November 6, 2014

PTC Therapeutics Reports Third Quarter Financial Results and Provides Corporate Update

- Confirmatory Phase 3 ACT DMD clinical trial fully enrolled -
- Rolling NDA submission for Translarna in nmDMD planned before year end 2014 -
- European commercial launch activities well underway -
- Initiation of Phase 1b/2a trial for SMA program planned -
- IND application for PTC596 to be submitted before year end 2014 -
- Successful financing raised estimated net proceeds of $117.5 million -

SOUTH PLAINFIELD, N.J., Nov. 6, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ended September 30, 2014.

"During the quarter we continued to make significant progress during this transformative time for PTC. For the first time in the company's history, we recognized revenue from the sale of Translarna™ (ataluren) for the treatment of Duchenne muscular dystrophy through our European reimbursed early access programs. Furthermore, before the end of the year, we plan to begin submitting a New Drug Application with the FDA on a rolling basis for Translarna as a treatment for nonsense mutation Duchenne muscular dystrophy (nmDMD). We are committed to making Translarna available to all patients who may benefit and this is another step forward in achieving this goal," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We continued to expand our clinical efforts with the completion of enrollment for our confirmatory Phase 3 ACT DMD clinical trial, the initiation of our confirmatory Phase 3 ACT CF clinical trial, the preparation of a Phase 2 proof-of-concept study of Translarna in MPS I, and the anticipated initiation of a Phase 1b/2a clinical trial of RG7800 in spinal muscular atrophy patients in conjunction with our partners, Roche and the SMA Foundation. Our discovery efforts are also progressing with plans to file an investigational new drug application, or IND, with the FDA for PTC596, our cancer stem cell program, and the declaration of a development candidate in our antibacterial program. After recently completing a successful public equity offering where we raised net proceeds of approximately $117.5 million, PTC is well positioned to realize our goal of discovering, developing and commercializing new treatments that have the potential to transform the lives of patients."

Corporate Highlights:

- **ACT DMD:** The confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) trial evaluating Translarna™ in patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) completed enrollment. Top-line data from the trial is expected in the fourth quarter of 2015.
- **ACT CF:** The confirmatory Phase 3 ACT CF (Ataluren Confirmatory Trial in CF) trial evaluating Translarna™ in patients with nonsense mutation cystic fibrosis is underway. Enrollment is expected to be completed in the second half of 2015, with data approximately one year later.
- **Regulatory update:** PTC plans to submit a New Drug Application to the FDA on a rolling basis for Translarna™ as a treatment for nonsense mutation Duchenne muscular dystrophy (nmDMD) in the U.S. before the end of 2014.
- **Commercial launch:** Commercial activities are ongoing in support of the anticipated launch of Translarna across Europe, subject to the completion of each country's market access process and timeline. PTC expects to initially launch in Germany within the next few months, with other key countries expected to follow throughout 2015 and beyond.
- **Reimbursed Early Access Programs:** Reimbursed early access programs (EAP) are well underway and the company has already begun to receive payments and recognize revenue in connection with the initiation of supply of Translarna to patients under these programs.
- **SMA program:** Initiation of a Phase 1b/2a, multi-center, randomized, double-blind, placebo-controlled, multiple-dose clinical trial in SMA patients is anticipated in the coming weeks in conjunction with our partners, Roche and the SMA Foundation. PTC expects to receive a $10 million milestone payment from Roche upon initiation of the study.
- **Oncology program:** Successfully completed IND-enabling studies for PTC596, an orally active small molecule that targets drug-resistant tumor stem cell populations. An investigational new drug (IND) application for PTC596 is expected to be submitted before the end of 2014 with plans to initiate a Phase 1 clinical trial in the first half of 2015.
- **Antibacterial program:** We have identified and are chemically optimizing several small molecule compounds for the treatment of life-threatening infections caused by multidrug-resistant Gram-negative bacteria. In this program, we recently selected a Development Candidate for the treatment of MDR gonorrhea.
- **Financing:** Recently completed a successful public offering of common stock which raised net proceeds of approximately $117.5 million. This additional capital is expected to help fund the business through late 2017.
- **Key appointments:** PTC continued to expand its team in preparation for the launch of Translarna in the European

Union and the expansion of our clinical programs. Senior executives with strong rare disease and commercial launch experience have been appointed in European regional roles as well as across key countries including Germany, the UK, France, Italy and the Nordic Region. Leadership is also being added across the organization including clinical development, regulatory affairs, medical affairs, supply chain and quality.

- **International headquarters**: During fiscal 2014, PTC established its international headquarters in Dublin, Ireland. This office will serve as the central hub for the commercial launch of Translarna to take advantage of its location with respect to PTC's third party manufacturing and supply chain. In addition, PTC has begun establishing subsidiaries in key European countries where Translarna is expected to initially become commercially available.
- **Upcoming Events:** PTC's Annual Science Day will be held on November 24th in New York City. PTC will also be participating in the following conferences in the fourth quarter:
  - Credit Suisse Healthcare Conference on November 11th in Arizona.
  - Panel at the Boston Biotech's CEO Conference on November 12th in New York.
  - Deutsche Bank BioFEST on December 2nd in Boston.
  - Panel at the Genetic Rx Conference on December 3rd in Boston.
  - Oppenheimer Annual Healthcare Conference on December 10th-11th in New York.

Third Quarter 2014 Financial Highlights:

- Cash, cash equivalents, and marketable securities totaled $209.4 million at September 30, 2014 compared to $142.5 million at December 31, 2013. Pro forma for PTC's recent public offering of common stock, cash, cash equivalents and marketable securities totaled $326.9 million as of September 30, 2014.
- Revenue of $0.1 million from net product sales of Translarna was recognized for the first time in the history of the company during the third quarter of 2014. Revenue from grants and collaborations was $1.6 million for the third quarter of 2014, compared to $16.3 million for the same period in 2013. The decrease was due to a milestone payment of $10 million that was recognized in the third quarter of 2013 related to the SMA collaboration as well as a decrease in the recognition of non-cash deferred revenue.
- Research and development expenses were $18.8 million for the third quarter of 2014, including $2.4 million in non-cash, stock-based compensation expense, compared to $13.9 million for the same period in 2013, including $0.7 million in non-cash, stock-based compensation expense. The increase primarily resulted from the additional stock-based compensation expense and an increase in clinical trial expenses.
- General and administrative expenses were $10.5 million for the third quarter of 2014, including $2.3 million in non-cash stock-based compensation expense, compared to $6.7 million for the same period in 2013, including $2.0 million in non-cash stock-based compensation expense. The increase primarily results from additional costs associated with commercial activities in support of the anticipated launch of Translarna across Europe and legal costs associated with establishing our international infrastructure.
- Net loss for the third quarter of 2014 was $27.3 million compared to a net loss of $4.4 million for the same period in 2013.
- Shares issued and outstanding as of September 30, 2014 were 30.1 million, which includes 0.7 million shares of unvested restricted stock. Pro forma for our recently completed public offering of common stock, shares issued and outstanding were 33.6 million.
- Based on our current run-rate of expenses, we now expect total cash operating expenses to be between $93 million and $103 million, excluding expected non-cash stock-based compensation of approximately $17 million, for total operating expenses of approximately $110 million to $120 million. As a result of our recent financing, we now expect to end 2014 with approximately $295 million to $305 million in cash, cash equivalents and marketable securities.

Today's Conference Call and Webcast Reminder:
The PTC management team will host a conference call to discuss the company's financial results and recent and upcoming developments today, Thursday, November 6, 2014, at 9:00 a.m. ET. 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 21354324.

The live, listen-only webcast of the conference call can also be accessed by visiting the "Investors Relations" section of the company's website at ir.ptcbio.com. A replay of the webcast will be archived on the PTC website for 30 days following the call.

About PTC Therapeutics, Inc.
PTC is a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are essential to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and in collaborations with leading biopharmaceutical companies. 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. Translarna is an investigational new drug in the United States. 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 our commercial launch; our Phase 3 clinical trials for Translarna™ (ataluren) in nmDMD and nmCF; our Phase 2 proof-of-concept study in MPS I; our collaboration in SMA with Roche and the SMA Foundation including anticipated milestone payments; our current and planned regulatory filings, including with the FDA and the EMA; our earlier stage programs, including BMI1 and our antibacterial program; our strategy, future operations, future financial position, future revenues or projected costs; the development of and potential market for Translarna and our other product candidates; 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.

Our actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements we make as a result of a variety of risks and uncertainties, including those related to the initiation and conduct of clinical trials, availability of data from clinical trials, expectations for regulatory approvals, our scientific approach and general development progress, the availability or commercial potential of Translarna and our other product candidates and the factors discussed in the "Risk Factors" section of our most recent Quarterly Report on Form 10-Q in PTC's most recent Quarterly Report as well as any updates to these risk factors filed from time to time in PTC's other filings with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent PTC's views only as of the date of this press release. 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 we do 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.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2014	**2013**	**2014**	**2013**
Revenues:				
Net product sales	$81	—	$81	—
Collaboration revenue	$716	15,455	$11,280	27,395
Grant revenue	897	834	1,226	2,890
Total revenues	1,694	16,289	12,587	30,285
Operating expenses:				
Research and development (1)	18,765	13,886	52,967	39,855
General and administrative (1)	10,530	6,679	26,803	17,735
Total operating expenses	29,295	20,565	79,770	57,590
Loss from operations	(27,601)	(4,276)	(67,183)	(27,305)
Interest income (expense), net	354	27	774	(6,250)
Loss on extinguishment of debt	—	(130)	—	(130)
Other income (expense), net	(35)	(37)	(75)	(3)
Net loss	(27,282)	(4,416)	(66,484)	(33,688)
Deemed dividend	—	—	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	—	—	3,391
Net loss attributable to common shareholders	($27,282)	($4,416)	($66,484)	($48,546)

Weighted-average shares outstanding (in shares):				
Basic and diluted	29,351,693	23,803,282	28,441,827	8,995,167
Net loss per share applicable to common stockholders - basic and diluted (in dollars per share)	($0.93)	($0.19)	($2.34)	($5.40)

(1) Non-cash share-based compensation expense included in operating expenses are as follows:				
Research and development	$2,363	$677	$6,517	$1,378
General and administrative	2,258	1,963	6,088	3,764
Total share-based compensation expense	$4,621	$2,640	$12,605	$5,142

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

	September 30, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	$209,389	$142,467
Total assets	$220,795	$151,903
Total debt	-	49
Total deferred revenue	769	878
Total liabilities	$19,499	$15,361
Total stockholders' equity (29,373,327 and 23,803,282 common shares issued and outstanding at September 30, 2014 and December 31, 2013, respectively)	201,296	136,542
Total liabilities and stockholders' equity	$220,795	$151,903

SOURCE PTC Therapeutics, Inc.