PTC Therapeutics Reports Third Quarter 2020 Financial Results and Provides a Corporate Update

October 29, 2020

- Evrysdi™ makes rapid progress with multiple international approvals and strong U.S. launch -

- Total net revenue of $118.4 million, 66% YoY increase; Total net product revenue of $82.7 million, 16% YoY increase -

- Initiated registration-directed trial with vatiquinone (PTC743) for mitochondrial epilepsy -

SOUTH PLAINFIELD, N.J., Oct. 29, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced financial results for the third quarter ending September 30, 2020 and provided a corporate update.

"I am very pleased by the rapid progress that we have made this quarter with the approval and strong U.S. launch of Evrysdi™ for spinal muscular atrophy and the continued growth across our global Duchenne muscular dystrophy franchise," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We have also made significant advances across our clinical pipeline with a registration-directed trial in mitochondrial epilepsy initiated this quarter and an additional registration-directed trial in Friedreich ataxia to initiate before year end. The mission at PTC has always been to bring therapies to patients with high unmet medical need through investment in innovative technologies and we believe we are well positioned to bring long-term value for all of our stakeholders."

Key Third Quarter and Other Corporate Updates

- In August 2020, the U.S. Food and Drug Administration (FDA) approved Evrysdi (risdiplam), the first at-home, orally administered treatment for spinal muscular atrophy (SMA) in adults and children 2 months and older. In the early stages of launch, patients across a broad age range and SMA type have been treated. In October 2020, there were additional approvals in Brazil, Chile and Ukraine, further expanding the global reach of Evrysdi. Evrysdi is a product of the SMA collaboration between PTC, the SMA Foundation, and Roche.
- Also in August 2020, the European Medicines Agency (EMA) accepted the Marketing Authorization Application (MAA) filed by Roche for Evrysdi for the treatment of SMA. The EMA previously granted PRIME (PRIority MEdicines) designation to risdiplam for the treatment of SMA, providing a pathway for accelerated evaluation by the agency.
- The EMA's acceptance of the MAA filed by Roche for Evrysdi for the treatment of SMA triggered a $15 million milestone payment to PTC and the first commercial sale of Evrysdi in the U.S. triggered a $20 million milestone payment to PTC. As a result, PTC recognized $35.0 million in collaboration revenue associated with Roche milestone events in the third quarter of 2020.
- In October 2020, Chugai Pharmaceutical, Co. Ltd, a member of the Roche group, announced that a new drug application for Evrysdi for the treatment of SMA was filed in Japan. Risdiplam received orphan drug designation from the Japan Ministry of Health, Labour and Welfare and the application is subject to a priority review. The filing in Japan triggered a $7.5 million milestone payment to PTC in the fourth quarter of 2020. Roche is executing an aggressive global regulatory strategy and now has over 20 additional applications filed for Evrysdi around the world.
- PTC has multiple aromatic L-amino acid decarboxylase (AADC) deficiency patient finding initiatives ongoing which continue to progress even amid COVID-19. PTC has expanded the number of programs globally and in September 2020, PTC launched its newest program, PTC PINPOINT. The program offers testing at no charge to patients which can lead to an earlier diagnosis and treatment.

Third Quarter Clinical Updates

- Two-year data from Part 1 of the Evrysdi FIREFISH study were presented at the 25th International Annual Congress of the World Muscle Society (WMS). The data demonstrated that infants treated with Evrysdi continued to improve and achieve motor milestones.
- A registration-directed Phase 2/3 placebo-controlled trial to evaluate vatiquinone (PTC743) in children with mitochondrial epilepsy began enrollment in October 2020. This MIT-E trial is targeting the highly morbid and life-threatening symptom of refractory seizures in children with inherited mitochondrial disease. Vatiquinone, developed from PTC's Bio-e platform, is an investigational oral small molecule that inhibits 15-Lipoxygenase, a key enzyme that regulates oxidative stress and inflammation response pathways that underpin neurological disease pathology including epilepsy. The FDA has granted vatiquinone orphan drug designation and pediatric rare disease designation for mitochondrial epilepsy.

- The Phase 3 vatiquinone Friedreich ataxia trial (MOVE-FA) is expected to initiate in the fourth quarter of 2020 and the Phase 3 PTC923 phenylketonuria (PKU) trial is expected to initiate in mid-2021.
- Muscle biopsies have been completed on all 20 enrolled subjects in PTC's Translarna ™(ataluren) dystrophin study (Study 045). The samples from these biopsies are in the process of being analyzed and PTC expects to report top-line results in the first quarter of 2021.
- A Phase 1 study of PTC518 in healthy volunteers is expected to begin enrollment this quarter. PTC518 is another candidate from PTC's validated splicing platform and is being developed for the treatment of Huntington disease.
- The Phase 1 single ascending dose study for PTC857 has been completed and dosing in the multiple ascending dose study is expected to be completed by the end of 2020. PTC857 was developed from the Bio-e platform with the potential to address multiple CNS disorders, with the first planned indication being glucocerebrosidase (GBA) Parkinson's disease.
- A Phase 2/3 clinical trial for PTC299 for COVID-19 (FITE19) continues to enroll patients. PTC is expecting Stage 1 of this two-stage trial to be completed by the end of 2020 and continues to anticipate reporting top-line results from both stages in the first half of 2021. Stage 1 of this study is being conducted in the U.S., Brazil, Spain, and Australia.
- Due to COVID-19 related delays, PTC expects the EMA's Committee for Medicinal Products for Human Use final opinion for the AADC deficiency application in the first half of 2021. PTC expects the biologics license application (BLA) for AADC deficiency to be submitted to the FDA in the first half of 2021.

Financial Highlights

- Total revenue was $118.4 million for the third quarter of 2020, compared to $71.4 million for the third quarter of 2019, a 66% year-over-year increase, Total revenue includes net product revenue of $82.7 million and collaboration and royalty revenue of $35.7 million in the third quarter of 2020.
- Total net product revenue across the commercial portfolio was $82.7 million for the third quarter of 2020, a 16% year-over-year increase.
- The Duchenne muscular dystrophy franchise continued to show strong performance in the third quarter with a 15% year-over-year increase. Emflaza® continued its momentum from the second quarter of 2020 into the third quarter of 2020 driven by new patient prescriptions and ongoing operational improvements in PTC's commercial business. New Duchenne patients continue to be identified in Europe, LATAM and other key markets for Translarna.
- Translarna net product revenue was $43.4 million for the third quarter of 2020, compared to $48.3 million for the third quarter of 2019, a 10% year-over-year decrease. Revenue for the third quarter of 2020 was impacted by the timing of a group purchase order from Brazil, which is the primary driver for the year-over-year decrease compared to the third quarter of 2019.
- In October, PTC entered into a purchase agreement with the Brazil Ministry of Health for Translarna. The initial shipment was received by the Brazil Ministry of Health in October 2020 with the additional shipment expected in the first half of 2021.
- Emflaza net product revenue was $38.5 million for the third quarter of 2020, compared to $22.9 million for the third quarter of 2019. Growth in net product revenue was driven by new patient prescriptions and continued operational improvements and efficiencies in the commercial business.
- Roche reported initial Evrysdi August and September sales of approximately CHF 8 million. The acceptance of the MAA filed by Roche for Evrysdi for the treatment of SMA triggered a $15 million milestone payment to PTC and the first commercial sale of Evrysdi in the U.S. triggered a $20 million milestone payment to PTC. As a result, PTC recognized $35.0 million in collaboration revenue associated with Roche milestone events in the third quarter of 2020.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), research and development (R&D) expenses were $93.0 million for the third quarter of 2020, compared to $63.1 million for the third quarter of 2019. The increase in R&D expenses reflects costs associated with advancing the gene therapy and Bio-e platforms, increased investment in research programs, and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $83.8 million for the third quarter of 2020, excluding $9.2 million in non-cash, stock-based compensation expense, compared to $58.1 million for the third quarter of 2019, excluding $5.0 million in non-cash, stock-based compensation expense.
- GAAP selling, general and administrative (SG&A) expenses were $57.8 million for the third quarter of 2020, compared to $49.3 million for the third quarter of 2019. The increase reflects continued investment to support PTC's commercial activities including the expanding commercial portfolio.
- Non-GAAP SG&A expenses were $50.2 million for the third quarter of 2020, excluding $7.6 million in non-cash, stock-based compensation expense, compared to $43.8 million for the third quarter of 2019, excluding $5.5 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $8.4 million for the third quarter of 2020, compared to $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 $69.7 million for the third quarter of 2020, compared to net loss of $60.0 million for the third quarter of 2019.

- Cash, cash equivalents and marketable securities were $1,141.0 million as of September 30, 2020, compared to $686.6 million as of December 31, 2019. The cash, cash equivalents and marketable securities balance as of September 30, 2020 includes $650.0 million in consideration received related to the July 17, 2020 Royalty Purchase Agreement between PTC and RPI 2019 Intermediate Finance Trust ("RPI").
- Shares issued and outstanding as of September 30, 2020 were 67,809,560.

Non-GAAP Financial Measures

In this press release, the financial results of PTC are provided in accordance with 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 September 30,		Nine Months Ended September 30,	
	2020	**2019**	**2020**	**2019**
Revenues:				
Net product revenue	$ 82,708	$ 71,369	$ 226,143	$ 209,899
Collaboration and grant revenue	35,000	47	35,063	622
Royalty revenue	696	-	696	-
Total revenues	118,404	71,416	261,902	210,521
Operating expenses:				
Cost of product sales	4,667	3,006	14,056	8,593
Amortization of acquired intangible asset	9,630	7,025	26,309	19,677
Research and development (1)	92,998	63,076	359,630	175,621
Selling, general and administrative (2)	57,840	49,284	169,708	139,044
Change in the fair value of deferred and contingent consideration	8,400	9,500	16,980	35,960
Settlement of deferred and contingent consideration	-	-	10,613	-
Total operating expenses	173,535	131,891	597,296	378,895
Loss from operations	(55,131)	(60,475)	(335,394)	(168,374)
Interest expense, net	(21,039)	(2,666)	(32,060)	(7,028)
Other income, net	28,766	2,800	26,242	2,509
Loss before income tax expense	(47,404)	(60,341)	(341,212)	(172,893)
Income tax (expense) benefit	(22,288)	344	(22,594)	(1,006)
Net loss attributable to common stockholders	$ (69,692)	$ (59,997)	$ (363,806)	$ (173,899)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	67,641,171	56,463,528	65,068,281	57,798,968
Net loss per share—basic and diluted (in dollars per share)	$ (1.03)	$ (1.06)	$ (5.59)	$ (3.01)
(1) Research and development reconciliation				
GAAP research and development	$ 92,998	$ 63,076	$ 359,630	$ 175,621
Less: share-based compensation expense	9,220	4,988	25,961	15,191
Non-GAAP research and development	$ 83,778	$ 58,088	$ 333,669	$ 160,430
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 57,840	$ 49,284	$ 169,708	$ 139,044
Less: share-based compensation expense	7,559	5,496	22,948	15,477
Non-GAAP selling, general and administrative	$ 50,281	$ 43,788	$ 146,760	$ 123,567

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

	September 30, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 1,141,019	$ 686,563
Total Assets	**$ 2,181,059**	**$ 1,623,782**
Total debt	$ 302,971	$ 313,859
Total deferred revenue	5,992	11,657
Total liability for sale of future royalties	664,258	-
Total liabilities	**$ 1,670,105**	**$ 1,029,452**
Common stock, $0.001 par value. Authorized 125,000,000 shares; issued and outstanding 67,809,560 shares at September 30, 2020. Authorized 125,000,000 shares; issued and outstanding 61,935,870 shares at December 31, 2019.	$ 510,954	$ 594,330
Total liabilities and stockholders' equity	**$ 2,181,059**	**$ 1,623,782**

Conference Call and Webcast

PTC will host a conference call to discuss the third quarter of 2020 operational and financial today 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 7096445. 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. 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. To learn more about PTC, please visit us at 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 statements regarding: the future expectations, plans and prospects for PTC, including with respect to the expected timing of clinical trials and studies, availability of data, regulatory submissions and responses and other matters; 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; the timing with respect to orders for PTC's products; expectations with respect to the impacts of the COVID-19 pandemic and related response measures; 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 ongoing 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 respect to Evrysdi; 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 ™; the enrollment, conduct and results of PTC's PTC299 clinical trial for COVID-19; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, potential regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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; 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, Evrysdi, Tegsedi, Waylivra or PTC-AADC.

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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