February 27, 2015

PTC Therapeutics Reports Fourth Quarter and 2014 Full Year Financial Results and Provides Corporate Update

-Translarna™ approved in the European Union and recently launched in Germany
-42 patients now on commercial Translarna therapy-
-Rolling NDA submitted for Translarna in the US-
-Confirmatory Phase 3 ACT CF trial initiated-
-SMA program advanced into Phase 2 MOONFISH study-
-Advanced internally developed programs to enhance the pipeline-
-Maintained strong balance sheet with $315 million in cash-

SOUTH PLAINFIELD, N.J., Feb. 27, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the fourth quarter and full year ended December 31, 2014.

"2014 was a transformative year for PTC. We are now a growing commercial-stage biopharma company, focused on delivering and developing RNA-targeted therapies in the rare disease space," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We are proud to bring the first treatment for Duchenne muscular dystrophy to patients who suffer from this devastating disorder. Concurrent with our ongoing launch activities in Europe and around the world, we are beginning to build out our commercial infrastructure in the US in preparation for a potential launch in 2016. We are equally focused on bringing additional innovative therapies to patients by moving our earlier stage pipeline forward. Importantly, we completed two successful public equity financings in 2014, providing us with a strong cash position to advance our ongoing commercial and clinical efforts."

Key 2014 Achievements:

- **Translarna approved in the European Union:** 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.
- **Launched in EU and select countries:** In the second half of 2014, reimbursed early access programs were authorized in a number of countries both within Europe and outside of Europe where the EMA approval is referenced. Translarna was recently launched on a commercial basis in Germany in December of 2014. Additional country launches are expected to continue across the EU throughout 2015, subject to successful completion of pricing and reimbursement negotiations. As of February 25, 2015, PTC had 42 DMD patients on commercial Translarna therapy through either reimbursed early access programs or commercial sales.
- **Rolling NDA submitted for Translarna in the US:** 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.
- **Confirmatory Phase 3 ACT CF trial initiated:** PTC initiated its global confirmatory Phase 3 ACT CF trial in nonsense mutation cystic fibrosis patients. 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.
- **SMA program completed Phase 1a study, demonstrating proof of mechanism in healthy volunteers; Phase 2 study initiated in SMA patients:** In the spring of 2014, a Phase 1 clinical study in healthy volunteers was successfully completed. The single-ascending dose study was well tolerated at all dose levels studied. Importantly, a dose-dependent effect on SMN2 splicing was observed which may be interpreted as proof of mechanism based on the expected pharmacodynamic effect. A multiple-dose, Phase 2 clinical study called MOONFISH was initiated in November 2014 with results expected in 2016. PTC received $17.5 million in milestone payments from Roche over the course of the year.
- **Advanced internally developed pipeline programs:** In the fourth quarter of 2014, PTC completed IND-enabling studies for PTC596, a cancer stem cell targeting program, and filed an investigational new drug application with the FDA. An open-label Phase 1 clinical study for this program is expected to begin in the first half of 2015. PTC also declared a development candidate and is initiating IND-enabling studies for its anti-bacterial program focused on the treatment of multi-drug resistant gonorrhea.

- **Established international headquarters in Dublin, Ireland:** This office will serve as the central hub for the commercialization of Translarna on a global basis and will enable PTC to take advantage of its proximity to our third-party manufacturing and supply chain.
- **Maintained strong balance sheet with $315 million in cash and cash equivalents:** PTC completed two successful public equity offerings during the year raising net proceeds of approximately $236 million. PTC finished 2014 with over $315 million in cash and cash equivalents.

Upcoming Events:

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

- Cowen & Co 35th Annual Health Care conference on March 2nd in Boston, MA
- 27th Annual Roth Conference on March 9th in Orange County, CA
- Barclays Capital Healthcare Conference on March 10th in Miami, FL

Fourth Quarter and Full year 2014 Financial Highlights:

- Total revenues for the fourth quarter of 2014 were approximately $12.7 million, including $0.6 million in Translarna product sales revenue, which was recognized on a cash-basis, and $12.0 million in grants and collaborations. This compared to total revenue in the fourth quarter of 2013 of approximately $4.4 million. The increase in grants and collaborations revenue was due to receipt of a milestone payment of $10 million from Roche recognized in the fourth quarter of 2014 related to our SMA collaboration. Total revenue in 2014 was $25.2 million, including $0.7 million in Translarna product sales revenue, which was recognized on a cash-basis and $24.5 million in grants and collaborations revenue. Total invoiced Translarna product sales in 2014 was approximately $2.5 million, of which approximately $1.7 million has been booked as deferred revenue until cash payment has been received. This compared to total revenue in 2013 of $34.7 million which was entirely from grants and collaborations. The decrease in grants and collaborations revenue primarily resulted from a reduction in non-cash deferred revenue of $16.8 million in 2014 vs. 2013.
- Research and development expenses were $26.9 million for the fourth quarter of 2014, including $3.2 million in non-cash, stock-based compensation expense, compared to $15.0 million for the same period in 2013, including $1.6 million in non-cash, stock-based compensation expense. Research and development expenses for the full year 2014 were $79.8 million, including $9.7 million in non-cash, stock-based compensation expense compared to $54.9 million for the same period in 2013, including $4.3 million in non-cash, stock-based compensation expense. The increase in R&D expense for the quarter and year ended December 31, 2014 as compared to the prior year periods was primarily due to additional costs associated with our ongoing clinical trials, including the initiation of the Phase 3 ACT CF trial, and increased costs associated with regulatory, quality and supply chain functions including the manufacturing of Translarna drug product.
- Selling, general and administrative expenses were $18.0 million for the fourth quarter of 2014, including $3.5 million in non-cash stock-based compensation expense, compared to $7.5 million for the same period in 2013, including $1.7 million in non-cash stock- based compensation expense. SG&A expenses for the full year 2014 were $44.8 million, including $9.6 million in non-cash stock-based compensation expense, compared to $25.2 million in 2013, including $4.1 million in non-cash stock-based compensation expense. The increase in SG&A expense for the quarter and year ended December 31, 2014 as compared to the prior year periods was primarily resulted from additional costs associated with commercial activities in support of the launch of Translarna across Europe and select other regions as well as finance and legal costs associated with establishing our international infrastructure.
- Net loss for the fourth quarter of 2014 was $27.3 million compared to a net loss of $17.9 million for the same period in 2013. Net loss for the full year 2014 was $93.8 million compared to $51.6 million for the same period in 2013.
- Cash, cash equivalents, and marketable securities totaled $315.2 million at December 31, 2014 compared to $142.5 million at December 31, 2013. This increase was primarily as a result of two public equity offerings that were completed in 2014 which raised net proceeds of approximately $236 million. We also received $4.9 million in the fourth quarter of 2014 from the sale of net operating losses and research and development credits as part of the New Jersey Technology Business Tax Certificate Transfer Program.
- Shares issued and outstanding as of December 31, 2014 were 33.6 million, which includes 0.7 million shares of unvested restricted stock.

2015 Guidance:

- Operating expense for the full year 2015 is anticipated to be between $160- $170 million, excluding expected non-cash stock-based compensation expense of approximately $30 million, for total operating expenses of approximately $190 million to $200 million. These expenses will be primarily in support of our ongoing and planned confirmatory Phase 3 clinical trials for Translarna in nmDMD and nmCF, commercial launch activities for Translarna in the EU as well as pre-commercial activities in the US, and the continued research and clinical development of other product pipeline candidates.

- For 2015, PTC anticipates providing the number of nmDMD patients on Translarna therapy at the end of each quarter.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2014	**2013**	**2014**	**2013**
Revenues:				
Net product revenue	$636	—	$717	—
Collaboration revenue	10,966	3,931	22,246	31,326
Grant revenue	1,056	480	2,282	3,370
Total revenues	12,658	4,411	25,245	34,696
Operating expenses:				
Research and development (1)	26,871	15,020	79,838	54,875
General and administrative (1)	18,017	7,483	44,820	25,219
Total operating expenses	44,888	22,503	124,658	80,094
Loss from operations	(32,230)	(18,092)	(99,413)	(45,398)
Interest income (expense), net	406	166	1,180	(6,084)
Loss on extinguishment of debt	—	—	—	(130)
Other income (expense), net	(138)	41	(213)	38
Loss from operations before tax benefit	(31,962)	(17,885)	(98,446)	(51,574)
Net tax benefit	4,693	—	4,693	—
Net loss	(27,269)	(17,885)	(93,753)	(51,574)
Deemed dividend	—	—	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	—	—	3,391
Net loss attributable to common shareholders	($27,269)	($17,885)	($93,753)	($66,432)
Weighted-average shares outstanding (in shares):				
Basic and diluted	32,274,729	23,803,282	31,565,310	12,829,411
Net loss per share applicable to common stockholders - basic and diluted (in dollars per share)	($0.84)	($0.75)	($2.97)	($5.18)
(1) Non-cash share-based compensation expense included in operating expenses are as follows:				
Research and development	$3,221	$1,577	$9,739	$4,312
General and administrative	3,483	1,708	9,571	4,115
Total share-based compensation expense	$6,704	$3,285	$19,310	$8,427

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

	December 31, 2014	**December 31, 2013**
Cash, cash equivalents and marketable securities	$315,241	$142,467
Total assets	$333,219	$151,903
Total debt	-	49
Total deferred revenue	3,354	878
Total liabilities	$34,752	$15,361
Total stockholders' equity (32,898,392 and 23,803,282 common shares issued and outstanding at December 31, 2014 and December 31, 2013, respectively)	298,467	136,542
Total liabilities and stockholders' equity	$333,219	$151,903

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. 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 molecule is copied from DNA through the transcription process. PTC has received conditional marketing authorization in the European Economic Area for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged five years and older. 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

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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 of Translarna for the treatment of nmDMD, nmCF and nmMPS I, as well as trials in its SMA collaboration with Roche and the SMA Foundation and its cancer stem cell program, including statements regarding the timing of initiation, enrollment and completion of the trials and the period during which the results of the trials 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 nmDMD, 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; 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 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. 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-fourth-quarter-and-2014-full-year-financial-results-and-provides-corporate-update-300042686.html

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