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

August 6, 2019

-Translarna™ approved in Brazil & PTC entered first annual contract with Ministry of Health-
-Emflaza® receives FDA approval for a label expansion to treat DMD patients 2-5 years of age-
-PTC expands gene therapy infrastructure & portfolio-

SOUTH PLAINFIELD, N.J., Aug. 6, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ending June 30, 2019.

"Over the second quarter we've made important progress towards our strategic plan of building a robust diversified orphan drug franchise," said Stuart W. Peltz, Ph.D., CEO of PTC Therapeutics. "We believe that one of PTC's key differentiators is our ability to work across multiple scientific platforms to bring orphan products to patients. Our 5-year vision elucidates PTC's mission of bringing differentiated therapies to patients living with rare disorders that have limited or no treatment options. By fulfilling this mission, we will be creating value for all of our stakeholders."

Key Second Quarter and Other Corporate Highlights:

Commercial portfolio

- Translarna received approval from the Brazilian health regulatory authority (ANVISA) and subsequently, PTC has entered into its first annual contract with the Brazilian Ministry of Health.
- Emflaza® received FDA approval for a label expansion to include DMD patients aged 2-5 years. This demographic is estimated to comprise approximately 25% of the prevalent DMD population in the U.S.
- First commercial patient on Tegsedi™ inLatin America received treatment. The regulatory application submitted to the Brazilian health regulatory authority (ANVISA) was granted priority review, with expected approval by year end 2019.
- We are working with Akcea to review the recent clinical results for Waylivra® in Familial Partial Lipodystrophy (FPL) and we will determine the potential commercial strategy in LATAM.
- The CHMP adopted a negative opinion to expand the label for Translarna to include non-ambulatory DMD patients. PTC has requested a re-examination with a tentative date for an opinion in October.

Advancing gene therapy portfolio & infrastructure

- PTC has signed a long-term lease agreement securing a state-of-the-art biologics facility to support the Company's expansion into multiple gene therapy programs. The facility is currently operating under cGMP standards by its current tenant, Bristol-Myers Squibb, and will be fully transitioned to PTC by mid-2020. PTC intends to consolidate its discovery and research operations in the same campus.
- The expected BLA for our gene therapy candidate to treat patients with AADC deficiency is on track for submission to the FDA in Q4 2019, with anticipated commercial launch in the U.S. in 2020.
- PTC has expanded its gene therapy portfolio by entering into a strategic licensing agreement with Odylia Therapeutics for multiple preclinical programs to treat rare inherited retinal disorders. The lead program is for Leber Congenital Amaurosis (LCA6), a rare early onset retinal dystrophy.
- Completed strategic equity investment in MRI Interventions provides PTC with devices that directly delivers gene therapies into the CNS.

Risdiplam remains on track for NDA submission with the FDA in 2H19

- Data from pivotal FIREFISH and SUNFISH studies were presented at AAN and demonstrate continued clinical benefit with risdiplam in Type 1, 2, and 3 SMA.
- Risdiplam continues to be well tolerated at all doses across studies and there have been no drug related safety findings leading to withdrawal.
- Planned NDA filing with the FDA is on track for the second half of this year with the intention to support a broad label to treat SMA Types 1, 2, & 3 patients. Filing of the MAA in the EU is expected to occur in the first half of 2020.

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.

Second quarter 2019 financial highlights:

- Total revenues were $85.5 million for the second quarter of 2019, compared to $68.7 million for the second quarter of 2018.
- Translarna net product revenues were $57.8 million for the second quarter of 2019, compared to $47.8 million for the second quarter of 2018. These results reflect the expanded commercialization of Translarna.
- Emflaza net product revenues were $27.6 million for the second quarter of 2019, compared to $20.3 million for the second quarter of 2018. These results reflect the continued transition to a new specialty pharmacy distributor.
- GAAP R&D expenses were $60.0 million for the second quarter of 2019, compared to $32.6 million for the second quarter of 2018. The increase in R&D expenses reflects costs associated with advancing the gene therapy platform, increased investment in research programs and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $54.5 million for the second quarter of 2019, excluding $5.5 million in non-cash, stock-based compensation expense, compared to $28.7 million for the second quarter of 2018, excluding $3.9 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $49.2 million for the second quarter of 2019, compared to $33.5 million for the second quarter of 2018. The increase in SG&A expenses reflects continued investment in commercial activities including our expanding commercial portfolio.
- Non-GAAP SG&A expenses were $43.8 million for the second quarter of 2019, excluding $5.4 million in non-cash, stock-based compensation expense, compared to $29.4 million for the second quarter of 2018, excluding $4.1 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $5.3 million for the second 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 $41.8 million for the second quarter of 2019, compared to net loss of $9.5 million for the second quarter of 2018.
- Cash, cash equivalents, and marketable securities were $363.5 million at June 30, 2019, compared to $227.6 million at December 31, 2018.
- Shares issued and outstanding as of June 30, 2019 were 58,707,185.

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.

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 6594813. 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. To learn more about PTC, please visit us on www.ptcbio.com and follow us on Facebook, on Twitter at @PTCBio, and on LinkedIn.

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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; expectations with respect to the potential financial impact and benefits of its leased biologics facility and PTC's ability to satisfy its obligations under the terms of the lease agreement for such 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.

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

	Three Months Ended June 30,		Six Months Ended June 30,	
	2019	2018	2019	2018
Revenues:				
Net product revenue	$ 85,476	$ 68,170	$ 138,530	$ 124,151
Collaboration and grant revenue	46	573	575	654
Total revenues	85,522	68,743	139,105	124,805
Operating expenses:				
Cost of product sales	3,211	2,572	5,587	5,616
Amortization of Acquired intangible assets	6,575	5,593	12,652	11,022
Research and development (1)	59,979	32,607	112,544	63,970
Selling, general and administrative (2)	49,215	33,545	89,760	66,514
Change in the fair value of deferred and contingent consideration	5,300	—	26,460	—
Total operating expenses	124,280	74,317	247,003	147,122
Loss from operations	(38,758)	(5,574)	(107,898)	(22,317)
Interest expense, net	(2,074)	(2,884)	(4,362)	(6,187)
Other (expense) income, net	(183)	(673)	(292)	332
Loss before income tax expense	(41,015)	(9,131)	(112,552)	(28,172)
Income tax expense	(774)	(389)	(1,350)	(610)
Net loss attributable to common stockholders	$ (41,789)	$ (9,520)	$ (113,902)	$ (28,782)

Weighted-average shares outstanding:				
Basic and diluted (in shares)	55,912,748	46,137,833	57,113,141	46,257,397
Net loss per share—basic and diluted (in dollars per share)	$ (0.75)	$ (0.21)	$ (1.99)	$ (0.62)
(1) Research and development reconciliation				
GAAP research and development	$ 59,979	$ 32,607	$ 112,544	$ 63,970
Less: share-based compensation expense	5,516	3,932	10,203	7,678
Non-GAAP research and development	$ 54,463	$ 28,675	$ 102,341	$ 56,292
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 49,215	$ 33,545	$ 89,760	$ 66,514
Less: share-based compensation expense	5,404	4,152	9,981	8,153
Non-GAAP selling, general and administrative	$ 43,811	$ 29,393	$ 79,779	$ 58,361

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

	June 30, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 363,541	$ 227,586
Total Assets	**$ 1,291,572**	**$ 1,119,222**
Total Debt	$ 155,683	$ 153,014
Total deferred revenue	15,802	13,438
Total liabilities	**802,887**	**768,495**
Total stockholders' equity (58,707,185 and 50,606,147 common shares issued and outstanding at June 30, 2019 and December 31, 2018 respectively)	$ 488,685	$ 350,727
Total liabilities and stockholders' equity	**$ 1,291,572**	**$ 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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