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

July 29, 2021

- Total quarterly net revenue of $117 million; 55% increase over second quarter 2020 -
- Raises 2021 DMD franchise revenue guidance to $370-$390M from $355-$375M -
- Progress across robust clinical pipeline with 3 ongoing registration-directed trials -

SOUTH PLAINFIELD, N.J., July 29, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the second quarter ending June 30, 2021.

"I am delighted to say all facets of our business made substantial progress this quarter and we anticipate having four registration-directed trials ongoing by next quarter," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "I am also proud of the execution of the global commercial team, which is driving the continued robust growth of the DMD franchise and has led us to raise our 2021 revenue guidance despite ongoing challenges from the pandemic."

Key Second Quarter and Other Corporate Updates:

- The Duchenne muscular dystrophy (DMD) franchise had a total quarterly net product revenue of $102 million in the second quarter of 2021. This represents a 36% growth over the second quarter of 2020, continuing PTC's trend of strong quarterly commercial revenues.
    - Translarna™ (ataluren) revenue growth was driven by expansion of the patient base, continued high compliance and broader access in existing geographies as well as continued geographic expansion including Russia, Central and Eastern Europe, Middle East and North Africa.
    - Emflaza® (deflazacort) revenue growth was primarily due to new patient starts, continued high adherence and fewer discontinuations.
- Evrysdi™ (risdiplam) received approval from the Ministry of Health Labor and Welfare in Japan in June 2021. Evrysdi is a product of a collaboration between PTC, Roche and the SMA Foundation.
    - Evrysdi is now approved in 54 countries.
    - In the U.S., more than 1800 patients are currently treated with Evrysdi, approaching 20% total market share.

Second Quarter Clinical Updates:

- The registration-directed Phase 3 PTC923 phenylketonuria (PKU) trial, APHENITY, is expected to initiate in the third quarter of 2021.
- PTC has multiple ongoing clinical trials, three of which are registration-directed clinical studies:
    - The MIT-E Phase 2/3 vatiquinone trial for mitochondrial epilepsy with data anticipated in the third quarter of 2022.
    - The MOVE-FA Phase 3 vatiquinone trial for Friedreich ataxia with data anticipated in 2023.
    - The FITE19 Phase 2/3 emvododstat trial in patients with COVID-19 is expected to be completed by year end 2021.
- A Phase 1 healthy volunteer trial of the second Bio-e compound, PTC857, was recently completed and results demonstrated predictable pharmacology and no reported tolerability findings. This allows dose selection necessary to move forward to Phase 2, which we plan to initiate for amyotrophic lateral sclerosis (ALS) in the first quarter of 2022.
- Results from additional cohorts are expected from the Phase 1 healthy volunteer trial of PTC518 for Huntington's disease in the third quarter of this year. Phase 2 planning is already underway and is expected to be initiated by the end of 2021.
- The Committee for Medicinal Products for Human Use (CHMP) imposed a clock stop in the aromatic L-amino acid decarboxylase deficiency (AADC-d) review process to allow for completion of its pre-approval inspections. This process is still ongoing and therefore the CHMP opinion is now expected in the fourth quarter of 2021.
- For the Biologics License Application (BLA) for AADC-d, the third cannula surgery has been completed, and PTC will align with the FDA and expects to submit the BLA by the end of this year.

Second Quarter 2021 Financial Highlights:

- Total revenues were $116.7 million for the second quarter of 2021, compared to total revenues of $75.2 million for the second quarter of 2020.
- Total revenue includes net product revenue across the commercial portfolio of $103.1 million and royalty revenue of $13.6 million for the second quarter of 2021.
- Translarna net product revenues were $52.6 million for the second quarter of 2021, compared to $38.6 million for the

second 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 $49.1 million for the second quarter of 2021, compared to $36.2 million for the second quarter of 2020. These results reflect new patient prescriptions, high compliance, and fewer discontinuations.
- Roche reported first half of 2021 Evrysdi sales of approximately CHF 243 million, resulting in year-to-date royalty revenue of $20.2 million. Evrysdi is a product of a collaboration between PTC, Roche and the SMA Foundation.
- U.S. GAAP (generally accepted accounting principles) research and development (R&D) expenses were $125.5 million for the second quarter of 2021, compared to $176.5 million for the second quarter of 2020. The decrease in research and development expenses is primarily related to one-time charges in the second quarter of 2020 of $53.6 million for our Censa merger, as well as $41.2 million for our commercial manufacturing service agreement with MassBiologics of the University of Massachusetts Medical School, or MassBio, related to dedicated manufacturing space for our lead gene therapy program, AADC deficiency. This was partially offset by increased investment in research programs and advancement of the clinical pipeline in the second quarter of 2021.
- Non-GAAP R&D expenses were $112.0 million for the second quarter of 2021, excluding $13.4 million in non-cash stock-based compensation expense, compared to $168.0 million for the second quarter of 2020, excluding $8.6 million in non-cash stock-based compensation expense.
- GAAP selling, general and administrative (SG&A) expenses were $68.9 million for the second quarter of 2021, compared to $53.7 million for the second quarter of 2020. The increase reflects our continued investment to support commercial activities including expanding our commercial portfolio, including an increase in rent and related expenses associated with entering into a long-term lease for our facility located in Hopewell Township.
- Non-GAAP SG&A expenses were $56.6 million for the second quarter of 2021, excluding $12.3 million in non-cash stock-based compensation expense, compared to $45.3 million for the second quarter of 2020, excluding $8.3 million in non-cash stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $0.7 million for the second quarter of 2021, compared to $7.7 million for the second 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 $118.4 million for the second quarter of 2021, compared to net loss of $181.4 million for the second quarter of 2020.
- Cash, cash equivalents and marketable securities was $947.1 million at June 30, 2021, compared to $1.1 billion at December 31, 2020.
- Shares issued and outstanding as of June 30, 2021 were 70,559,330.

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 $370 and $390 million.
- PTC continues to anticipate GAAP R&D and SG&A expense for the full year 2021 to be between $825 and $855 million.
- PTC continues to anticipate 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 June 30,		Six Months Ended June 30,	
	2021	2020	2021	2020
Revenues:				
Net product revenue	$ 103,113	$ 75,239	$ 194,393	$ 143,435
Collaboration revenue	-	-	20,007	63
Royalty revenue	13,563	-	20,220	-

Total revenues	116,676	75,239	234,620	143,498
Operating expenses:				
Cost of product sales	7,358	5,304	16,462	9,389
Amortization of acquired intangible asset	12,751	8,731	24,028	16,679
Research and development (1)	125,482	176,525	259,995	266,632
Selling, general and administrative (2)	68,878	53,659	129,973	111,869
Change in the fair value of deferred and contingent consideration	700	7,680	800	8,580
Settlement of deferred and contingent consideration	-	10,613	-	10,613
Total operating expenses	215,169	262,512	431,258	423,762
Loss from operations	(98,493)	(187,273)	(196,638)	(280,264)
Interest expense, net	(22,559)	(5,379)	(41,718)	(11,021)
Other income (expense), net	3,170	11,309	(7,716)	(2,523)
Loss before income tax expense	(117,882)	(181,343)	(246,072)	(293,808)
Income tax expense	(488)	(84)	(940)	(306)
Net loss attributable to common stockholders	$ (118,370)	$ (181,427)))	$ (247,012)	$ (294,114)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	70,414,632	65,150,780	70,302,241	63,769,958
Net loss per share—basic and diluted (in dollars per share)	$ (1.68)	$ (2.78)	$ (3.51)	$ (4.61)
(1) Research and development reconciliation				
GAAP research and development	$ 125,482	$ 176,525	$ 259,995	$ 266,632
Less: share-based compensation expense	13,443	8,562	27,168	16,741
Non-GAAP research and development	$ 112,039	$ 167,963	$ 232,827	$ 249,891
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 68,878	$ 53,659	$ 129,973	$ 111,869
Less: share-based compensation expense	12,256	8,348	24,238	15,389
Non-GAAP selling, general and administrative	$ 56,622	$ 45,311	$ 105,735	$ 96,480

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

	June 30, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 947,081	$ 1,103,650
Total Assets	**$ 2,058,669**	**$ 2,208,278**
Total debt	$ 430,496	$ 309,145
Total liability for sale of future royalties	709,164	679,762
Total deferred revenue	-	4,151
Total liabilities	**$ 1,862,092**	**$ 1,726,296**
Total stockholders' equity (70,559,330 and 69,718,096 common shares issued and outstanding at June 30, 2021 and December 31, 2020 respectively)	$ 196,577	$ 481,982
Total liabilities and stockholders' equity	**$ 2,058,669**	**$ 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 second 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 the dystrophin results from Study 045 and the totality of existing clinical and real-world data or 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 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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