PTC Therapeutics Reports First Quarter 2020 Financial Results and Provides a Corporate Update

April 30, 2020

- Risdiplam demonstrated clinically meaningful and statistically significant results across broadest population of spinal muscular atrophy (SMA) patients studied to date; PDUFA date August 24, 2020 -

- PTC provides an update on clinical and regulatory programs -

- First quarter 2020 total revenues were $68.3 million, 27% year over year growth -

SOUTH PLAINFIELD, N.J., April 30, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2020.

"PTC continued to execute on our strategic priorities including the development and commercial fronts in the first quarter highlighted by DMD franchise revenue growth and strong risdiplam results," said Stuart Peltz, Ph.D., CEO of PTC Therapeutics. "The breadth of positive clinical risdiplam data reinforces the global commercial competitive profile of this therapy, which has the potential to be the first and only at-home SMA treatment, a critical advantage in the COVID-19 environment. We look forward to a number of potential value-creating catalysts including the risdiplam PDUFA date, and multiple clinical milestones in the upcoming months."

Key First Quarter and Other Corporate Updates:

- Total net product revenues were $68.2 million across our commercial portfolio in the first quarter of 2020 representing 28% year over year growth. Translarna ™(ataluren) net product revenues were $40.5 million for the first quarter of 2020 and Emflaza® (deflazacort) net product revenues were $27.5 million for the first quarter of 2020.
- PTC announced the appointments of Matthew Klein, M.D., to Chief Development Officer and Eric Pauwels to Chief Business Officer.
- Due to the uncertainty of the duration and severity of the COVID-19 impact, PTC is withdrawing financial guidance for the 2020 fiscal year.

Compelling Data Across Patients with Type 1, 2 & 3 SMA

- The FIREFISH Part 2 data in type 1 Spinal muscular atrophy (SMA) patients demonstrated achievement of motor milestones and motor function improvements not observed in natural history. The study met its primary endpoint ($p<0.0001$), with 12 of 41 patients sitting, and all of its key secondary endpoints. Safety data was consistent with the known safety profile. There were no new safety signals and risdiplam was generally well tolerated.
- The clinical data from Part 2 of the pivotal SUNFISH study evaluating risdiplam in people aged 2-25 years with non-ambulatory type 2 and type 3 SMA demonstrated statistically significant results in primary and key secondary endpoints. Safety for risdiplam was consistent with its known safety profile and no new safety signals were identified.
- Risdiplam clinical studies are ongoing, including RAINBOWFISH and JEWELFISH. When necessary, patients are receiving drug through contactless home delivery to ensure study compliance.
- Earlier this year, Roche opened early access programs in the United States (U.S.) and European countries for type 1 SMA patients. Recently, Roche announced that the program will be expanded to include patients with type 2 SMA in countries where applicable, at the moment of filing of the regulatory application for risdiplam. In addition, in response to requests received as well as to the unique pressures of the COVID-19 pandemic, Roche has decided to amend the programs for type 1 and type 2 patients whose current treatment have been interrupted as a direct consequence of the COVID-19 pandemic. The Prescription Drug User Fee Act (PDUFA) date for risdiplam is now August 24, 2020, following the submission of additional data including SUNFISH Part 2.

Advancing Gene Therapy Platform

- PTC now expects to file the biologics license application (BLA) submission with the U.S. Food and Drug Administration (FDA) in the second half of 2020 for its gene therapy program for Aromatic L-amino acid decarboxylase (AADC) deficiency. The study of the use of the commercial cannula in young patients has been delayed due to COVID-19.
- The Marketing Authorization Application (MAA) process in the European Union (EU) for the AADC deficiency program is ongoing and PTC expects to receive the Committee for Medicinal Products for Human Use (CHMP) final opinion by the end of 2020.
- For our Friedreich ataxia (FA) and Angelman syndrome gene therapy programs, COVID-19 has impacted multiple investigational new drug application (IND) enabling activities. Therefore, we now anticipate the IND filings will be delayed by at least one quarter and we will provide an update on timing as we better understand the impact of COVID-19.
- Due to COVID-19, PTC has deferred certain capital expenditures related to our leased biologics facility in Hopewell

Township, New Jersey and now anticipates that GMP manufacturing of clinical material at this facility will begin in early 2021.

Updates in PTC's Diverse Product Pipeline

- PTC now anticipates results from the U.S. Translarna dystrophin study in the third quarter of 2020. Due to COVID-19, the study site is currently closed to elective procedures, which includes those required to complete the dystrophin study.
- The Huntington disease program remains on track to be in healthy volunteers prior to the end of 2020. A development candidate has been chosen and IND toxicology studies are ongoing.
- PTC now expects to initiate potential registrational trials in its Bio-e platform with PTC743 in refractory mitochondrial epilepsy in the third quarter of 2020 and in Friedreich ataxia in the fourth quarter of 2020. The initiation of these studies has been delayed due to COVID-19.
- The Phase 1 trial of PTC857, which is being developed for GBA Parkinson's disease, remains on track to start in the third quarter, with no current delays due to COVID-19.
- In the interest of the health and safety of employees and guests, PTC has postponed the Analyst Day previously scheduled for mid-June. We plan to host virtual deep dives on our platforms throughout the year.

Financial Highlights:

- Total revenues were $68.3 million for the first quarter of 2020, compared to total revenues of $53.6 million for the first quarter of 2019.
- Translarna net product revenues were $40.5 million for the first quarter of 2020, compared to $35.3 million for the first quarter of 2019. These results reflect an increase in net product sales in existing markets as well as continued geographic expansion into new territories.
- Emflaza net product revenues were $27.5 million for the first quarter of 2020, compared to $17.8 million for the first quarter of 2019. These results reflect new patient growth driven in part by diagnostic and educational efforts as well as ongoing operational improvements.
- Generally accepted accounting principles in the United States (GAAP) research and development (R&D) expenses were $90.1 million for the first quarter of 2020, compared to $52.6 million for the first quarter of 2019. The increase in R&D expenses reflects costs associated with advancing the gene therapy and Bio-e platforms and increased investment in research programs as well as advancements of the clinical pipeline.
- Non-GAAP R&D expenses were $81.9 million for the first quarter of 2020, excluding $8.2 million in non-cash stock-based compensation expense, compared to $47.9 million for the first quarter of 2019, excluding $4.7 million in non-cash stock-based compensation expense.
- GAAP selling, general and administrative (SG&A) expenses were $58.2 million for the first quarter of 2020, compared to $40.6 million for the first quarter of 2019, reflecting continued investment to support our commercial activities including our expanding commercial portfolio.
- Non-GAAP SG&A expenses were $51.2 million for the first quarter of 2020, excluding $7.0 million in non-cash stock-based compensation expense, compared to $36.0 million for the first quarter of 2019, excluding $4.6 million in non-cash stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $0.9 million for the first quarter of 2020, compared to $21.2 million for the first quarter of 2019. The change in fair value of deferred and contingent consideration is related to the fair valuation of potential future consideration to be paid to former equity holders of Agilis Biotherapeutics, Inc. (Agilis) in connection with PTC's acquisition of Agilis, which closed in August 2018.
- In discussions with certain former shareholders of Agilis, PTC has agreed to exchange their pro rata share of specific future cash milestone payments in the aggregate amount of $225 million for a mixture of cash and equity. Under this agreement, which the former shareholders and PTC entered into on April 29th, PTC has pre-paid 94% of time-based cash milestones due in August 2020, and has agreed to issue 2,821,176 shares of common stock in exchange for 94% of future cash milestones for the AADCd BLA approval by the FDA and the receipt of a Priority Review Voucher in connection with that approval.
- Net loss was $112.7 million for the first quarter of 2020, compared to net loss of $72.1 million for the first quarter of 2019.
- Cash, cash equivalents and marketable securities totaled $595.9 million at March 31, 2020, compared to $686.6 million at December 31, 2019.
- Shares issued and outstanding as of March 31, 2020 were 62,758,520.

Due to the uncertainty with respect to the duration, nature and extent of impacts of the COVID-19 pandemic and responsive measures relating thereto, we cannot be certain that the pandemic will not cause us to experience further delays to the timelines set forth above or other negative additional impacts.

Non-GAAP Financial Measures:
In this press release, the financial results of PTC are provided in accordance with GAAP and using certain non-GAAP financial measures. In particular, the non-GAAP financial measures exclude non-cash, stock-based compensation expense. These non-GAAP financial measures are provided as a complement to financial measures reported in GAAP because management uses these non-GAAP financial measures when assessing and identifying

operational trends. In management's opinion, these non-GAAP financial measures are useful to investors and other users of PTC's financial statements by providing greater transparency into the historical and projected operating performance of PTC and the company's future outlook. Non-GAAP financial measures are not an alternative for financial measures prepared in accordance with GAAP. Quantitative reconciliations of the non-GAAP financial measures to their respective closest equivalent GAAP financial measures are included in the table below.

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

	Three Months Ended March 31,	
	2020	**2019**
Revenues:		
Net product revenue	$ 68,196	$ 53,054
Collaboration and grant revenue	63	529
Total revenues	68,259	53,583
Operating expenses:		
Cost of product sales	4,085	2,376
Amortization of acquired intangible asset	7,949	6,077
Research and development (1)	90,107	52,566
Selling, general and administrative (2)	58,209	40,544
Change in the fair value of deferred and contingent consideration	900	21,160
Total operating expenses	161,250	122,723
Loss from operations	(92,991)	(69,140)
Interest expense, net	(5,642)	(2,288)
Other expense, net	(13,832)	(109)
Loss before income tax expense	(112,465)	(71,537)
Income tax expense	(222)	(576)
Net loss attributable to common stockholders	$ (112,687)	$ (72,113)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	62,389,158	55,855,111
Net loss per share—basic and diluted (in dollars per share)	$ (1.81)	$ (1.29)
(1) Research and development reconciliation		
GAAP research and development	$ 90,107	$ 52,566
Less: share-based compensation expense	8,179	4,686
Non-GAAP research and development	$ 81,928	$ 47,880
(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 58,209	$ 40,544
Less: share-based compensation expense	7,041	4,577
Non-GAAP selling, general and administrative	$ 51,168	$ 35,967

PTC Therapeutics, Inc.
Summary Consolidated Balance Sheets
(in thousands, except share data)

	March 31, 2020	**December 31, 2019**
Cash, cash equivalents and marketable securities	$ 595,859	$ 686,563
Total Assets	**$ 1,545,592**	**$ 1,623,782**
Total debt	$ 314,574	$ 313,859
Total deferred revenue	10,406	11,657
Total liabilities	**$ 1,016,858**	**$ 1,029,452**
Total stockholders' equity (62,758,520 and 61,935,870 common shares issued and outstanding at March 31, 2020 and December 31, 2019 respectively)	$ 528,734	$ 594,330
Total liabilities and stockholders' equity	**$ 1,545,592**	**$ 1,623,782**

Today's Conference Call and Webcast Reminder:
Today's conference call will take place at 4:30 pm (ET) and can be access by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 8267466. A live, listen-only webcast of the conference call can be accessed on the investor relations section of the PTC website at www.ptcbio.com. The accompanying slide presentation will be posted on the investor relations section of the PTC website. A webcast replay of the call will be available approximately two hours after completion of the call and will be archived on the company's website for 30 days following the call.

About PTC Therapeutics, Inc.
PTC is a science-driven, global biopharmaceutical company focused on the discovery, development and commercialization of clinically differentiated medicines that provide benefits to patients with rare disorders. PTC's ability to globally commercialize products is the foundation that drives investment in a robust and diversified pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

For More Information:

Investors:
Alex Kane
+1 (908) 912-9643
akane@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167
jbaj@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 contained in this release, other than statements of historic fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; expectations with respect to the impacts of the COVID-19 pandemic and related response measures; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, commercialization or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its products and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; and the objectives of management. Other forward-looking statements may be identified by the words "guidance", "plan," "anticipate," "believe," "estimate," "expect," "intend," "may," "target," "potential," "will," "would," "could," "should," "continue," and similar expressions.

PTC's actual results, performance or achievements could differ materially from those expressed or implied by forward-looking statements it makes as a result of a variety of risks and uncertainties, including those related to: the outcome of pricing, coverage and reimbursement negotiations with third party payors for PTC's products or product candidates that PTC commercializes or may commercialize in the future; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions and potential approvals, manufacturing capabilities and the potential financial impact and benefits of its leased biologics manufacturing facility and the potential achievement of development, regulatory and sales milestones and contingent payments that PTC may be obligated to make; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, potential regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; the enrollment, conduct, and results of studies under the SMA collaboration and events during, or as a result of, the studies that could delay or prevent further development under the program, including any potential regulatory submissions and potential commercialization with regards to risdiplam; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in the European Economic Area (EEA), including whether the European Medicines Agency (EMA) determines in future annual renewal cycles that the benefit-risk balance of Translarna authorization supports renewal of such authorization; PTC's ability to enroll, fund, complete and timely submit to the EMA the results of Study 041, a randomized, 18-month, placebo-controlled clinical trial of Translarna for the treatment of nmDMD followed by an 18-month open-label extension, which is a specific obligation to continued marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; significant business effects, including the effects of industry, market, economic, political or regulatory conditions; changes in tax and other laws, regulations, rates and policies; the eligible patient base and commercial potential of PTC's products and product candidates; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of the lease agreement for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the senior secured term loan facility with MidCap Financial; the sufficiency of PTC's cash resources and its ability to obtain adequate financing in the future for its foreseeable and unforeseeable operating expenses and capital expenditures; and the factors discussed in the "Risk Factors" section of PTC's most recent Quarterly Report on Form 10-Q and Annual Report on Form 10-K, as well as any updates to these risk factors filed from time to time in PTC's other filings with the SEC. You are urged to carefully consider all such factors.

As with any pharmaceutical under development, there are significant risks in the development, regulatory approval and commercialization of new products. There are no guarantees that any product will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra or risdiplam.

The forward-looking statements contained herein represent PTC's views only as of the date of this press release and PTC does 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 press release except as required by law.

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