August 7, 2014

PTC Therapeutics Reports Second Quarter Financial Results and Provides Corporate Update

-Conference Call Today at 8:30 am ET-

SOUTH PLAINFIELD, N.J., Aug. 7, 2014 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ended June 30, 2014.

"This has been an exciting quarter for PTC. In May we received a positive opinion from the CHMP for marketing approval for Translarna™ (ataluren) an investigational new drug in the US, for nonsense mutation Duchenne muscular dystrophy and we recently received the approval from the European Commission granting the conditional marketing authorization. It is an honor to bring the world's first therapy for Duchenne muscular dystrophy to patients who have been waiting too long for a treatment. We are actively focused on providing Translarna to patients as quickly as possible and are working aggressively to prepare for launch across the EU," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "In addition to our efforts in nmDMD, we recently initiated our confirmatory Phase 3 clinical trial in nonsense mutation cystic fibrosis and it is our goal to initiate a Phase 2 proof-of-concept study for Translarna in a new indication, MPS I caused by a nonsense mutation, later this year. We expect the second half of the year will be a transformative time at PTC, as we expand our global commercial organization focused on our mission of bringing new therapies to patients with rare and neglected disorders."

Corporate Highlights:

- **ACT DMD:** The confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) trial of Translarna™ (ataluren) in patients with nonsense mutation Duchenne muscular dystrophy (nmDMD) is well underway. Enrollment is expected to be completed near term with initial, top-line data available in the second half of 2015.
- **ACT CF:** In June, the confirmatory Phase 3 ACT CF clinical trial was initiated. This trial is a 48-week, double-blind, placebo-controlled global study with FEV1 as the primary endpoint and pulmonary exacerbations as a key secondary endpoint. The trial will enroll patients who have a confirmed nonsense mutation, who are six years of age or older and whose FEV1 is greater than 40% and less than 90% predicted. Patients who are using chronic inhaled aminoglycosides will not be eligible for the trial. Aminoglycosides are ribosome-binding drugs and have been shown to interfere with Translarna's activity. Enrollment is expected to be completed in the second half of 2015, with data expected a year later.
- **Additional indication:** Based on an evaluation process and in discussion with outside experts, PTC has selected mucopolysaccharidosis type I (MPS I) as the next indication to pursue for Translarna. It is PTC's goal to initiate a Phase 2 proof-of-concept study for MPS I in the second half of 2014. MPS I is an inherited genetic disorder caused by a deficiency in an essential enzyme that is responsible for the breakdown of by-products of chemical reactions in the body's cells. Globally, MPS I occurs in about 1 in every 100,000 births. It is estimated that 60-80% of MPS I patients have their disease as a result of a nonsense mutation. There is no cure for MPS I and enzyme replacement therapies do not sufficiently address the central nervous system, skeletal or cardiac symptoms associated with the disorder. Prognosis of patients with MPS I is poor and there is an urgent need for the development of new treatments targeting the underlying cause of MPS I.
- **Regulatory update:** In May 2014, the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) adopted a positive opinion regarding the application for the conditional marketing authorization of Translarna for the treatment of nmDMD in ambulatory patients aged 5 years and older. On August 4th, PTC was notified that the European Commission ratified the CHMP's positive opinion and Translarna was granted conditional marketing approval in the EU. In addition to our efforts in the EU, PTC is engaging in further dialogue with the FDA to discuss potential pathways to accelerate bringing Translarna to US patients.
- **Commercialization Plans:** Commercial launch activities have been initiated to support the anticipated launch of Translarna in selected countries in the first half of 2015, subject to the completion of each country's market access process and timeline. Market access timelines vary from country to country and can take over eighteen months in certain countries.
- **EAP Program:** In June 2014, PTC initiated a reimbursed expanded access program for Translarna for nmDMD patients in selected territories. The EAP program is intended to make Translarna available to patients before commercial product becomes available in specific countries in accordance with local regulations. Funded Named Patient Programs have already been authorized in Turkey and Spain, and the French National Agency for Medicines and Health Products Safety (ANSM) has recently granted a Temporary Authorization for Use (Autorisation Temporaire d'Utilisation de cohort - ATU) of Translarna in a cohort of nmDMD patients. PTC has recently initiated the supply of Translarna to the first patients authorized under the EAP program.
- **SMA Program:** In January 2014, a Phase 1a single ascending dose, placebo-controlled clinical trial in healthy

volunteers was initiated. The primary objectives of this trial were to explore safety and pharmacokinetics of the drug candidate, RG7800. This trial has now completed and a multiple dose clinical trial in SMA patients is currently in preparation. Preliminary findings in the Phase 1a study indicate that RG7800 was well-tolerated at all dose levels studied. There were no deaths, serious adverse events (SAEs) or withdrawals due to adverse events (AEs) and no dose-related trends were identified. Additionally, RG7800 demonstrated a dose-dependent effect on SMN2 splicing, as shown by a change in the ratio of full-length SMN2 mRNA to SMN2 mRNA without exon 7 (SMND7), which may be interpreted as proof of mechanism in terms of the expected pharmacodynamic effect.

Second Quarter 2014 Financial Highlights:

- Cash, cash equivalents, and marketable securities totaled $226.9 million at June 30, 2014 compared to $142.5 million at December 31, 2013.
- Revenue from grants and collaborations was $1.7 million for the second quarter of 2014, compared to $6.9 million for the same period in 2013. The decrease was due to a decrease in the recognition of non-cash deferred revenue compared to the same period in 2013.
- Research and development expenses were $18.3 million for the second quarter of 2014, including $2.2 million in non-cash, stock based compensation expense, compared to $14.7 million for the same period in 2013, including $1.1 million in non-cash, stock-based compensation expense. The increase primarily results from additional costs associated with clinical trials including the manufacturing of drug product for our clinical trials and regulatory costs associated with the efforts to obtain conditional approval for Translarna in Europe.
- General and administrative expenses were $8.7 million for the second quarter of 2014, including $2.1 million in non-cash stock based compensation expense, compared to $6.6 million for the same period in 2013, including $0.8 million in non-cash stock based compensation expense. The increase primarily results from additional costs associated with efforts to obtain conditional approval for Translarna in Europe, pre-commercial activities and public company costs.
- Net loss for the second quarter of 2014 was $25.1 million compared to a net loss of $14.6 million for the same period in 2013.
- Shares issued and outstanding as of June 30, 2014 were 30.1 million, which includes 0.7 million shares of unvested restricted stock.
- In conjunction with the European approval and ongoing commercial launch activities, PTC now expects total 2014 operating expenses to be between $103 million and $113 million, excluding approximately $17 million in non-cash stock-based compensation. PTC expects to end 2014 with approximately $160 million to $170 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, August 7, 2014, at 8:30 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 75347535.

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. For more information on the company, please visit our website www.ptcbio.com

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 ataluren in nmDMD and nmCF; our Phase 2 proof of concept trial in MPS I; our collaboration in SMA with Roche and the SMA Foundation; our current and planned regulatory filings; 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 PTC's 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 our product candidates and the factors discussed in the "Risk

Factors" section of our most recent Quarterly Report on Form 10-Q, which is on file with the Securities and Exchange Commission. 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 June 30,		Six Months Ended June 30,	
	2014	**2013**	**2014**	**2013**
Revenues:				
Collaboration revenue	$1,418	$5,868	$10,565	$11,940
Grant revenue	259	986	329	2,056
Total revenues	1,677	6,854	10,894	13,996
Operating expenses:				
Research and development (1)	18,313	14,712	34,202	25,969
General and administrative (1)	8,733	6,595	16,273	11,056
Total operating expenses	27,046	21,307	50,475	37,025
Loss from operations	(25,369)	(14,453)	(39,581)	(23,029)
Interest income (expense), net	248	(114)	419	(6,276)
Other income (expense), net	17	(19)	(40)	34
Net loss	(25,104)	(14,586)	(39,202)	(29,271)
Deemed dividend	—	—	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	—	—	3,391
Net loss attributable to common shareholders	($25,104)	($14,586)	($39,202)	($44,129)
Weighted-average shares outstanding (in shares):				
Basic and diluted	29,332,227	2,648,832	27,976,847	1,326,679
Net loss per share applicable to common stockholders - basic and diluted (in dollars per share)	($0.86)	($5.51)	($1.40)	($33.26)
(1) Non-cash share-based compensation expense included in operating expenses are as follows:				
Research and development	$2,209	$1,107	$4,153	$1,364
General and administrative	2,069	774	3,830	1,138
Total share-based compensation expense	$4,278	$1,881	$7,983	$2,502

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

	June 30, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	$226,859	$142,467
Total assets	$237,558	$151,903
Total debt	—	49
Total deferred revenue	242	878
Total liabilities	$13,802	$15,361
Total stockholders' equity (29,340,577 and 23,803,282 common shares issued and outstanding at June 30, 2014 and December 31, 2013, respectively)	223,756	136,542

Total liabilities and stockholders' equity	$237,558	$151,903

Logo - http://photos.prnewswire.com/prnh/20010919/PTCLOGO

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