PTC Therapeutics Reports Fourth Quarter and Full Year 2019 Financial Results and Provides a Corporate Update

March 2, 2020

SOUTH PLAINFIELD, N.J., March 2, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the fourth quarter and full year ending December 31, 2019.

"2019 was a year of outstanding execution for PTC. I'm proud of the progress made in every area of the business," said Stuart Peltz, Ph.D., CEO of PTC Therapeutics. "We've significantly expanded our rare disease portfolio, strengthened our global commercial engine and added vital gene therapy manufacturing capabilities, all while delivering on our revenue guidance. The innovation in our diverse, multiplatform pipeline continues to create value for stakeholders."

Key Fourth Quarter and Other Corporate Highlights:

Expanding commercial footprint including continued revenue growth

- Total revenue for the full year 2019 was $307 million. The Duchenne muscular dystrophy (DMD) franchise, consisting of Translarna™ (ataluren) and Emflaza® (deflazacort), continues to grow with 2019 revenue of $291 million.
- PTC continues to leverage its strong Latin American infrastructure to support the launch of multiple products. In 2019, Translarna received approval from the Brazilian health regulatory authority (ANVISA) for ambulatory nmDMD patients 5 years and older. In addition, Tegsedi (inotersen) received ANVISA approval for the treatment of stage 1 or 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR). In 2019, PTC established an early access program for patients with Familial Chylomicronemia Syndrome (FCS) in Latin America. PTC anticipates filing for approval of Waylivra® (volanesorsen) for the treatment of FCS from ANVISA in 2H 2020.
- Real-world data from the Translarna STRIDE registry demonstrated that boys with nonsense mutation Duchenne muscular dystrophy (nmDMD) treated with Translarna and standard of care, preserved the ability to walk for 3.5 years longer than compared with those in a matched natural history cohort. Importantly, pulmonary function was also preserved in those treated with Translarna.
- A recent publication on Emflaza adds to the growing body of evidence showing its relative benefit and supports the advantage of switching from prednisone to Emflaza. The data in the publication demonstrated that patients treated with Emflaza had better ambulatory function and were able to walk longer, had a lower risk of scoliosis, greater percentage lean body mass and lower weight than patients treated with prednisone.

Positive pivotal trials for risdiplam support planned global launch in broad range of SMA patient types

- Following the U.S. Food and Drug Association's (FDA) acceptance of the New Drug Application (NDA) for risdiplam, a Prescription Drug User Fee Act (PDUFA) date for risdiplam has been set for May 24, 2020.
- Roche's Marketing Authorization Application (MAA) filing with the European Medicines Agency (EMA) for risdiplam is expected mid-year 2020.
- Data from part 2 of the FIREFISH trial will be presented during the American Academy of Neurology (AAN) 2020 Annual Meeting, from April 25 to May 1, 2020.

PTC gene therapy platform continues to make progress with regulatory filings and long-term lease of manufacturing facility

- In January 2020, the EMA accepted the MAA for the potential approval of PTC-AADC for the treatment of Aromatic l-amino acid decarboxylase (AADC) deficiency. PTC expects the Committee for Medicinal Products for Human Use (CHMP) opinion by year end 2020.
- PTC expects to submit a Biologics License Application (BLA) to the FDA in 2Q 2020.
- PTC continues to anticipate that more than 300 global, addressable AADC patients will be identified by launch.
- In 2019, PTC leased a state-of-the-art biologics manufacturing facility with supporting research and operations buildings in N.J. PTC expects manufacturing at this facility to begin in 2020.

Continued progress with PTC's multi-platform, multi-product pipeline

- The Phase 2 STAR trial in patients with nonsense mutation aniridia evaluating the effect of ataluren did not meet its primary endpoint of Maximum Reading Speed as measured by MNREAD. The results did show a trend in favor of ataluren and the results will be discussed with experts in the coming weeks, after which a decision will be made on a path forward

for the program in this indication.
- Topline data from the U.S. study to assess dystrophin levels in subjects with nmDMD after treatment with ataluren are expected in 2Q 2020.
- In 2019, PTC declared a development candidate in its Huntington's Disease program. The compound is currently in GLP safety toxicology studies and PTC expects to initiate a clinical trial by the end of the year. The Huntington's Disease program was developed through PTC's alternative splicing platform.
- In 2020, PTC plans to initiate three clinical trials in two compounds that regulate inflammation and oxidative stress from its Bio-e (redox) platform. PTC acquired the Bio-e platform through an acquisition of assets from BioElectron Technology Corporation in 2019.
    - Potential registrational trial of PTC743 in patients with Mitochondrial Epilepsy (MEDS) expected to begin in 2Q 2020. MEDS is estimated to affect 5,000-6,000 patients in the U.S. and E.U. combined.
    - Potential registrational trial of PTC743 in patients with Friedreich Ataxia (FA) expected to begin in 3Q 2020. This is anticipated to be complementary to PTC-FA, a gene therapy candidate for the treatment of FA, which is expected to enter the clinic in 3Q 2020. FA is estimated to affect 25,000 patients worldwide.
    - PTC857 will enter into a Phase 1 healthy volunteer study in 3Q 2020. The first indication for PTC857 is planned to be GBA Parkinson's disease based on strong pre-clinical rationale. This condition affects approximately 50,000-90,000 patients in the U.S.
- Additional updates on PTC's platforms and pipeline will be provided at PTC's Analyst Day on June 16, 2020.

Fourth Quarter and Full-year 2019 Financial Highlights:

- Total revenues were $96.5 million for the fourth quarter of 2019, compared to $86.3 million for the fourth quarter of 2018. Total revenues were $307 million for the full year 2019, compared to $264.7 million for the full year 2018.
- Translarna net product revenues were $48.4 million for the fourth quarter of 2019, compared to $56 million for the fourth quarter of 2018. Translarna net product revenues were $190 million for the full year 2019, compared to $171 million for the full year 2018.
- Emflaza net product revenues were $32.7 million for the fourth quarter of 2019, compared to $29.8 million for the fourth quarter of 2018. Emflaza net product revenues were $101 million for the full year 2019, compared to $92 million for the full year 2018.
- GAAP R&D expenses were $81.8 million for the fourth quarter of 2019, compared to $53.6 million for the fourth quarter of 2018. GAAP R&D expenses were $257.4 million for the full year 2019, compared to $172 million for the full year 2018. The increase in R&D expenses reflects costs associated with advancing the gene therapy platform, increased investment in research programs, advancement of the clinical pipeline and the upfront $10M payment for the acquisition of the BioElectron assets.
- Non-GAAP R&D expenses were $76.2 million for the fourth quarter of 2019, excluding $5.6 million in non-cash, stock-based compensation expense, compared to $49.7 million for the fourth quarter of 2018, excluding $4 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $236.6 million for the full year 2019, excluding $20.8 million in non-cash, stock-based compensation expense, compared to $155.9 million for the full year 2018, excluding $16.1 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $63.5 million for the fourth quarter of 2019, compared to $48.7 million for the fourth quarter of 2018. GAAP SG&A expenses were $202.5 million for the full year 2019, compared to $153.6 million for the full year 2018. The increase in SG&A expenses was primarily due to continued investment to support our commercial activities.
- Non-GAAP SG&A expenses were $57.7 million for the fourth quarter of 2019, excluding $5.8 million in non-cash, stock-based compensation expense, compared to $44.2 million for the fourth quarter of 2018, excluding $4.5 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $181.2 million for the full year 2019, excluding $21.3 million in non-cash, stock-based compensation expense, compared to $136.4 million for the full year 2018, excluding $17.2 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $12.4 million for the quarter ended December 31, 2019, compared to $19.3 million for the quarter ended December 31, 2018. Change in the fair value of deferred and contingent consideration was $48.4 million for the year ended December 31, 2019, compared to $19.3 million for the year ended December 31, 2018. The change is related to the fair valuation of the potential future consideration to be paid to former equity holders of Agilis, as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.
- Net loss was $77.7 million for the fourth quarter of 2019, compared to net loss of $48.3 million for the fourth quarter of 2018. Net loss was $251.6 million for the full year 2019, compared to net loss of $128.1 million for the full year 2018.
- Cash, cash equivalents, and marketable securities was $686.6 million at December 31, 2019, compared to $227.6 million at December 31, 2018.
- Shares issued and outstanding as of December 31, 2019 were 61,935,870.

PTC Reaffirms Full Year 2020 Guidance as Follows

- PTC anticipates full year DMD franchise net product revenues to be between $320 and $340 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2020 to be between $610 and $640 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2020 to be between $545 and $575 million, excluding estimated non-cash, stock-based compensation expense of approximately $65 million.

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 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 December 31,		Twelve Months Ended December 31,	
	2019	**2018**	**2019**	**2018**
Revenues:				
Net product revenue	$ 81,407	$ 85,833	$ 291,306	$ 263,005
Collaboration and grant revenue	15,052	505	15,674	1,729
Total revenues	96,459	86,338	306,980	264,734
Operating expenses:				
Cost of product sales	3,542	3,761	12,135	12,670
Amortization of acquired intangible asset	7,973	6,062	27,650	22,877
Research and development (1)	81,831	53,647	257,452	171,984
Selling, general and administrative (2)	63,497	48,666	202,541	153,548
Change in the fair value of deferred and contingent consideration	12,400	19,340	48,360	19,340
Total operating expenses	169,243	131,476	548,138	380,419
Loss from operations	(72,784)	(45,138)	(241,158)	(115,685)
Interest expense, net	(5,463)	(3,248)	(12,491)	(12,554)
Other expense (income), net	11,214	(937)	13,723	129
Loss before income tax expense	(67,033)	(49,323)	(239,926)	(128,110)
Income tax (expense) benefit	(10,644)	993	(11,650)	29
Net loss attributable to common stockholders	$ (77,677)	$ (48,330)	$ (251,576)	$ (128,081)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	56,570,002	50,331,914	58,863,185	46,576,313
Net loss per share—basic and diluted (in dollars per share)	$ (1.37)	$ (0.96)	$ (4.27)	$ (2.75)
(1) Research and development reconciliation				
GAAP research and development	$ 81,831	$ 53,647	$ 257,452	$ 171,984
Less: share-based compensation expense	5,645	3,986	20,836	16,096
Non-GAAP research and development	$ 76,186	$ 49,661	$ 236,616	$ 155,888
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 63,497	$ 48,666	$ 202,541	$ 153,548
Less: share-based compensation expense	5,821	4,492	21,298	17,156
Non-GAAP selling, general and administrative	$ 57,676	$ 44,174	$ 181,243	$ 136,392

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

	December 31, 2019	**December 31, 2018**
Cash, cash equivalents and marketable securities	$ 686,563	$ 227,586
Total Assets	**$ 1,623,782**	**$ 1,119,222**
Total debt	$ 313,859	$ 153,014
Total deferred revenue	11,657	13,438
Total liabilities	**$ 1,029,452**	**$ 768,495**

Total stockholders' equity (61,935,870 and 50,606,147 common shares issued and outstanding at December 31, 2019 and December 31, 2018 respectively)	$ 594,330	$ 350,727
Total liabilities and stockholders' equity	**$ 1,623,782**	**$ 1,119,222**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2020 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 610,000	$ 640,000
Less: projected non-cash, stock-based compensation expense	65,000	65,000
Projected non-GAAP R&D and SG&A expense	**$ 545,000**	**$ 575,000**

Today's Conference Call and Webcast Reminder:
Today's conference call will take place at 4:30 pm ET and 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 1732477. 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.

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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 "Full Year 2020 Guidance", including with respect to (i) 2020 net product revenue guidance and (ii) 2020 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 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; 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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