November 14, 2013

PTC THERAPEUTICS REPORTS THIRD QUARTER 2013 FINANCIAL AND CORPORATE RESULTS

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SOUTH PLAINFIELD, NJ - November 14, 2013 - PTC Therapeutics, Inc. (NASDAQ: PTCT), a biopharmaceutical company focused on the discovery and development of orally administered, proprietary small molecule drugs that target post-transcriptional control processes, today announced financial and corporate results for the quarter ended September 30, 2013.

"2013 has thus far been an exciting year for us. PTC was founded based upon a strong scientific platform focused on RNA biology with the goal of bringing new therapies to patients with rare disorders and we have made tremendous progress this year towards realizing that goal." said Stuart Peltz, PhD, Chief Executive Officer, PTC Therapeutics, Inc. "We initiated our nonsense mutation Duchenne muscular dystrophy confirmatory Phase 3 clinical trial, which was informed by our previous clinical results that defined the natural history of ambulation in DMD patients as measured by the six-minute walk test. These natural history results have been corroborated by subsequent studies, giving us a high degree of confidence that our ongoing confirmatory study is both well designed and well powered to demonstrate the efficacy of ataluren. In addition, a development candidate was selected in our spinal muscular atrophy (SMA) program in collaboration with Roche and the SMA Foundation. This development candidate was originally developed using PTC's Alternative Splicing platform, which we are also deploying against other medically important targets."

"As we look ahead to 2014, we are well positioned to execute on our strategy. This includes completing enrollment in our nonsense mutation Duchenne muscular dystrophy confirmatory Phase 3 clinical trial and the initiation of our Phase 3 confirmatory trial in nonsense mutation cystic fibrosis. We also look forward to advancement of our other programs highlighted in our R&D day."

Corporate Highlights:

- **Publications in *Muscle and Nerve* validating six-minute walk test and describing ambulatory natural history in DMD patients:** In August 2013, data published in two manuscripts 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 trial of ataluren in 174 patients, the first registration-directed, placebo-controlled, multi-national study of a new chemical entity for DMD.
- **Presentations at multiple scientific conferences:** During the quarter, PTC presented at multiple scientific conferences including the European Pediatric Neurology Society Congress (EPNS), where PTC hosted a symposium titled "New perspectives in the management of Duchenne muscular dystrophy", the World Muscle Society Congress (WMS) and the North American Cystic Fibrosis Conference (NACFC).
- **Chosen to join the Russell 2000 Index:** On September 30th, 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.
- **Hosted first R&D Day:** On October 25th, 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.
- **Confirmatory Phase 3 Trial in nmDMD on track:** In April 2013, PTC initiated a confirmatory Phase 3 clinical trial evaluating our investigational new drug ataluren as a potential treatment for patients with nonsense mutation Duchenne muscular dystrophy (nmDMD). The study is on-track to complete enrollment by mid-2014. The Phase 3 study is expected to be conducted at 53 clinical sites across 18 countries.
- **EMA regulatory process progressing:** Following the October 2012 submission of a Marketing Authorization Application (MAA) to the European Medicines Agency (EMA) for conditional approval of ataluren in nmDMD, the regulatory process continues to progress. The Committee for Medicinal Products for Human Use (CHMP) has recently

notified us that it intends to convene a Scientific Advisory Group (SAG) meeting as part of the regulatory review process. The SAG meeting may shift the timing of a final opinion from the CHMP on the conditional approval of ataluren in nmDMD from the last quarter of 2013 to the first quarter of 2014. To manage our regulatory priorities, PTC also plans to delay the filing of an MAA for conditional approval of ataluren in nonsense mutation cystic fibrosis (nmCF) until the first quarter of 2014.

Third Quarter 2013 Financial Highlights:

- Cash, cash equivalents, and marketable securities totaled $157.2 million at September 30, 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 a $10 million milestone achieved in the third quarter of 2013 from Roche for the selection of a development candidate in our SMA collaboration.
- Revenue from grants and collaborations was $16.3 million for the third quarter of 2013, compared to $7.2 million for the same period in 2012. The increase was primarily the result of the achievement of a $10 million milestone in our Roche collaboration. 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 $20.6 million for the third quarter of 2013, including $2.6 million in stock based compensation expense, compared to $14.3 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 Phase 3 clinical trial of ataluren for the treatment of nmDMD, as well as higher general and administrative expenses related to public company expenses and pre-commercial activities.
- Net loss for the third quarter of 2013 was $4.4 million compared to a net loss of $7.3 million for the same period in 2012.
- Shares issued and outstanding as of September 30, 2013 were 24.9 million, which includes 1.1 million 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, Thursday, November 14, 2013, at 8:00 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 80749157.

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 Therapeutics is 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 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:
Any statements in this press release about future expectations, plans and prospects for PTC, the development of and potential market for PTC's product candidates, 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 EMA, our earlier stage programs, and other statements containing the words "anticipate," "believe," "estimate," "expect," "intend," "may," "plan" "predict," "project," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions, constitute forward-looking statements within the meaning of The Private Securities Litigation Reform Act of 1995. Forward-looking statements involve substantial risks and uncertainties that could cause our future results, performance or achievements to differ significantly from those expressed or implied by these forward-looking statements. Such risks and uncertainties include, among others, 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 other factors discussed in the "Risk Factors" in the most recent Quarterly Report, which is on file 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. We anticipate that subsequent events and developments will cause our views to change. However, while we may elect to update these forward-looking statements at some point in the future, the Company specifically disclaim any obligation to do so. These forward-looking statements should not be relied upon as representing PTC's views as of any date subsequent to the date of this press release.

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

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2012	2013	2012	2013
Revenues:				
Collaboration revenue	$6,043	$15,455	$22,861	$27,395
Grant revenue	1,152	834	4,445	2,890
Total revenues and non-cash cancellation revenue	7,195	16,289	27,306	30,285
Operating expenses:				
Research and development (1)	10,466	13,886	36,689	39,855
General and administrative (1)	3,784	6,679	11,391	17,735
Total operating expenses	14,250	20,565	48,080	57,590
Loss from operations	(7,055)	(4,276)	(20,774)	(27,305)
Interest (expense) income, net	(247)	27	(1,007)	(6,250)
Loss on extinguishment of debt	—	(130)	—	(130)
Other income (expense), net	5	(37)	1,818	(3)
Net loss	(7,297)	(4,416)	(19,963)	(33,688)
Deemed dividend	—	—	—	(18,249)
Gain on exchange of convertible preferred stock in connection with recapitalization	—	—	159,954	3,391
Less beneficial conversion charge	—	—	(378)	—
Net (loss) income attributable to common stockholders	($7,297)	($4,416)	$139,613	($48,546)
Weighted-average shares outstanding (in shares):				
Basic	4,545	23,803,282	2,937	8,995,167
Diluted	4,545	23,803,282	13,593	8,995,167
Net (loss) income per share applicable to common stockholders - basic (in dollars per share)	($1,605.53)	($0.19)	$182.41	($5.40)
Net (loss) income per share applicable to common stockholders - diluted (in dollars per share)	($1,605.53)	($0.19)	$39.41	($5.40)
(1) Non-cash share-based compensation expense included in operating expenses are as follows:				
Research and development	$193	$677	$654	$1,378
General and administrative	327	1,963	1,162	3,764
Total share-based compensation expense	$520	$2,640	$1,816	$5,142

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

	December 31, 2012	September 30, 2013
Cash, cash equivalents and marketable securities	$2,726	$157,227
Total assets	$13,072	$167,244
Total debt	4,883	84
Total deferred revenue	17,432	3,631
Total liabilities	$31,889	$16,212
Total stockholders' (deficit) equity (4,526 and 23,803,282 common shares issued and outstanding at December 31, 2012 and September 30, 2013, respectively)	(99,641)	151,032
Total liabilities, convertible preferred stocks and stockholders' (deficit) equity	$13,072	$167,244