PTC Therapeutics Reports First Quarter 2019 Financial Results and Provides a Corporate Update

May 2, 2019

Translarna™ Approved by Brazilian Health Regulatory Authority (ANVISA)
Waylivra™ Receives Positive CHMP Opinion

SOUTH PLAINFIELD, N.J., May 2, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the first quarter ending March 31, 2019.

"We believe we are making great strides towards our vision of having many more treatments for patients of rare disorders," said Stuart Peltz, Ph.D., CEO of PTC Therapeutics. "The approval of Translarna expands our footprint in Brazil. We are now preparing for the launches for multiple products, including Tegsedi for hATTR and Waylivra for FCS in Latin America. These differentiated therapies for rare disorders have the potential to be part of growing our revenue base to $1.5 billion by 2023."

Key First Quarter and Other Corporate Highlights:

Expanding commercial platform

- Translarna received approval from the Brazilian health regulatory authority (ANVISA) which will allow for expanded market access.
- Waylivra™ received a positive CHMP opinion from the European medical authorities in the first quarter 2019 and will be referred to the European Commission for consideration. After ratification by the European Commission, PTC plans to initiate early access programs in Latin America in 2019.
- Tegsedi™ application submitted to the Brazilian health regulatory authority (ANVISA) was granted priority review, with expected approval by year end 2019. PTC has initiated early access programs for Tegsedi to support patient diagnosis and monitoring in Latin America.
- PTC has submitted a sNDA with the FDA for Emflaza® for patients 2 to 5 years old and received an approval action date of July 4, 2019.

Advancing gene therapy portfolio

- As previously disclosed, PTC plans to submit a BLA with the FDA in late 2019 followed by an MAA in Europe for the AADC deficiency gene therapy program with anticipated commercial launch in the United States in 2020.
- Friedreich ataxia program is advancing with an expected IND submission in late 2019 and subsequent entry in the clinic.
- PTC is in the process of finalizing a long-term lease on an existing biologics manufacturing facility for the gene therapy pipeline.

Risdiplam SMA data expected at American Academy of Neurology (AAN)

- As previously disclosed, based on regulatory interactions an NDA and MAA is planned for the second half of this year with the intention to support a broad label to treat SMA Types 1, 2, & 3 patients.
- The SMA program is a collaboration between PTC, Roche and SMA Foundation.
- Net sales over $1B would be subject to mid-teens royalties to PTC from Roche, resulting in potential royalties to PTC in excess of $200M per year. Potential remaining regulatory and sales-based milestones are approximately $400M.

Growing pipeline and R&D capabilities

- As previously disclosed, PTC's splicing platform has generated another development candidate, PTC258, for Familial dysautonomia, a rare genetic neurological disorder causing life-threatening medical complications from birth. PTC258 is advancing through IND-enabling studies to enter the clinic in late 2019. This program is in collaboration with MGH and NYU.
- Translarna's dystrophin study in currently enrolling for potential U.S. regulatory re-submission for accelerated approval in 2020.
- PTC's oncology portfolio continues to advance with studies in AML with PTC299 and DIPG & LMS for PTC596. PTC expects these studies to fully enroll by the end of 2019.

PTC Re-iterates Full Year 2019 Guidance:

- PTC anticipates full year DMD franchise net product revenues to be between $285 and $305 million.

- PTC anticipates GAAP R&D and SG&A expense for the full year 2019 to be between $395 and $405 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2019 to be between $360 and $370 million, excluding estimated non-cash, stock-based compensation expense of approximately $35 million.

First Quarter 2019 Financial Highlights:

- Total revenues were $53.6 million for the first quarter of 2019, compared to $56.1 million for the first quarter of 2018.
- Translarna net product revenues were $35.3 million for the first quarter of 2019, compared to $36.8 million for the first quarter of 2018. These results reflect lumpiness in ordering patterns from Latin America.
- Emflaza net product revenues were $17.8 million for the first quarter of 2019, compared to $19.2 million for the first quarter of 2018. These results reflect first quarter dynamics including seasonality and a planned transition to a new specialty pharmacy distributor.
- GAAP R&D expenses were $52.6 million for the first quarter of 2019, compared to $31.4 million for the first quarter of 2018. The increase in R&D expenses reflects costs associated with advancing the gene therapy platform and increased investment in research programs as well as advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $47.9 million for the first quarter of 2019, excluding $4.7 million in non-cash, stock-based compensation expense, compared to $27.6 million for the first quarter of 2018, excluding $3.7 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $40.6 million for the first quarter of 2019, compared to $33.0 million for the first quarter of 2018. The increase in SG&A expenses were primarily due to continued investment to support our commercial activities.
- Non-GAAP SG&A expenses were $36.0 million for the first quarter of 2019, excluding $4.6 million in non-cash, stock-based compensation expense, compared to $29.0 million for the first quarter of 2018, excluding $4.0 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $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.
- Net loss was $72.1 million for the first quarter of 2019, compared to net loss of $19.3 million for the first quarter of 2018.
- Cash, cash equivalents, and marketable securities were $407.2 million at March 31, 2019, compared to $227.6 million at December 31, 2018.
- Shares issued and outstanding as of March 30, 2019 were 58,418,790.

Non-GAAP Financial Measures:
In this press release, the financial results and financial guidance of PTC are provided in accordance with accounting principles generally accepted in the United States (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 measure are included in the table below.

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 1559069. 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 two weeks.

About PTC Therapeutics, Inc.
PTC is a science-led, 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 pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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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 contained in this release, other than statements of historic fact, are forward-looking statements, including the information provided under the heading "PTC Re-iterates Full Year 2019 Guidance", including with respect to (i) 2019 net product revenue guidance and (ii) 2019 GAAP and non-GAAP R&D and SG&A expense guidance, and statements regarding: the future expectations, plans and prospects for PTC; expectations with respect to PTC's gene therapy platform, including any potential regulatory submissions; PTC's expectations with respect to the licensing, regulatory submissions and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna and Emflaza and related regulatory submissions; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions or royalty or milestone payments; 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 Emflaza and Translarna and any other product candidates that PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; 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 potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; expectations with respect to the potential financial impact and benefits of the collaboration and licensing agreement with Akcea Therapeutics, Inc., including with respect to the timing of regulatory approval of Tegsedi and Waylivra in countries in Latin America and the Caribbean, the commercialization of Tegsedi and Waylivra, and PTC's expectations with respect to contingent payments to Akcea based on net sales and the potential achievement of regulatory milestones; 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 with regards to risdiplam; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition of its gene therapy pipeline, as well as other 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 Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra, risdiplam or any of PTC's other product candidates; PTC's scientific approach and general development progress; 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.

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

	Three Months Ended March 31	
	2019	**2018**
Revenues:		
Net product revenue	$ 53,054	$ 55,981
Collaboration and grant revenue	529	81
Total revenues	53,583	56,062
Operating expenses:		
Cost of product revenues	2,376	3,045
Amortization of acquired intangible asset	6,077	5,428
Research and development (1)	52,566	31,363
Selling, general and administrative (2)	40,544	32,969
Change in the fair value of deferred and contingent consideration	21,160	—
Total operating expenses	122,723	72,805
Loss from operations	(69,140)	(16,743)
Interest expense, net	(2,288)	(3,303)

Other (expense) income, net	(109)	1,004
Loss before income tax expense	(71,537)	(19,042)
Income tax expense	(576)	(221)
Net loss attributable to common stockholders	$ (72,113)	$ (19,263)
Weighted-average shares outstanding:		
Basic (in shares)	55,855,111	41,626,617
Diluted (in shares)	55,855,111	41,626,617
Net loss per share—basic and diluted (in dollars per share)	$ (1.29)	$ (0.46)
(1) Research and development reconciliation		
GAAP research and development	$ 52,566	$ 31,363
Less: non-cash, stock-based compensation expense	4,686	3,747
Non-GAAP research and development	$ 47,880	$ 27,616
(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 40,544	$ 32,969
Less: non-cash, stock-based compensation expense	4,577	4,001
Non-GAAP selling, general and administrative	$ 35,967	$ 28,968

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

	March 31, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 407,162	$ 227,586
Total assets	**$ 1,283,911**	**$ 1,119,222**
Total debt	$ 155,135	$ 153,014
Total deferred revenue	14,350	13,438
Total liabilities	**$ 770,969**	**$ 768,495**
Total stockholders' equity (58,418,790 and 50,606,147 common shares issued and outstanding at March 31, 2019 and December 31, 2018, respectively)	512,942	350,727
Total liabilities and stockholders' equity	**$ 1,283,911**	**$ 1,119,222**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2019 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A expense	$ 395,000	$ 405,000
Less: projected non-cash, stock-based compensation expense	35,000	35,000
Projected non-GAAP R&D and SG&A expense	**$ 360,000**	**$ 370,000**

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