┆ Bank of America Merrill Lynch Healthcare conference May 12<sup>th</sup> in Las Vegas, NV
- ┆ Barclays Select West Coast Biotech conference May 28<sup>th</sup> in Napa, CA

## First Quarter 2015 Financial Highlights:

- ┆ Total revenues for the first quarter of 2015 were approximately \$7.5 million, including \$5.1 million in Translarna product sales revenue and \$2.4 million in grants and collaborations revenue. Total Translarna product sales in first quarter 2015 includes \$1.4 million of revenue which was deferred during 2014 when we recognized revenue on a cash basis. As of January 1, 2015, we began recognizing revenue for Translarna as product is shipped, given we have established a pattern of collectability. This compared to total revenue in the first quarter of 2014 of approximately \$9.2 million. The decrease in total revenue was due to receipt of a milestone payment of \$7.5 million from Roche recognized in the first quarter of 2014 related to our SMA collaboration, partially offset by Translarna product sales.
- ┆ Research and development expenses were \$27.9 million for the first quarter of 2015, including \$4.7 million in non-cash, stock-based compensation expense, compared to \$15.9 million for the same period in 2014, including \$1.9 million in non-cash, stock-based compensation expense. The increase in R&D expense for the first quarter 2015 as compared to the prior year quarter was primarily due to additional costs associated with our ongoing clinical trials and supply chain activities in support of the launch of Translarna as well as in conjunction with our expanding clinical-stage pipeline.
- ┆ Selling, general and administrative expenses were \$17.6 million for the first quarter of 2015, including \$5.1 million in non-cash stock-based compensation expense, compared to \$7.5 million for the same period in 2014, including \$1.8 million in non-cash stock-based compensation expense. The increase in SG&A expense for the first quarter 2015 as compared to the prior year period primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna across Europe and other regions.
- ┆ Net loss for the first quarter of 2015 was \$37.9 million compared to a net loss of \$14.1 million for the same period in 2014.
- ┆ Cash, cash equivalents, and marketable securities totaled \$280.5 million at March 31, 2015 compared to \$315.2 million at December 31, 2014.
- ┆ Shares issued and outstanding as of March 31, 2015 were 33.9 million, which includes 0.4 million shares of unvested restricted stock.

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

	<b>Three Months Ended</b>	
	<b>March 31,</b>	
	<b>2015</b>	<b>2014</b>
Revenues:		
Net product revenue	\$5,069	\$ -
Collaboration revenue	338	9,147
Grant revenue	2,075	70
Total revenues	7,482	9,217
Operating expenses:		
Research and development (1)	27,938	15,889
Selling, general and administrative (1)	17,615	7,540
Total operating expenses	45,553	23,429
Loss from operations	(38,071)	(14,212)
Interest income	524	171
Other income (expense), net	(368)	(57)
Net loss	<u>(\$37,915)</u>	<u>(\$14,098)</u>
Weighted-average shares outstanding (in shares):		
Basic and diluted	<u>33,067,752</u>	<u>24,492,487</u>
Net loss per share - basic and diluted (in dollars per share)	<u>(\$1.15)</u>	<u>(\$0.58)</u>

(1) Non-cash share-based compensation expense included in operating expenses are as follows:		
Research and development	\$4,667	\$1,944
Selling, general and administrative	5,081	1,761
Total share-based compensation expense	\$9,748	\$3,705

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

	<b>March 31, 2015</b>	<b>December 31, 2014</b>
Cash, cash equivalents and marketable securities	\$280,467	\$315,241
<b>Total assets</b>	<b>\$299,520</b>	<b>\$333,219</b>
Total deferred revenue	373	3,354
<b>Total liabilities</b>	<b>\$26,200</b>	<b>\$34,752</b>
Total stockholders' equity (33,483,306 and 32,898,392 common shares issued and outstanding at March 31, 2015 and December 31, 2014, respectively)	273,320	298,467
<b>Total liabilities and stockholders' equity</b>	<b>\$299,520</b>	<b>\$333,219</b>

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

The call 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 85749873.

A live, listen-only webcast of the conference call can be accessed 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, oncology and infectious diseases. 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™ (ataluren)**

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:**

This press release contains forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. All statements, other than those of historical fact, contained in this release, including statements regarding the future expectations, plans and prospects for PTC; the timing and scope of PTC's commercial and early access program launches; the rate and degree of market acceptance and clinical utility 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 and conduct of PTC's clinical trials and studies of Translarna for the treatment of DMD, CF, MPS I and aniridia caused by nonsense mutation, as well its Phase 2 MOONFISH study 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; our current and planned regulatory filings, including with the FDA and in the European Union; our strategy, future operations, future financial position, future revenues or projected costs; and objectives of management, are forward-looking statements. 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 its ability to commercialize Translarna in general and specifically as a treatment for nonsense mutation DMD, including its ability to successfully negotiate favorable pricing and reimbursement processes on a timely basis in the countries in which it may obtain regulatory approval, including the countries in the European Economic Area; the initiation, conduct and availability of data from clinical trials and studies; expectations for regulatory approvals; PTC's scientific approach and general development progress; the eligible patient base and commercial potential of Translarna and PTC's other product candidates 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. 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 release except as required by law.

To view the original version on PR Newswire, visit: <http://www.prnewswire.com/news-releases/ptc-therapeutics-reports-first-quarter-2015-financial-results-and-provides-corporate-update-300076977.html>

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