



November 9, 2015

PTC Therapeutics Reports Third Quarter 2015 Financial Results, Provides Corporate Update and Reviews Key Findings from ACT DMD

- **152 DMD patients now on Translarna™ (ataluren) commercial therapy**
- **Strong sales growth with YTD 9/30 Translarna revenues of \$21.0M -**
- **Phase 3 ACT DMD clinical trial results confirm clinical benefit of Translarna -**
- **SMA program expected to resume clinical development in early 2016 -**

SOUTH PLAINFIELD, N.J., Nov. 9, 2015 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ending September 30, 2015.

"We recently reported results from our Phase 3 ACT DMD clinical trial and are now actively finalizing our regulatory submissions to both the FDA and EMA, which we plan to complete by the end of this year," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Our ongoing launch of Translarna for Duchenne muscular dystrophy across Europe and other parts of the world continues to perform well, and based on the consistently positive feedback we are receiving from key opinion leaders, physicians, and patient advocacy groups regarding our ACT DMD results, we expect this momentum to build."

Key Third Quarter 2015 Corporate Highlights:

- ▮ **Translarna now available on a commercial basis in 13 countries.** As of November 5th, there were 152 DMD patients on commercial therapy, including patients from both direct commercial sales and reimbursed early access programs. Translarna received marketing authorization from the European Medicines Agency (EMA) in August 2014 for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) in ambulatory patients aged 5 and over, representing the first-ever treatment approved for the underlying cause of the disease. More than 550 DMD patients are currently receiving Translarna therapy either through open-label extension studies or commercial access.
- ▮ **ACT DMD results confirm clinical benefit of Translarna in nonsense mutation Duchenne muscular dystrophy.** On October 15th, PTC announced results from the Phase 3 ACT DMD clinical trial of Translarna in patients with nmDMD. The totality of the clinical data from two large, placebo-controlled clinical trials across over 400 patients demonstrates Translarna's ability to slow disease progression. Today, on PTC's quarterly investor call the Company will review key findings from the ACT DMD clinical trial. In the overall intent-to-treat population, the primary endpoint of change from baseline in the 6-minute walk test (6MWT) demonstrated a 15 meter benefit ($p=0.213$), which was not statistically significant. A benefit of 47 meters (nominal $p=0.007$) was demonstrated in the pre-specified patient population of 300-400 meters at baseline as measured by the 6MWT, which is in line with the Company's prior experience in its Phase 2b trial and consistent with the evolving understanding of the 6MWT in DMD. A meta-analysis of the combined data from ACT DMD and the ambulatory decline phase patients from the Phase 2b trial demonstrate a statistically significant benefit for Translarna across the primary (6MWT) and key secondary endpoints (timed function tests). PTC plans to complete its rolling new drug application (NDA) submission to the U.S. Food and Drug Administration (FDA) and to submit the data to the EMA by the end of 2015.
- ▮ **ACT CF Phase 3 clinical trial on track with enrollment to be completed by the end of 2015.** Enrollment of the ACT CF Phase 3 clinical trial has been extremely robust due to high patient demand. Screening for the study has now closed, and enrollment will be completed before the end of 2015, with topline data expected about a year later. During the third quarter, PTC submitted a variation to its marketing authorization of Translarna to the EMA to request approval of Translarna for the treatment of cystic fibrosis, a potential second indication in the European Union.
- ▮ **Update on additional indications for Translarna.** Given its mechanism of action, Translarna has the potential to address numerous genetic disorders caused by a nonsense mutation. In addition to its advanced DMD and CF programs, PTC is pursuing two additional indications, MPS I and aniridia, for which Translarna has received orphan drug designations from both the FDA and EMA. In May of this year, PTC amended the MPS I clinical trial protocol to allow patients currently using enzyme replacement therapy to be included in the trial. This protocol revision resulted in delays to opening clinical trial sites and accruing patients. As a result, PTC now expects data for Translarna in MPS I in 2016. 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.

- SMA program update.** Clinical data from the first cohort of the Phase 2a Moonfish study was recently presented at the 20th International Annual Congress of the World Muscle Society. This data demonstrated that treatment with RG7800 shifts SMN2 splicing toward the production of full length SMN mRNA and generated up to two-fold increases in SMN protein in patients with SMA. Pre-clinical investigations regarding our lead compound, RG7800, are ongoing after the observation of an unexpected finding in a chronic animal safety study in April. Concurrent with the advancement of our lead compound, a robust research effort regarding SMN2 splicing has continued to advance through IND-enabling studies. Additional data are expected in the coming months, which will be utilized to determine the best clinical development path forward for the SMA program. PTC and the program collaborators remain highly committed to this program and expect that clinical development will resume in early 2016.

Upcoming Events:

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

- Credit Suisse 24th Annual Healthcare Conference, November 11th in Scottsdale, AZ
- Stifel 2015 Healthcare Conference, November 18th in New York, NY
- Oppenheimer 26th Annual Healthcare Conference, December 9th in New York, NY

Third Quarter 2015 Financial Highlights:

- Translarna net product sales were \$9.8 million for the third quarter of 2015, representing 59% sequential growth versus \$6.2 million in net product sales in the second quarter of 2015. Translarna has generated \$21.0 million in net product sales through the first three quarters of 2015.
- Total revenues for the third quarter of 2015 were \$9.8 million. This compared to total revenue in the third quarter of 2014 of approximately \$1.7 million. The increase in total revenue was a result of the commercial launch of Translarna, which received marketing authorization from the EMA in August 2014, partially offset by lower grant revenue.
- Research and development expenses were \$30.6 million for the third quarter of 2015, including \$3.8 million in non-cash, stock-based compensation expense, compared to \$18.8 million for the same period in 2014, including \$2.4 million in non-cash, stock-based compensation expense. The increase in R&D expense for the third quarter 2015 as compared to the prior year period was primarily due to expansion of our clinical development activities including late stage studies in both Duchenne muscular dystrophy and cystic fibrosis.
- Selling, general and administrative expenses were \$21.4 million for the third quarter of 2015, including \$4.2 million in non-cash, stock-based compensation expense, compared to \$10.5 million for the same period in 2014, including \$2.3 million in non-cash, stock-based compensation expense. The increase in SG&A expense for the third 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 interest expense for the third quarter of 2015 was \$0.9 million compared to net interest income of \$0.4 million in the same period in 2014. The increase in interest expense is 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 third quarter of 2015 was \$43.2 million compared to a net loss of \$27.3 million for the same period in 2014.
- Cash, cash equivalents, and marketable securities totaled \$371.5 million at September 30, 2015 compared to \$315.2 million at December 31, 2014. This includes net proceeds of approximately \$145.4 million from a \$150 million convertible debt offering completed in the third quarter.
- Shares issued and outstanding as of September 30, 2015 were 34.3 million, which includes 0.4 million shares of unvested restricted stock.

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

| Three Months Ended | | Nine Months Ended | |
|--------------------|------|-------------------|------|
| September 30, | | September 30, | |
| 2015 | 2014 | 2015 | 2014 |

Revenues:

| | | | | |
|---|--------------------|--------------------|---------------------|--------------------|
| Net product revenue | \$ 9,772 | \$ 81 | \$ 21,002 | \$ 81 |
| Collaboration revenue | 2 | 716 | 547 | 11,280 |
| Grant revenue | 2 | 897 | 2,483 | 1,226 |
| Total revenues | 9,776 | 1,694 | 24,032 | 12,587 |
| Operating expenses: | | | | |
| Research and development (1) | 30,640 | 18,765 | 86,768 | 52,967 |
| Selling, general and administrative (1) | 21,368 | 10,530 | 56,193 | 26,803 |
| Total operating expenses | 52,008 | 29,295 | 142,961 | 79,770 |
| Loss from operations | (42,232) | (27,601) | (118,929) | (67,183) |
| Interest (expense)/income | (852) | 354 | 170 | 774 |
| Other expense, net | (51) | (35) | (507) | (75) |
| Loss before income tax expense | (43,135) | (27,282) | (119,266) | (66,484) |
| Income tax expense | (88) | — | (233) | — |
| Net loss | <u>\$ (43,223)</u> | <u>\$ (27,282)</u> | <u>\$ (119,499)</u> | <u>\$ (66,484)</u> |

Weighted-average shares outstanding (in shares):

| | | | | |
|---|-------------------|-------------------|-------------------|-------------------|
| Basic and diluted | <u>33,908,853</u> | <u>29,351,693</u> | <u>33,528,833</u> | <u>28,441,827</u> |
| Net loss per share - basic and diluted (in dollars per share) | <u>\$ (1.27)</u> | <u>\$ (0.93)</u> | <u>\$ (3.56)</u> | <u>\$ (2.34)</u> |

(1) Non-cash share-based compensation expense included in operating expenses are as follows:

| | | | | |
|--|----------|----------|-----------|-----------|
| Research and development | \$ 3,828 | \$ 2,363 | \$ 12,452 | \$ 6,517 |
| Selling, general and administrative | 4,226 | 2,258 | 13,678 | 6,088 |
| Total share-based compensation expense | \$ 8,054 | \$ 4,621 | \$ 26,130 | \$ 12,605 |

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

| | September 30, 2015 | December 31, 2014 |
|---|-----------------------|----------------------|
| Cash, cash equivalents and marketable securities | \$ 371,521 | \$ 315,241 |
| Total assets | \$ 396,100 | \$ 333,219 |
| Total debt | \$ 93,198 | \$ - |
| Total deferred revenue | - | 3,354 |
| Total liabilities | \$ 126,949 | \$ 34,752 |
| Total stockholders' equity (33,915,059 and 32,898,392 common shares issued and outstanding at September 30, 2015 and December 31, 2014, respectively) | \$ 269,151 | \$ 298,467 |
| Total liabilities and stockholders' equity | \$ 396,100 | \$ 333,219 |

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 60395335. The accompanying slide presentation will be posted 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. The webcast and slide presentation 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

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, are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC; the timing of PTC's planned regulatory filings, including with the FDA, the EMA and other regulatory bodies outside of the United States and European Economic Area, or EEA; 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; 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 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 strategy, future operations, future financial position, future revenues or projected costs; and 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 outcome of final analyses of the data from ACT DMD, which may vary from PTC's initial analysis, lead to different (including more or less favorable) interpretations of the results than the analyses conducted to date, and identify further important information that is not available at the time of this press release; whether the FDA or the EMA or other regulators agree with PTC's interpretation of the results of ACT DMD; expectations for regulatory approvals, including PTC's ability to make regulatory submissions in a timely manner (or at all), 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™ (ataluren) for the treatment of nonsense mutation DMD in the EEA, which is conditioned upon, among other things, completion of ACT DMD and submission of the final report, including additional efficacy and safety data from ACT DMD, during 2015 and 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 commercialize Translarna and commercially manufacture 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 United States, EEA and other territories; 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 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-third-quarter-2015-financial-results-provides-corporate-update-and-reviews-key-findings-from-act-dmd-300175016.html>

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