PTC Therapeutics Reports Fourth Quarter and Full Year 2018 Financial Results and Provides a Corporate Update

February 28, 2019

SOUTH PLAINFIELD, N.J., Feb. 28, 2019 /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, 2018.

"We are proud to be in the strong position of having a growing revenue base and robust pipeline with both small molecule and gene therapy programs," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "In our corporate presentation we have outlined our vision to develop these commercial and pipeline programs to achieve potential revenues of $1.5 billion by 2023."

Key Fourth Quarter and Other Corporate Highlights:

Advancing gene therapy portfolio

- PTC plans to submit a Biologics Licensing Application (BLA) with the FDA by late 2019 followed by a Marketing Authorization Application (MAA) in Europe for the AADC deficiency gene therapy program. A U.S. commercial launch is expected in 2020. Identification of patients with AADC deficiency has been a priority for PTC, with approximately 100 patients identified to date in the U.S. and Europe and additional patients being identified on a weekly basis. Starting this quarter, PTC expects to screen about 100,000 patients who are at risk for AADC deficiency before the regulatory approval to maximize patient benefit at time of launch.
- Friedreich's ataxia program is advancing with an expected IND submission in late 2019 and subsequent entry into the clinic.
- PTC continues to build its gene therapy pipeline through investment in internal research and in-house manufacturing capabilities.

Risdiplam SMA regulatory submission planned for 2019

- Based on recent regulatory interactions, an NDA/MAA is planned for the second half of 2019 with the intention to support a broad label to treat SMA Types 1, 2, & 3 patients.
- Successfully completed enrollment of pivotal portion of FIREFISH & SUNFISH trials in 2018.
- The SMA program is a collaboration between PTC, Roche and the SMA Foundation.
- As an orally available small molecule, risdiplam has the potential to be the most competitive SMA product globally. 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.

Expanding commercial platform

- TEGSEDI™ application filed with Brazilian regulatory authority (ANVISA) and granted priority review, with expected approval by year end 2019.
- Duchenne franchise expected to continue to grow over the next 5 years. Translarna™ ex-U.S. launch in patients 2 to 5 years of age now initiated. Non-ambulatory label expansion is currently under EMA regulatory review.
- PTC recently submitted a supplementary NDA (sNDA) for Emflaza® for patients 2 to 5 years old and has recently received an approval action date of July 4, 2019.

Growing pipeline and R&D capabilities

- 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 to IND-enabling studies to enter the clinic in late 2019. This program is in collaboration with Massachusetts General Hospital and NYU.
- Translarna's dystrophin study was initiated in 4Q 2018 for potential U.S. regulatory re-submission for accelerated approval in 2020.
- PTC's oncology portfolio continues to advance with the initiation of a study in AML with PTC299 and a DIPG study for PTC596. PTC expects these studies to fully enroll by the end of 2019.

Fourth Quarter and Full year 2018 Financial Highlights:

- Total revenues were $86.3 million for the fourth quarter of 2018, compared to $78.0 million for the fourth quarter of 2017.

Total revenues were $264.7 million for the full year 2018, compared to $194.4 million for the full year 2017. The change in total revenue was a result of revenue from Emflaza, which launched in May 2017, and the expanded commercialization of Translarna.

- Translarna net product revenues were $56.0 million for the fourth quarter of 2018, compared to $41.0 million for the fourth quarter of 2017. Translarna net product revenues were $171.0 million for the full year 2018, compared to $145.2 million for the full year 2017.
- Emflaza net product revenues were $29.8 million for the fourth quarter of 2018, compared to $17.0 million for the fourth quarter of 2017. Emflaza net product revenues were $92.0 million for the full year 2018, compared to $28.8 million for the full year 2017.
- GAAP R&D expenses were $53.6 million for the fourth quarter of 2018, compared to $29.2 million for the fourth quarter of 2017. GAAP R&D expenses were $172.0 million for the full year 2018, compared to $117.5 million for the full year 2017. 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 $49.6 million for the fourth quarter of 2018, excluding $4.0 million in non-cash, stock-based compensation expense, compared to $25.7 million for the fourth quarter of 2017, excluding $3.5 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $155.9 million for the full year 2018, excluding $16.1 million in non-cash, stock-based compensation expense, compared to $102.0 million for the full year 2017, excluding $15.5 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $48.7 million for the fourth quarter of 2018, compared to $35.5 million for the fourth quarter of 2017. GAAP SG&A expenses were $153.6 million for the full year 2018, compared to $121.3 million for the full year 2017. The increase in SG&A expenses was primarily due to continued investment in commercial activities for Emflaza and Translarna.
- Non-GAAP SG&A expenses were $44.2 million for the fourth quarter of 2018, excluding $4.5 million in non-cash, stock-based compensation expense, compared to $32.5 million for the fourth quarter of 2017, excluding $3.0 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $136.4 million for the full year 2018, excluding $17.2 million in non-cash, stock-based compensation expense, compared to $106.2 million for the full year 2017, excluding $15.1 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $19.3 million for the fourth quarter and full year 2018. 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 Agilis' equity holders in connection with PTC's acquisition of Agilis, which closed in August 2018.
- Net loss was $48.3 million for the fourth quarter of 2018, compared to net income of $1.3 million for the fourth quarter of 2017. Net loss was $128.1 million for the full year 2018, compared to net loss of $79.0 million for the full year 2017.
- Cash, cash equivalents, and marketable securities was $227.6 million at December 31, 2018, compared to $191.2 million at December 31, 2017.
- Shares issued and outstanding as of December 31, 2018 were 50.6 million.
- PTC recently completed a public offering of 7,563,725 shares of common stock resulting in net offering proceeds of $224.1 million.

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.

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 measure 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 3370226. 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 company 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 "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 and potential commercialization of TEGSEDI and Waylivra; expansion of commercialization of Translarna and Emflaza; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions; 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," "vision," "project," 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 Annual Report on Form 10-K for the year ended December 31, 2018, 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 December 31,		Twelve Months Ended December 31,	
	2018	**2017**	**2018**	**2017**
Revenues:				

Net product revenue	$ 85,833	$ 57,953	$ 263,005	$ 174,066
Collaboration and grant revenue	505	20,077	1,729	20,326
Total revenues	86,338	78,030	264,734	194,392
Operating expenses:				
Cost of product revenues	3,761	2,434	12,670	4,577
Amortization of acquired intangible asset	6,062	5,428	22,877	15,380
Research and development (1)	53,647	29,234	171,984	117,456
Selling, general and administrative (2)	48,666	35,482	153,548	121,271
Change in the fair value of deferred and contingent consideration	19,340	—	19,340	—
Total operating expenses	131,476	72,578	380,419	258,684
(Loss) income from operations	(45,138)	5,452	(115,685)	(64,292)
Interest expense, net	(3,248)	(3,446)	(12,554)	(12,094)
Other (expense) income, net	(937)	93	129	(1,279)
(Loss) income before income tax expense	(49,323)	2,099	(128,110)	(77,665)
Income tax benefit (expense)	993	(829)	29	(1,335)
Net (loss) income attributable to common stockholders	$ (48,330)	$ 1,270	$ (128,081)	$ (79,000)
Weighted-average shares outstanding:				
Basic (in shares)	50,331,914	41,344,897	46,576,313	39,183,073
Diluted (in shares)	50,331,914	41,965,276	46,576,313	39,183,073
Net (loss) income per share—basic and diluted (in dollars per share)	$ (0.96)	$ 0.03	$ (2.75)	$ (2.02)
(1) Research and development reconciliation				
GAAP research and development	$ 53,647	$ 29,234	$ 171,984	$ 117,456
Less: share-based compensation expense	3,986	3,470	16,096	15,456
Non-GAAP research and development	$ 49,661	$ 25,764	$ 155,888	$ 102,000
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 48,666	$ 35,483	$ 153,548	$ 121,271
Less: share-based compensation expense	4,492	3,007	17,156	15,103
Non-GAAP selling, general and administrative	$ 44,174	$ 32,476	$ 136,392	$ 106,168

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

	December 31, 2018	December 31, 2017
Cash, cash equivalents and marketable securities	$ 227,586	$ 191,246
Total assets	**$ 1,119,222**	**$ 391,653**
Total debt	$ 153,014	$ 144,971
Total deferred revenue	13,438	11,891
Total liabilities	**$ 768,495**	**$ 235,216**
Total stockholders' equity (50,606,147 and 41,612,395 common shares issued and outstanding at December 31, 2018 and December 31, 2017, respectively)	350,727	156,437
Total liabilities and stockholders' equity	**$ 1,119,222**	**$ 391,653**

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
Total projected non-GAAP R&D and SG&A expense	**$ 360,000**	**$ 370,000**

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