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

May 4, 2021

- PTC518 demonstrated dose-dependent reduction of HTT mRNA in preliminary results from Phase 1 healthy volunteer trial -

- Total net revenue of $118 million; 73% increase over first quarter 2020 -

- DMD franchise net product revenue of $90 million; 32% increase over first quarter 2020 -

- PTC has clinical trials ongoing in multiple disease areas, three of which are registration-directed studies -

SOUTH PLAINFIELD, N.J., May 4, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the first quarter ending March 31, 2021.

"Overall PTC has had a strong performance this quarter through all aspects of the company from discovery to commercial revenue," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "I would like to highlight the continued strong growth of the DMD franchise which has had one of our largest quarterly revenues to date. The other key milestone was the positive preliminary results in our PTC518 Huntington's disease program demonstrating dose dependent lowering of the HTT mRNA. Analogous to the SMA program we are now well positioned with a clear path for success."

Key First Quarter and Other Corporate Updates:

- The Duchenne muscular dystrophy (DMD) franchise had a total net product revenue of $90 million for Translarna™ (ataluren) and Emflaza® (deflazacort) in the first quarter of 2021. This represents 32% growth over the first quarter of 2020 and one of PTC's strongest quarterly commercial revenues to date.
  - Broader uptake due to new patients in existing geographies and geographic expansion drove Translarna growth.
  - Emflaza revenue growth was primarily due to increased new prescriptions, high compliance, and fewer discontinuations.
- In March 2021, the European Medicines Agency (EMA) approved Evrysdi™ (risdiplam) in the European Union (EU). The first sale of Evrysdi in this region was recorded the following day, triggering a $20 million milestone payment to PTC. Evrysdi is a product of a collaboration between PTC, Roche and the SMA Foundation.
- Preliminary results from the PTC518 Phase 1 healthy volunteer trial demonstrated dose-dependent reduction of Huntington mRNA beyond the 30-50% target.
- PTC received Gallup's Don Clifton Strengths-Based Culture Award, which reflects the Company's ongoing deep commitment to its employees.

First Quarter Clinical Updates:

- PTC has multiple clinical trials ongoing, three of which are registration-directed clinical studies:
  - The MIT-E Phase 2/3 trial with vatiquinone for mitochondrial epilepsy with data anticipated in the third quarter of 2022.
  - The MOVE-FA Phase 3 trial with vatiquinone for Friedreich ataxia with data anticipated in 2023.
  - The FITE19 Phase 2/3 clinical trial for PTC299 in patients with COVID-19 with an expected data readout in the second half of 2021.
- The second Bio-e compound, PTC857 healthy volunteer study was recently completed, and data will be communicated in the second quarter.
- The registration-directed Phase 3 PTC923 phenylketonuria (PKU) trial, APHENITY, is expected to initiate in mid-2021.
- The Committee for Medicinal Products for Human Use (CHMP) has requested a clock stop in the aromatic L-amino acid decarboxylase (AADC) deficiency review process to allow for completion of its pre-approval inspections, which were delayed due to COVID-19. The CHMP opinion is now anticipated in the third quarter of 2021.
- Due to COVID-related surgical delays, the AADC-deficiency biologics license application submission to the U.S. Food and Drug Administration is anticipated to be delayed by at least one quarter.

First Quarter 2021 Financial Highlights:

- Total revenues were $117.9 million for the first quarter of 2021, compared to total revenues of $68.3 million for the first quarter of 2020, a 32% increase. Total revenue includes net product revenue of $91.3 million and collaboration and royalty revenue of $26.7 million in the first quarter of 2021.
- Translarna net product revenues were $46.5 million for the first quarter of 2021, compared to $40.5 million for the first

quarter of 2020. These results reflect an increase in net product sales in existing markets as well as continued geographic expansion into new territories.

- Emflaza net product revenues were $43.5 million for the first quarter of 2021, compared to $27.5 million for the first quarter of 2020. These results reflect new patient prescriptions, high compliance, and fewer discontinuations.
- Roche reported Evrysdi first quarter 2021 sales of approximately CHF 80 million. During the first quarter of 2021, the first commercial sale of Evrysdi in the EU triggered a $20 million milestone payment to PTC, which was reported as collaboration revenue.
- U.S. GAAP (generally accepted accounting principles) R&D expenses were $134.5 million for the first quarter of 2021, compared to $90.1 million for the first quarter of 2020. The increase in R&D expenses reflects costs associated with increased investment in research programs, and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $120.8 million for the first quarter of 2021, excluding $13.7 million in non-cash, stock-based compensation expense, compared to $81.9 million for the first quarter of 2020, excluding $8.2 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $61.1 million for the first quarter of 2021, compared to $58.2 million for the first quarter of 2020. The increase in SG&A expenses was associated with entering into a long-term lease for the Hopewell facility that commenced on July 1, 2020.
- Non-GAAP SG&A expenses were $49.1 million for the first quarter of 2021, excluding $12.0 million in non-cash, stock-based compensation expense, compared to $51.2 million for the first quarter of 2020, excluding $7.0 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $0.1 million for the first quarter of 2021, compared to $0.9 million for the first 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 $128.6 million for the first quarter of 2021, compared to net loss of $112.7 million for the first quarter of 2020.
- Cash, cash equivalents and marketable securities was $988.4 million at March 31, 2021, compared to $1.1 billion at December 31, 2020.
- Shares issued and outstanding as of March 31, 2021 were 70,405,905.

PTC Reaffirms Full Year 2021 Guidance as Follows:

- PTC anticipates net product revenues for the DMD franchise for the full year 2021 to be between $355 and $375 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2021 to be between $825 and $855 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2021 to be between $725 and $755 million, excluding estimated non-cash, stock-based compensation expense of $100 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 table below.

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

	Three Months Ended March 31,	
	2021	**2020**
Revenues:		
Net product revenue	$ 91,280	$ 68,196
Collaboration and grant revenue	20,007	63
Royalty revenue	6,655	-
Total revenues	117,942	68,259
Operating expenses:		
Cost of product sales	9,104	4,085
Amortization of acquired intangible asset	11,278	7,494
Research and development (1)	134,513	90,107
Selling, general and administrative (2)	61,095	58,209
Change in FV of deferred & contingent consideration	100	900
Total operating expenses	216,090	161,250
Loss from operations	(98,148)	(92,991)

Interest expense, net	(19,159)	(5,642)
Other expense, net	(10,884)	(13,832)
Loss before income tax expense	(128,191)	(112,465)
Income tax expense	(451)	(222)
Net loss attributable to common stockholders	$ (128,642)	$ (112,687)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	70,188,602	62,389,158
Net loss per share—basic & diluted (in dollars per share)	$ (1.83)	$ (1.81)
(1) Research and development reconciliation		
GAAP research and development	$ 134,513	$ 90,107
Less: share-based compensation expense	13,725	8,179
Non-GAAP research and development	$ 120,788	$ 81,928
(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 61,095	$ 58,209
Less: share-based compensation expense	11,982	7,041
Non-GAAP selling, general and administrative	$ 49,113	$ 51,168

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

	March 31, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 988,406	$ 1,103,650
Total Assets	**$ 2,111,941**	**$ 2,208,278**
Total debt	$ 430,038	$ 309,145
Total liability for sale of future royalties	694,984	679,762
Total deferred revenue	1,887	4,151
Total liabilities	**$ 1,819,365**	**$ 1,726,296**
Total stockholders' equity (70,405,905 and 69,718,096 common shares issued and outstanding at March 31, 2021 and December 31, 2020 respectively)	$ 292,576	$ 481,982
Total liabilities and stockholders' equity	**$ 2,111,941**	**$ 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	$ 825,000	$ 855,000
Less: projected non-cash, stock-based compensation expense	100,000	100,000
Projected non-GAAP R&D and SG&A expense	**$ 725,000**	**$ 755,000**

Today's Conference Call and Webcast Reminder:

PTC will host a conference call to discuss the first 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 4292410. 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 Reaffirms 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 the dystrophin results from Study 045 and the totality of existing clinical and real-world data or, alternatively, data 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 PTC299 clinical trial 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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