



August 12, 2013

## PTC Therapeutics Reports Second Quarter 2013 Financial And Corporate Results

-- Conference call to be held today at 4:30 p.m. ET --

**SOUTH PLAINFIELD, NJ - August 12, 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 June 30, 2013.

"The first half of the year was a very exciting time for PTC. We successfully completed an IPO raising gross proceeds of more than \$144 million. With over \$165 million in cash on our balance sheet at the end of the quarter, we are now well capitalized to continue the late-stage clinical development of our lead product candidate, ataluren, in nonsense mutation Duchenne muscular dystrophy and cystic fibrosis." said Stuart Peltz, PhD, Chief Executive Officer, PTC Therapeutics, Inc. "We began enrolling patients in our Phase 3 nmDMD clinical trial earlier this year and we plan to begin enrollment in our Phase 3 nmCF clinical trial in the first half of 2014. We are also excited about the progress that is being made in our SMA program that is partnered with Roche where we recently announced the selection of a development candidate."

### Key Recent Accomplishments

- | **Successful Completion of Initial Public Offering (IPO):** In June 2013, PTC completed its 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.
- | **Initiated Confirmatory Phase 3 Study of Ataluren in nmDMD:** In April 2013, PTC initiated a confirmatory Phase 3 clinical trial evaluating ataluren as a potential treatment for patients with Duchenne muscular dystrophy due to a nonsense mutation (nmDMD). The study is on-track to complete enrollment by mid-2014 with country approvals for the trial and clinical trial site initiation proceeding according to plan. The Phase 3 study is expected to be conducted at 53 clinical sites across 18 countries.
- | **Submitted Responses to Day 120 Questions for MAA Submission:** 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 EMA provided a list of questions to PTC regarding its application, known as the Day 120 Questions. In July 2013, PTC submitted responses to the Day 120 Questions to the EMA. The company anticipates a response from the Committee for Medicinal Products for Human Use, or CHMP, on the MAA submission for conditional approval of ataluren for the treatment of nmDMD by the end of 2013.
- | **Selected Lead Candidate for SMA Program:** On August 8, 2013, in collaboration with its partners, the SMA Foundation and Roche, PTC declared a lead development candidate in the spinal muscular atrophy (SMA) program. Selection of the development candidate triggered a \$10 million milestone payment by Roche to PTC, which will be recognized as revenue in the third quarter of 2013. This milestone is important for moving this program forward and evidences the value of PTC's splicing platform technology.

### Second Quarter 2013 Financial Highlights

- | Cash, cash equivalents, and marketable securities totaled \$165.7 million at June 30, 2013 compared to \$2.7 million at December 31, 2012.
- | Total operating expenses increased to \$21.3 million for the second quarter of 2013, including \$1.9 million in stock based compensation expense, from \$15.1 million for the second quarter of 2012, including \$0.6 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 attributable to common shareholders for the second quarter of 2013 was \$14.6 million compared to a net income attributable to common shareholders for the second quarter of 2012 of \$153.5 million. In 2012, the company recorded a gain of \$160.0 million on exchange of convertible preferred stock in connection with a recapitalization.

- l In July 2013, the company paid in full the outstanding principal and interest balance of \$2.6 million under promissory notes issued related to a secured debt facility with a syndicate of two lenders.

### **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, Monday, August 12, 2013, 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 24942756.

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](http://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 biopharmaceutical company focused on the discovery and development of orally-administered, proprietary small molecule drugs that target post-transcriptional control processes. While PTC's discovery programs are directed at targets in multiple therapeutic areas, PTC is focusing particularly on the development and commercialization of treatments for orphan and ultra-orphan disorders. 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 neuromuscular disorders, oncology and infectious diseases. For more information on the company, please visit our website [www.ptcbio.com](http://www.ptcbio.com).

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### **FORWARD LOOKING STATEMENTS:**

Any statements in this press release about future expectations, plans and prospects for the company, including statements about the development of ataluren, such as the timing and conduct of the company's Phase 3 clinical trial, the timing and likelihood of conditional approval of ataluren, pre-commercial activities, the advancement of the company's earlier stage pipeline 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. Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, 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, the sufficiency of our cash resources and need for additional financing and other factors discussed in the "Risk Factors" section of the final prospectus for our initial public offering, which is on file with the Securities and Exchange Commission. In addition, the forward-looking statements included in this press release represent the company's views as of the date of this release. The company anticipates that subsequent events and developments will cause the company's views to change. However, while the company may elect to update these forward-looking statements at some point in the future, the company specifically disclaims any obligation to do so. These forward-looking statements should not be relied upon as representing the company's views as of any date subsequent to the date of this release.

PTC THERAPEUTICS, INC.

Statements of Operations

	Three Months Ended June 30,		Six Months Ended June 30,	
	2012	2013	2012	2013
Revenues:				
Collaboration revenue	\$ 6,064,413	5,867,806	16,817,797	11,939,954
Grant revenue	1,520,764	986,406	3,293,100	2,055,922
Total revenues and non-cash cancellation revenue	7,585,177	6,854,212	20,110,897	13,995,876
Operating expenses:				
Research and development	11,918,999	14,712,091	26,222,332	25,968,919
General and administrative	3,164,385	6,595,057	7,607,009	11,056,302
Total operating expenses	15,083,384	21,307,148	33,829,341	37,025,221
Loss from operations	(7,498,207)	(14,452,936)	(13,718,444)	(23,029,345)
Interest expense, net	(335,081)	(114,173)	(760,261)	(6,276,229)
Other income (expense), net	1,765,216	(19,469)	1,812,772	34,461
Net loss	(6,068,072)	(14,586,578)	(12,665,933)	(29,271,113)
Deemed dividend	—	—	—	(18,248,768)
Gain on exchange of convertible preferred stock in connection with recapitalization	159,954,069	—	159,954,069	3,390,750
Less beneficial conversion charge	(377,787)	—	(377,787)	—
Net income (loss) attributable to common stockholders	\$ 153,508,210	(14,586,578)	146,910,349	(44,129,131)

PTC THERAPEUTICS, INC.

Summary Balance Sheet

	December 31, 2012	June 30, 2013
Cash, cash equivalents and marketable securities	\$2,725,702	\$165,678,055
Total Assets	13,072,352	175,468,084
Total Liabilities	31,889,085	22,548,444
Total Liabilities, convertible preferred stock and stockholders' (deficit) equity	13,072,352	175,468,084