March 6, 2014

PTC Therapeutics Reports Fourth Quarter and Year-End 2013 Financial Results and Provides Corporate Update

-Conference Call Today at 4:30 pm ET-

SOUTH PLAINFIELD, NJ – March 6, 2014 – PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported its financial results for the fourth quarter and full-year ended December 31, 2013.

"2013 was a landmark year for PTC with significant accomplishments achieved across the organization. Our private financing early in the year combined with a very successful IPO positioned us to execute on our strategy of leveraging our scientific platform to bring potentially disease-modifying therapies to patients suffering from rare disorders," stated Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "As we look ahead to 2014, 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, into clinical development. We also plan to pursue additional indications for ataluren beyond nmDMD and nmCF and expect to initiate a proof-of-concept study for a third indication this year."

Recent Achievements

Ataluren
- Confirmatory Phase 3 ACT DMD clinical trial: In April 2013, PTC initiated the confirmatory Phase 3 ACT DMD (Ataluren Confirmatory Trial in DMD) clinical trial 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 with top-line data in mid-2015.
- EMA regulatory process: In December 2013, the European Medicines Agency (EMA) convened a scientific advisory group, or SAG, meeting as part of the regulatory review process followed by an oral explanation meeting with the Committee for Medicinal Products for Human Use (CHMP). We believe that both the SAG and oral explanation meetings allowed us and independent experts in the DMD field to provide information to the SAG and CHMP members about important aspects of our clinical data and trial design. In January 2014, the CHMP adopted a negative opinion recommending the refusal of the granting of the conditional marketing authorization for 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 Q2 2014.
- Ataluren-related publications: In August 2013, data published in the medical journal Muscle & Nerve demonstrated the clinical meaningfulness of the six-minute walk test as a primary endpoint to measure disease progression and walking ability in ambulatory Duchenne muscular dystrophy trials. The natural history data utilized for the analysis was obtained from PTC's Phase 2b clinical trial of ataluren in 174 patients, the first registration-directed, placebo-controlled, multi-national study of a new chemical entity for DMD. In December 2013, data published in PLOS One demonstrated that nmDMD patients treated with ataluren, experienced an increase in dystrophin expression. This data was obtained from PTC's Phase 2a open-label trial of ataluren in which change in full-length dystrophin expression, as assessed by immunofluorescent staining, was the primary endpoint. Additionally, multiple articles were published by independent investigators demonstrating ataluren's ability to read through premature stop codons in multiple animal and cell-based disease models.

SMA Program
- Selected Development Candidate for SMA Program: In August 2013, in collaboration with its partners, the SMA Foundation and Roche, a lead development candidate was declared in the spinal muscular atrophy (SMA) program. Selection of the development candidate triggered a $10 million milestone payment by Roche to PTC. In January 2014, a Phase 1 clinical program was initiated which triggered a $7.5 million milestone payment from Roche to PTC. In addition, the SMA clinical program is complemented by natural history and biomarker observational studies which are on-going in SMA patients.

Corporate
- Successful Completion of Initial Public Offering (IPO): In June 2013, PTC completed an IPO selling approximately 9.6 million shares of common stock at a public offering price of $15 per share, resulting in approximately $144.4 million in gross proceeds to the company, before deducting underwriting discounts and commissions and other offering expenses. In February 2014, PTC completed a public offering of approximately 5.2 million shares raising approximately $126.5 million in gross proceeds to the company, before deducting underwriting discounts and commissions and other offering expenses.
- Selected to join the Russell 2000 Index and the NASDAQ Biotechnology Index: In September 2013, PTC was added to the Russell 2000 Index. The Russell 2000® Index, a subset of the Russell 3000® Index, measures the performance of the small-

cap segment of the U.S. equity market. In December 2013, PTC was selected for addition to the NASDAQ Biotechnology Index, which is designed to track the performance of a set of NASDAQ-listed securities classified as either Biotechnology or Pharmaceuticals. ? Hosted first R&D Day: In October 2013, PTC hosted its first R&D day in New York City where PTC's senior management and research teams presented in-depth reviews of the company's scientific platforms and R&D programs.
- Expanded senior management team: Throughout the year, PTC added key members to our management team to enhance our clinical, financial and commercial efforts.

Fourth Quarter and Year-End Financial Results
- Revenue from grants and collaborations was $4.4 million for the fourth quarter of 2013, compared to $6.6 million for the same period in 2012. The decrease was primarily due to lower revenues recognized from deferred balances in the current period. Revenue from grants and collaborations was $34.7 million for the full year 2013, compared to $33.9 million for 2012. The increase primarily resulted from the achievement of a $10.0 million milestone related to the Roche agreement in July 2013, partially offset by a decrease in the recognition of the deferred revenue balance related to the value of the remaining performance obligations under our restructured agreement with Genzyme in 2012. Collaboration revenue includes deferred revenue from payments received in previous periods as well as payments received and recognized during the respective periods.
- Total operating expenses were $22.5 million for the fourth quarter of 2013, including $3.3 million in stock based compensation expense, compared to $12.7 million for the same period in 2012 including $0.5 million in stock based compensation expense. The increase was primarily due to higher research and development expenses related to the initiation of the confirmatory Phase 3 ACT DMD clinical trial, as well as higher general and administrative expenses related to public company expenses and pre-commercial activities. Total operating expenses for the full year 2013 were $80.1 million, including $8.4 million in stock-based compensation expenses, compared to $60.8 million for the same period in 2012, including $2.3 million in stock-based compensation expense. The increase was primarily due to the initiation of the confirmatory Phase 3 ACT DMD clinical trial, an increase in share-based compensation expense, and new expenses related to becoming a public company.
- Net loss for the fourth quarter of 2013 was $17.9 million compared to a net loss of $6.3 million for the same period in 2012. Net loss for the full year 2013 was $51.6 million, compared to $26.2 million for the same period in 2012. ? Cash, cash equivalents, and marketable securities totaled $142.5 million at December 31, 2013 compared to $2.7 million at December 31, 2012. The increase was primarily a result of receiving net proceeds of $60.8 million from PTC's equity private placement and $131.6 million from PTC's initial public offering in the first half of 2013 combined with receiving a $10.0 million milestone in the third quarter of 2013 from Roche for the selection of a development candidate in our SMA collaboration. ? Shares issued and outstanding as of December 31, 2013 were 24.9 million, which includes 1.1 million of unvested restricted stock. Pro forma for the completion of our recent public offering of common shares, shares issued and outstanding are 30.1 million.

2014 Financial Guidance
- PTC expects total 2014 operating expenses to be between $85 million and $95 million, excluding approximately $15 million in non-cash stock-based compensation. These expenses will be primarily in support of our ongoing and planned confirmatory Phase 3 clinical trials for ataluren in nmDMD and nmCF, our ongoing open-label studies, as well as the advancement of other product pipeline candidates including a proof-of-concept study for a third indication for ataluren. We believe this metric is useful to investors to understand management's view of our ongoing cash operating expenses.
- PTC expects to end 2014 with approximately $175 million to $185 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, March 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 3360375. 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 final prospectus dated February 12, 2014 for our public offering completed in February 2014, 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.

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

	Three Months Ended December 31,		Twelve Months Ended December 31,	
	2013	2012	2013	2012
Revenues:				
Collaboration revenue	$3,931	$5,918	$31,326	$28,779
Grant revenue	480	722	3,370	5,167
Total revenues	4,411	6,640	34,696	33,946
Operating expenses:				
Research and development (1)	15,020	9,450	54,875	46,139
General and administrative (1)	7,483	3,224	25,219	14,615
Total operating expenses	22,503	12,674	80,094	60,754
Loss from operations	(18,092)	(6,034)	(45,398)	(26,808)
Interest (expense) income, net	166	(203)	(6,084)	(1,210)
Loss on extinguishment of debt	—	—	(130)	—
Other income (expense), net	41	(35)	38	1,783
Net loss	(17,885)	(6,272)	(51,574)	(26,235)
Deemed dividend	—	—	(18,249)	—
Gain on exchange of convertible preferred stock in connection with recapitalization	—	—	3,391	159,954
Less beneficial conversion charge	—	—	—	(378)
Net (loss) income attributable to common stockholders	($17,885)	($6,272)	($66,432)	$133,341
Weighted-average shares outstanding (in shares):				
Basic	23,803,282	4,545	12,829,411	3,328
Diluted	23,803,282	4,545	12,829,411	17,205
Net (loss) income per share applicable to common stockholders - basic (in dollars per share)	($0.75)	($1,380.13)	($5.18)	$219.76
Net (loss) income per share applicable to common stockholders - diluted (in dollars per share)	($0.75)	($1,380.13)	($5.18)	$42.50
(1) Non-cash share-based compensation expense included in operating expenses are as follows:				
Research and development	$662	$150	$2,040	$804
General and administrative	2,623	322	6,387	1,484
Total share-based compensation expense	$3,285	$472	$8,427	$2,288

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

	December 31, 2013	December 31, 2012
Cash, cash equivalents and marketable securities	$142,468	$2,726
Total assets	$151,903	$13,072
Total debt	49	4,883
Total deferred revenue	877	17,432
Total liabilities	$15,360	$31,889
Total stockholders' equity (deficit) (23,803,282 and 4,526 common shares issued and outstanding at December 31, 2013 and 2012, respectively)	136,542	(99,641)
Total liabilities, convertible preferred stocks and stockholders' (deficit) equity	$151,903	$13,072