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

October 29, 2019

Tegsedi® approved in Brazil & PTC is prepared for launch
PTC completed financing with net proceeds of approximately $376 million

SOUTH PLAINFIELD, N.J., Oct. 29, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the third quarter ending September 30, 2019.

"We are following through on our commitment to build an innovative, diversified rare disorders company," said Stuart Peltz, Ph.D., CEO of PTC Therapeutics. "This quarter, we completed a successful financing to continue to drive our internal programs while continuing to evaluate select external opportunities. This most recent financing has put us in a position to execute on our strategic vision of reaching over $1.5B in revenue by 2023."

Corporate Highlights:

- Tegsedi® (inotersen) received approval from the Brazilian health regulatory authority (ANVISA) for the treatment of stage 1 or 2 polyneuropathy in adult patients with hereditary transthyretin amyloidosis (hATTR), to delay disease progression and improve quality of life. This approval will allow PTC to initiate the launch in Brazil with the only hATTR indication globally to include improvements in quality of life.
- PTC acquired assets from BioElectron Technology Corporation that are focused on inflammatory and central nervous system (CNS) disorders. The lead program is pivotal trial ready for CNS disorders with substantial unmet need and significant commercial opportunity that are complementary to PTC's existing pipeline.

Advancing CNS Gene Therapy Portfolio & Infrastructure:

- PTC has entered into a strategic collaboration with Aldevron to support GMP plasmid manufacturing for the gene therapy portfolio.
- As previously disclosed, PTC is on track to submit a BLA with the FDA in late 2019 followed by an MAA in Europe for the AADC deficiency gene therapy program. This will be followed by an anticipated commercial launch in 2020.
- Data presented in October at the Child Neurology Society meeting demonstrated that patients receiving PTC-AADC had sustained motor, cognitive, and language milestones representing up to five years of follow up post-treatment.
- The Friedreich ataxia program continues to advance with an IND submission now expected in mid-2020.
- PTC has signed a long-term lease agreement securing a state-of-the-art biologics facility located in Hopewell, N.J. to support the research and operations of multiple gene therapy programs.

Updates for Small Molecule Splicing Platform:

- Data from pivotal FIREFISH and SUNFISH part 1 studies were presented at the World Muscle Society Congress in October, demonstrating continued clinical benefit with risdiplam in Type 1, 2, and 3 SMA patients.
    - In part 1 of FIREFISH, babies with Type 1 SMA continue to achieve motor milestones including at least one patient now standing and two patients starting to walk.
    - Risdiplam continues to be well tolerated at all doses across studies and there have been no drug related safety findings leading to withdrawal.
- Part 2 SUNFISH data is expected by the end of the year followed by part 2 FIREFISH data in early 2020.
- 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.
- Based on initial feedback from the FDA on the complexity of a clinical pathway for the small population of patients, PTC has decided to discontinue the Familial Dysautonomia oral splicing program.
- PTC continues to prioritize our Huntington's program, which is scaling up with safety and toxicology work and is expected to enter the clinic in 2020.

PTC Full Year 2019 Guidance:

- PTC anticipates DMD franchise net product revenues for the full year 2019 to remain between $285 and $305 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2019 to be between $420 and $430 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2019 to be between $380 and $390 million, an increase from $360 and $370 million, excluding estimated non-cash, stock-based compensation expense of approximately

$40 million, an increase from $35 million. This increase in operating expense is primarily due to the acceleration of activities in our core programs, including research and gene therapy manufacturing.

Third Quarter 2019 Financial Highlights:

- Total revenues were $71.4 million for the third quarter of 2019, compared to $53.6 million for the third quarter of 2018.
- Translarna™ net product revenues were $48.3 million for the third quarter of 2019, compared to $30.4 million for the third quarter of 2018. These results reflect the expanded commercialization of Translarna.
- Emflaza® net product revenues were $22.9 million for the third quarter of 2019, compared to $22.6 million for the third quarter of 2018. These results reflect an increase in the utilization of Medicaid which changed our gross to net assumptions and includes the impact of transitioning to a new specialty pharmacy distributor.
- GAAP R&D expenses were $63.1 million for the third quarter of 2019, compared to $54.4 million for the third 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 $58.1 million for the third quarter of 2019, excluding $5.0 million in non-cash, stock-based compensation expense, compared to $49.9 million for the third quarter of 2018, excluding $4.4 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $49.3 million for the third quarter of 2019, compared to $38.4 million for the third quarter of 2018. The increase in SG&A expenses was primarily due to continued investment in support of our commercial activities.
- Non-GAAP SG&A expenses were $43.8 million for the third quarter of 2019, excluding $5.5 million in non-cash, stock-based compensation expense, compared to $33.9 million for the third quarter of 2018, excluding $4.5 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $9.5 million for the third 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 $60.0 million for the third quarter of 2019, compared to net loss of $51.0 million for the third quarter of 2018.
- Cash, cash equivalents, and marketable securities were $708.6 million at September 30, 2019, compared to $227.6 million at December 31, 2018. We completed a financing that amounted to $287.5 million in convertible bonds and $100.0 million in equity for a total consideration of $387.5 million, resulting in net offering proceeds of $376.3 million.
- Shares issued and outstanding as of September 30, 2019 were 61,578,992.

Upcoming investor conferences

Credit Suisse 28th Annual Healthcare Conference, November 12th 2:25 pm MT.

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 9224968. 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-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 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 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; the intended use of proceeds from PTC's public offering of common stock and private offering of convertible senior notes; 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 equity holders 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 September 30,		Nine Months Ended September 30,	
	2019	**2018**	**2019**	**2018**
Revenues:				
Net product revenue	$ 71,369	$ 53,021	$ 209,899	$ 177,172
Collaboration and grant revenue	47	570	622	1,224
Total revenues	71,416	53,591	210,521	178,396
Operating expenses:				
Cost of product sales	3,006	3,292	8,593	8,909
Amortization of Acquired intangible asset	7,025	5,793	19,677	16,815
Research and development (1)	63,076	54,368	175,621	118,337
Selling, general and administrative (2)	49,284	38,368	139,044	104,882
Change in the fair value of deferred and contingent consideration	9,500	—	35,960	—

Total operating expenses	131,891	101,821	378,895	248,943
Loss from operations	(60,475)	(48,230)	(168,374)	(70,547)
Interest expense, net	(2,666)	(3,118)	(7,028)	(9,306)
Other (expense) income, net	2,800	734	2,509	1,066
Loss before income tax expense	(60,341)	(50,614)	(172,893)	(78,787)
Income tax expense	344	(355)	(1,006)	(964)
Net loss attributable to common stockholders	$ (59,997)	$ (50,969)	$ (173,899)	$ (79,751)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	56,463,528	48,096,521	57,798,968	45,310,690
Net loss per share—basic and diluted (in dollars per share)	$ (1.06)	$ (1.06)	$ (3.01)	$ (1.76)
(1) Research and development reconciliation				
GAAP research and development	$ 63,076	$ 54,368	$ 175,621	$ 118,337
Less: share-based compensation expense	4,988	4,431	15,191	12,109
Non-GAAP research and development	$ 58,088	$ 49,937	$ 160,430	$ 106,228
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 49,284	$ 38,368	$ 139,044	$ 104,882
Less: share-based compensation expense	5,496	4,511	15,477	12,664
Non-GAAP selling, general and administrative	$ 43,788	$ 33,857	$ 123,567	$ 92,218

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

	September 30, 2019	December 31, 2018
Cash, cash equivalents and marketable securities	$ 708,649	$ 227,586
Total Assets	**$ 1,641,486**	**$ 1,119,222**
Total Debt	$ 313,149	$ 153,014
Total deferred revenue	14,306	13,438
Total liabilities	**977,832**	**768,495**
Total stockholders' equity (61,578,992 and 50,606,147 common shares issued and outstanding at September 30, 2019 and December 31, 2018 respectively)	$ 663,654	$ 350,727
Total liabilities and stockholders' equity	**$ 1,641,486**	**$ 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	$ 420,000	$ 430,000
Less: projected non-cash, stock-based compensation expense	40,000	40,000
Projected non-GAAP R&D and SG&A expense	**$ 380,000**	**$ 390,000**

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