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PTC Therapeutics Reports First Quarter Financial Results and Provides Corporate Update

-Conference Call Today at 4:30 pm ET-

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SOUTH PLAINFIELD, NJ - May 6, 2014 - PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported its financial results for the first quarter ended March 31, 2014.

"We are happy to report on the progress we have made in the first quarter of 2014 on each of the clinical, regulatory and corporate fronts. Our successful follow-on financing provides a strong foundation to advance our clinical programs and further PTC's pipeline of product candidates," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "As we look ahead, we are excited to complete enrollment in our confirmatory Phase 3 ACT DMD clinical trial in nonsense mutation Duchenne muscular dystrophy, initiate our confirmatory Phase 3 clinical trial in nonsense mutation cystic fibrosis, and advance our SMA program, partnered with Roche and the SMA Foundation. We also plan to pursue additional indications for ataluren beyond nmDMD and nmCF and expect to initiate a Phase 2 proof-of-concept study for an additional indication in the second half of this year."

Corporate Highlights:

- | **ACT DMD:** In April 2013, PTC initiated the confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) evaluating ataluren as a potential treatment for patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). This study, which is the largest clinical study ever conducted in DMD, is on-track to complete enrollment by mid-2014.
- | **ACT CF:** We are in the process of initiating the confirmatory Phase 3 ACT CF clinical trial. The design of our confirmatory Phase 3 trial is similar to the previous Phase 3 trial, with FEV1 as the primary endpoint and pulmonary exacerbations as a key secondary endpoint. The main change from the prior trial will be to exclude patients from enrolling who are using chronic inhaled tobramycin. Tobramycin is a ribosome binding drug that has been shown to confound ataluren's activity.
- | **Additional Indications:** There have now been 20 publications demonstrating ataluren's activity in a number of different disease models. We plan to initiate a Phase 2 proof-of-concept clinical trial for ataluren in an additional indication in the second half of 2014.
- | **EMA regulatory process:** In January 2014, the CHMP adopted a negative opinion regarding our application for the conditional marketing authorization of ataluren for the treatment of nmDMD. PTC has requested a re-examination of the CHMP opinion. A final decision from the CHMP is expected in the second quarter of 2014.
- | **SMA Program Entered Clinical Development:** In January 2014, a Phase 1 clinical program was initiated which triggered a \$7.5 million milestone payment from Roche to PTC. The SMA Phase 1 clinical program is complemented by ongoing natural history and biomarker observational studies in SMA patients.
- | **Completed Follow-on Financing:** In February, we closed a registered public offering of common stock, in which we raised gross proceeds of \$126.5 million, before deducting underwriting discounts and commissions and other offering expenses.

First Quarter 2014 Financial Highlights:

- | Cash, cash equivalents, and marketable securities totaled \$246.6 million at March 31, 2014 compared to \$142.5 million at December 31, 2013. The increase was primarily driven by net proceeds of \$118.2 million from PTC's follow-on equity financing as well as a \$7.5 million milestone achieved in our Roche collaboration.
- | Revenue from grants and collaborations was \$9.2 million for the first quarter of 2014, compared to \$7.1 million for the same period in 2013.
- | Total operating expenses were \$23.4 million for the first quarter of 2014, including \$3.7 million in non-cash, stock based compensation expense, compared to \$15.7 million for the same period in 2013, including \$0.6 million in non-cash, stock

based compensation expense. The increase primarily results from additional costs associated with clinical trials, pre-commercial activities and public company expenses.

- | Net loss for the first quarter of 2014 was \$14.1 million compared to a net loss of \$14.7 million for the same period in 2013.
- | Shares issued and outstanding as of March 31, 2014 were 30.1 million, which includes 0.7 million shares of unvested restricted stock.

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, Tuesday, May 6, 2014, at 4:30 p.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 33834345.

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 which form the basis for collaborations with leading biopharmaceutical 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, our Phase 3 clinical trials for ataluren in nmDMD and nmCF, our collaboration in SMA with Roche and the SMA Foundation, our current and planned regulatory filings with the EMA, the timing of the re-examination of the CHMP opinion, our earlier stage programs, including our initiation of a potential proof-of-concept study, 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 Annual Report on Form 10-K for the fiscal year ended December 31, 2013, 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.

Statements of Operations
(In thousands, except per share data)

	Three Months Ended	
	March 31,	
	2014	2013
Revenues:		
Collaboration revenue	\$9,147	\$6,072
Grant revenue	70	1,070
Total revenues	9,217	7,142
Operating expenses:		
Research and development (1)	15,889	11,257
General and administrative (1)	7,540	4,461
Total operating expenses	23,429	15,718
Loss from operations	(14,212)	(8,576)
Interest income (expense), net	171	(6,162)
Other (expense) income, net	(57)	54
Net loss	(14,098)	(14,684)
Deemed dividend	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	3,391
Net loss attributable to common shareholders	(\$14,098)	(\$29,542)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	24,492,487	4,526
Net loss per share applicable to common stockholders - basic and diluted (in dollars per share)	(\$0.58)	(\$6.527.30)

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

Research and development	\$1,944	\$257
General and administrative	1,761	364
Total share-based compensation expense	\$3,705	\$621

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

	March 31, 2014	December 31, 2013
Cash, cash equivalents and marketable securities	\$246,585	\$142,467
Total assets	\$255,840	\$151,903
Total debt	12	49
Total deferred revenue	492	878
Total liabilities	\$11,498	\$15,361
Total stockholders' equity (29,325,997 and 23,803,282 common shares issued and outstanding at March 31, 2014 and December 31, 2013, respectively)	244,342	136,542
Total liabilities and stockholders' equity	\$255,840	\$151,903