PTC Therapeutics Provides a Corporate Update and Reports Third Quarter 2021 Financial Results

October 28, 2021

- Total quarterly revenue of $139 million; 17% increase over third quarter 2020 -
- Raises 2021 DMD franchise revenue guidance to $400-$420M from $370-$390M -
- Continues to advance robust clinical pipeline by initiating a fifth registration-directed trial, APHENITY Phase 3 trial of PTC923 in phenylketonuria (PKU) -
- Brazil achievements include Category 1 pricing for Tegsedi®, regulatory approval of Waylivra™ and expansion of the Translarna™ label to include patients as young as 2 years of age -

SOUTH PLAINFIELD, N.J., Oct. 28, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the third quarter ending September 30, 2021.

"The stellar growth of our DMD franchise is remarkable." said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "I continue to be impressed by the team's performance. The commercial performance, along with the milestones achieved in Brazil, and the continued advancements in our pipeline, with initiating a fifth registration-directed trial, allows us continued value creation for all of our stakeholders."

Key Third Quarter and Other Corporate Updates:

- The Duchenne muscular dystrophy (DMD) franchise grew 39% over the third quarter of 2020, demonstrating PTC's continued strong commercial performance. Quarterly net product revenue for the franchise was $114 million in the third quarter of 2021.
  - Translarna™ (ataluren) revenue growth was driven by geographic expansion and new patient identification.
  - Emflaza® (deflazacort) revenue growth was driven primarily by new patient starts and maintained high compliance.
- Evrysdi™(risdiplam) continues to show strong global uptake. The first commercial sale of Evrysdi™ in Japan occurred in August 2021, resulting in a $10 million milestone payment from Roche to PTC with $325 million in sales-based milestones remaining. Evrysdi™ is a product of a collaboration between PTC, Roche and the SMA Foundation.
- Waylivra™ (volanesorsen) was approved by the Brazilian Health Regulatory Agency, ANVISA (Agência Nacional de Vigilância Sanitária), as the first treatment for familial chylomicronemia syndrome (FCS) in Brazil.
- Tegsedi® (inotersen) has successfully received Category 1 classification from CMED (Drug Market Regulation Chamber) in Brazil. Category 1 classification is given to innovative treatments that provide greater efficacy than current standards of care and allows for pricing in line with international markets.

Third Quarter Clinical Updates:

- The APHENITY registration-directed Phase 3 trial for PTC923 for phenylketonuria (PKU) has been initiated with results expected by the end of 2022.
- PTC continues to make progress in three additional ongoing registration-directed clinical studies:
  - The MIT-E Phase 2/3 vatiquinone trial for mitochondrial disease associated seizures with results anticipated in the third quarter of 2022.
  - The MOVE-FA Phase 3 vatiquinone trial for Friedreich ataxia with results anticipated in 2023.
  - Enrollment in the FITE19 Phase 2/3 emvododstat trial in patients with COVID-19 is expected to be completed by year end 2021.
- The Phase 1 healthy volunteer study of PTC518 for Huntington's disease met the objectives in the third quarter of this year. The Phase 2 study in patients with Huntington's disease is expected to be initiated by the end of 2021.
- The Committee for Medicinal Products for Human Use (CHMP) opinion on the PTC-AADC gene therapy for aromatic L-amino acid decarboxylase (AADC) deficiency is expected in the fourth quarter of 2021.
- For the Biologics License Application (BLA) for AADC deficiency, PTC expects to submit the BLA in the first quarter of 2022.

Third Quarter 2021 Financial Highlights:

- Total revenues were $138.7 million for the third quarter of 2021, compared to total revenues of $118.4 million for the third quarter of 2020.
- Total revenue includes net product revenue across the commercial portfolio of $115.6 million and royalty and collaboration revenue of $23.1 million for the third quarter of 2021, compared to net product revenues of $82.7 million and royalty and collaboration revenues of $35.7 million for the third quarter of 2020.
- Translarna net product revenues were $67.2 million for the third quarter of 2021, compared to $43.4 million for the third quarter of 2020. These results reflect an increase in net product sales in existing markets as well as continued geographic expansion.
- Emflaza net product revenues were $47.1 million for the third quarter of 2021, compared to $38.5 million for the third quarter of 2020. These results reflect new patient prescriptions, high compliance, and fewer discontinuations.

- Roche reported Evrysdi 2021 year to date sales of approximately CHF 396 million, resulting in year-to-date royalty revenue of $33.3 million to PTC. During the third quarter of 2021, the first commercial sale of Evrysdi in Japan triggered a $10 million milestone payment from Roche to PTC, which was reported as collaboration revenue.
- U.S. GAAP (generally accepted accounting principles) research and development (R&D) expenses were $130.8 million for the third quarter of 2021, compared to $93.0 million for the third quarter of 2020. The increase reflects additional investment in research programs and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $117.8 million for the third quarter of 2021, excluding $13.0 million in non-cash stock-based compensation expense, compared to $83.8 million for the third quarter of 2020, excluding $9.2 million in non-cash stock-based compensation expense.
- GAAP selling, general and administrative (SG&A) expenses were $69.3 million for the third quarter of 2021, compared to $57.8 million for the third quarter of 2020. The increase reflects our continued investment to support commercial activities including expanding our commercial portfolio.
- Non-GAAP SG&A expenses were $56.4 million for the third quarter of 2021, excluding $12.8 million in non-cash stock-based compensation expense, compared to $50.3 million for the third quarter of 2020, excluding $7.6 million in non-cash stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $10.8 million for the third quarter of 2021, compared to $8.4 million for the third quarter of 2020. 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 $133.6 million for the third quarter of 2021, compared to net loss of $69.7 million for the third quarter of 2020.
- Cash, cash equivalents and marketable securities was $867.9 million at September 30, 2021, compared to $1.1 billion at December 31, 2020.
- Shares issued and outstanding as of September 30, 2021 were 70,665,010.

PTC Updates Full Year 2021 Guidance as Follows:

- PTC now anticipates net product revenues for the DMD franchise for the full year 2021 to be between $400 and $420 million from previous guidance of $370 and $390 million.
- PTC now anticipates GAAP R&D and SG&A expense for the full year 2021 to be between $815 and $835 million from previous guidance of $825 and $855 million.
- PTC now anticipates Non-GAAP R&D and SG&A expense for the full year 2021 to be between $715 and $735 million, excluding estimated non-cash, stock-based compensation expense of $100 million, from previous guidance of $725 and $755 million.

Non-GAAP Financial Measures:

In this press release, the financial results and financial guidance 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 tables 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,	
	2021	**2020**	**2021**	**2020**
Revenues:				
Net product revenue	$ 115,605	$ 82,708	$ 309,998	$ 226,143
Collaboration revenue	10,011	35,000	30,018	35,063
Royalty revenue	13,127	696	33,348	696
Total revenues	138,743	118,404	373,364	261,902
Operating expenses:				
Cost of product sales	6,539	4,667	23,001	14,056
Amortization of acquired intangible asset	14,383	9,630	38,411	26,309
Research and development (1)	130,845	92,998	390,840	359,630
Selling, general and administrative (2)	69,252	57,840	199,225	169,708
Change in the fair value of deferred and contingent consideration	10,800	8,400	11,600	16,980
Settlement of deferred and contingent	-	-	-	10,613
Total operating expenses	231,819	173,535	663,077	597,296

Loss from operations	(93,076)	(55,131)	(289,713)	(335,394)
Interest expense, net	(21,802)	(21,039)	(63,520)	(32,060)
Other (expense) income, net	(18,782)	28,766	(26,499)	26,242
Loss before income tax expense	(133,660)	(47,404)	(379,732)	(341,212)
Income tax benefit (expense)	36	(22,288)	(904)	(22,594)
Net loss attributable to common stockholders	$ (133,624)	$ (69,692)	$ (380,636)	$ (363,806)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	70,585,938	67,641,171	70,397,846	65,068,281
Net loss per share—basic and diluted (in dollars per share)	$ (1.89)	$ (1.03)	$ (5.41)	$ (5.59)
(1) Research and development reconciliation				
GAAP research and development	$ 130,845	$ 92,998	$ 390,840	$ 359,630
Less: share-based compensation expense	13,048	9,220	40,216	25,961
Non-GAAP research and development	$ 117,797	$ 83,778	$ 350,624	$ 333,669
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 69,252	$ 57,840	$ 199,225	$ 169,708
Less: share-based compensation expense	12,823	7,559	37,061	22,948
Non-GAAP selling, general and administrative	$ 56,429	$ 50,281	$ 162,164	$ 146,760

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

	September 30, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 867,943	$ 1,103,650
Total Assets	**$ 2,007,325**	**$ 2,208,278**
Total debt	$ 430,962	$ 309,145
Total liability for sale of future royalties	723,200	679,762
Total deferred revenue	-	4,151
Total liabilities	**$ 1,902,399**	**$ 1,726,296**
Total stockholders' equity (70,665,010 and 69,718,096 common shares issued and outstanding at September 30, 2021 and December 31, 2020 respectively)	$ 104,926	$ 481,982
Total liabilities and stockholders' equity	**$ 2,007,325**	**$ 2,208,278**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2021 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 815,000	$ 835,000
Less: projected non-cash, stock-based compensation expense	100,000	100,000
Projected non-GAAP R&D and SG&A expense	**$ 715,000**	**$ 735,000**

Today's Conference Call and Webcast Reminder:

PTC will host a conference call to discuss the third quarter of 2021 corporate updates and financial results 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 7064479. 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. The Company's strategy is to leverage its strong scientific expertise and global commercial infrastructure to maximize value for its patients and other stakeholders. 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 the information provided under the heading "PTC Updates Full Year 2021 Guidance as Follows", including with respect to (i) 2021 net product revenue guidance and (ii) 2021 GAAP and non-GAAP R&D and SG&A expense guidance, and 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 regulatory submissions and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including the commercialization of any products therein or royalty or milestone payments; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its other 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: expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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 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 regulatory submissions and commercialization with respect to Evrysdi; PTC's ability to utilize results from Study 041 to support a marketing approval for Translarna for the treatment of nmDMD in the United States; 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 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 clinical trial for emvododstat for COVID-19; 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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