



July 30, 2015

PTC Therapeutics Reports Second Quarter 2015 Financial Results and Provides Corporate Update

- **106 DMD patients now on Translarna™ (ataluren) commercial therapy**
- **Strong Translarna sales growth with YTD 6/30 revenues of \$11.2M -**
- **New analyses of previous CF Phase 3 data show improved response in younger patients -**

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

"PTC is progressing on many fronts across the organization. We are excited to be near the completion of the largest Duchenne muscular dystrophy clinical trial ever conducted with topline results expected in the fourth quarter," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "Translarna is now commercially available in 12 countries and we have a presence in over 30 countries. We are also planning to submit an application for Translarna to treat nonsense mutation cystic fibrosis to the European Medicines Agency by the end of this year. This submission will include new and important analyses of our CF data."

Dr. Peltz continued, "This is just the beginning. There are many nonsense-mutation-based disorders with high unmet medical need where no existing treatment is available. To deliver on our commitment of bringing hope to patients with rare and neglected genetic disorders and to maximize the potential of Translarna, our goal is to commence clinical investigations of at least ten new indications by the year 2020."

Key Second Quarter 2015 Corporate Highlights:

- 1 **Translarna now available on a commercial basis in 12 countries.** As of July 28th, there are 106 DMD patients on commercial therapy, including patients from both direct commercial sales and reimbursed early access programs. New countries recently added include Denmark, Norway, and Brazil. Translarna received marketing authorization from the European Medicines Agency in August 2014 for the treatment of nonsense mutation Duchene muscular dystrophy in ambulatory patients aged 5 and over, representing the first-ever treatment approved for the underlying cause of the disease.
- 1 **Top-line data from Phase 3 ACT DMD expected in the fourth quarter of 2015.** In December 2014, PTC began submitting a rolling new drug application (NDA) 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. PTC anticipates a potential US approval and commercial launch of Translarna for nonsense mutation DMD in the first half of 2016.
- 1 **New analyses of previous Phase 3 CF data demonstrates greater response in younger patients not receiving chronic inhaled tobramycin.** Natural history data indicate that patients under 18 years of age experience more rapid rates of decline in pulmonary function. In preparation for the European application for Translarna for the treatment of nonsense mutation cystic fibrosis and in consultation with European thought leaders, PTC has performed additional analyses of the data from its previous Phase 3 trial completed in 2011. The subgroup of non-TOBI patients under 18 years of age experienced a robust treatment response with an increase in FEV1 and a 5.4% absolute benefit in FEV1 as well as a 60% reduction in pulmonary exacerbations rates versus placebo.
- 1 **Enrollment of Phase 3 ACT CF trial on track to be completed by the end of 2015.** ACT CF is expected to enroll approximately 208 patients with nonsense mutation cystic fibrosis. Top-line data are expected to be available by the end of 2016.
- 1 **PTC outlines Ten by '20 strategy for Translarna pipeline expansion.** 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 now pursuing two additional indications, MPS I and aniridia. 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.
- 1 **SMA program and Phase 2 MOONFISH study update.** Dosing of the first cohort in the Phase 2 MOONFISH study was successfully completed earlier in the year. RG7800 was well tolerated and preliminary review of the blinded data indicated substantial increases in full length SMN2 mRNA. As previously announced, an eye finding was detected in a longer term preclinical animal study at concentrations above those explored in patients. This finding was not observed in humans. As a precautionary measure, dosing of additional patients was suspended while the finding is investigated.

Analyses completed to date, combined with additional preclinical data expected later this fall, could potentially allow dosing to resume in patients in the first quarter of 2016.

Upcoming Events:

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

- ┆ Wedbush 2015 PacGrow Healthcare Conference August 12th in New York, NY
- ┆ Citi 10th Annual Biotech Conference September 10th in New York, NY
- ┆ J.P. Morgan 6th Annual "All Stars" Conference September 16th in London, UK
- ┆ Bank of America Global Healthcare Conference September 17th in London, UK

Second Quarter 2015 Financial Highlights:

- ┆ Translarna net product sales were \$6.2 million for the second quarter of 2015, representing 69% sequential growth versus \$3.7 million in adjusted net product sales in the first quarter of 2015. Translarna net product sales in first quarter of 2015 included \$1.4 million of revenue which was deferred during 2014 when we recognized revenue on a cash basis.
- ┆ Total revenues for the second quarter of 2015 were \$6.8 million, including \$6.2 million in Translarna net product sales revenue and \$0.6 million in grants and collaborations revenue. This compared to total revenue in the second 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 European Medicines Agency in August 2014.
- ┆ Research and development expenses were \$28.2 million for the second quarter of 2015, including \$4.0 million in non-cash, stock-based compensation expense, compared to \$18.3 million for the same period in 2014, including \$2.2 million in non-cash, stock-based compensation expense. The increase in R&D expense for the second quarter 2015 as compared to the prior year period was primarily due to additional costs associated with our ongoing clinical trials as well as our expanding clinical-stage pipeline.
- ┆ Selling, general and administrative expenses were \$17.2 million for the second quarter of 2015, including \$4.4 million in non-cash stock-based compensation expense, compared to \$8.7 million for the same period in 2014, including \$2.1 million in non-cash stock-based compensation expense. The increase in SG&A expense for the second 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 second quarter of 2015 was \$38.4 million compared to a net loss of \$25.1 million for the same period in 2014.
- ┆ Cash, cash equivalents, and marketable securities totaled \$255.2 million as of June 30, 2015 compared to \$315.2 million as of December 31, 2014.
- ┆ Shares issued and outstanding as of June 30, 2015 were 34.2 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 June 30,		Six Months Ended June 30,	
	2015	2014	2015	2014
Revenues:				
Net product revenue	\$6,161	\$ -	\$11,230	\$ -
Collaboration revenue	207	1,418	545	10,565
Grant revenue	406	259	2,481	329
Total revenues	6,774	1,677	14,256	10,894
Operating expenses:				
Research and development (1)	28,190	18,313	56,128	34,202
Selling, general and administrative (1)	17,210	8,733	34,825	16,273
Total operating expenses	45,400	27,046	90,953	50,475
Loss from operations	(38,626)	(25,369)	(76,697)	(39,581)
Interest income	498	248	1,022	419
Other (expense)/income, net	(88)	17	(456)	(40)
Loss before income tax expense	(38,216)	(25,104)	(76,131)	(39,202)
Income tax expense	(145)	—	(145)	—
Net loss	<u>(\$38,361)</u>	<u>(\$25,104)</u>	<u>(\$76,276)</u>	<u>(\$39,202)</u>

Weighted-average shares outstanding (in shares):				
Basic and diluted	<u>33,600,653</u>	<u>29,332,227</u>	<u>33,335,674</u>	<u>27,976,847</u>
Net loss per share - basic and diluted (in dollars per share)	<u>(\$1.14)</u>	<u>(\$0.86)</u>	<u>(\$2.29)</u>	<u>(\$1.40)</u>

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

Research and development	\$3,957	\$2,209	\$8,624	\$4,153
Selling, general and administrative	4,371	2,069	9,452	3,830
Total share-based compensation expense	\$8,328	\$4,278	\$18,076	\$7,983

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

	June 30, 2015	December 31, 2014
Cash, cash equivalents and marketable securities	\$255,219	\$315,241
Total assets	\$273,822	\$333,219
Total deferred revenue	—	3,354
Total liabilities	\$25,241	\$34,752
Total stockholders' equity (33,872,956 and 32,898,392 common shares issued and outstanding at June 30, 2015 and December 31, 2014, respectively)	248,581	298,467
Total liabilities and stockholders' equity	\$273,822	\$333,219

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 86272093.

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, 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, 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 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.

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