PTC Therapeutics Reports Fourth Quarter and Full Year 2020 Financial Results and Provides a Corporate Update

February 25, 2021

- $333 million 2020 total net product revenue representing 14% year-over-year growth -
- Advancing multiple registration-directed clinical trials -
- PTC518 healthy volunteer data expected in the first half of 2021 for Huntington disease program -
- 2021 DMD franchise revenue guidance reaffirmed at $355-375 million -
- Strong U.S. launch for Evrysdi™ with approvals in Europe and Japan expected in 2021 -

SOUTH PLAINFIELD, N.J., Feb. 25, 2021 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced financial results for the fourth quarter and full year ending December 31, 2020 and provided a corporate update.

"Despite the challenges of the pandemic, PTC has been able to make significant progress in moving our pipeline forward and has been able to continue to bring therapies to our patients throughout 2020," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "I am very proud of our team and their abilities to execute on our 2020 goals even in such turbulent times. I am confident that we will continue to progress on our 2021 goals that will create value for all of our stakeholders."

Key 2020 Corporate Highlights:

- Strong continued revenue growth for the DMD franchise, with total net product revenue of $331 million for Translarna™ (ataluren) and Emflaza® (deflazacort) in 2020.
  - Translarna growth was driven by broader uptake due to new patients in existing geographies, geographic expansion and label updates.
  - Emflaza experienced strong 38% year-over-year revenue growth in 2020 due to increased new prescriptions and high compliance.
- Evrysdi ™(risdiplam) was approved by the FDA in August 2020 for treatment of spinal muscular atrophy (SMA) patients aged 2 months and older. A strong global launch is under way, leading to increased patient uptake across all disease subtypes. Evrysdi is a product of a collaboration between PTC, Roche, and the SMA Foundation.
- PTC initiated five clinical trials in 2020, 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.
  - A healthy volunteer study for PTC857, the second Bio-e compound.
  - A healthy volunteer study for PTC518 for the Huntington disease program from the splicing platform.
- Submitted a Marketing Authorization Application (MAA) to European Medicines Agency (EMA) for PTC-AADC, a gene therapy for aromatic L-amino acid decarboxylase (AADC) deficiency.
- PTC strengthened its balance sheet with a $650 million upfront payment for a portion of the Evrysdi royalties.

2021 Potential Value-Creating Milestones:

- Results from Phase 1 healthy volunteer study of PTC518 for Huntington disease program from the splicing platform are expected in the first half.
- Data from the healthy volunteer study for PTC857 are expected in the first half.
- PTC expects an opinion on the PTC-AADC MAA from the EMA's Committee for Medicinal Products for Human Use (CHMP) in the second quarter.
- PTC is on track for a submission of a Biologics License Application (BLA) to the FDA in the second quarter for PTC-AADC.
- PTC anticipates an opinion from the CHMP for Evrysdi in the first quarter.
- A registration-directed study, APHENITY, for PTC923 in patients with phenylketonuria (PKU) is expected to initiate mid-year.
- The second stage of the FITE19 Phase 2/3 clinical trial to assess PTC299 in patients with COVID-19 has been initiated. Data from this study is expected in the second half of the year.
- PTC expects to dose the first patient with PTC-FA gene therapy for Friedreich ataxia before year end.
- Results from the ongoing clinical trials evaluating PTC596 in Leiomyosarcoma (LMS) and Diffuse Intrinsic Pontine Glioma

(DIPG) are expected by year end.

Fourth Quarter and Full Year 2020 Financial Highlights:

- Total revenues were $118.9 million for the fourth quarter of 2020, compared to $96.5 million for the fourth quarter of 2019. Total revenues were $380.8 million for the full year 2020, compared to $307.0 million for the full year 2019.
- Total revenue includes net product revenue across the commercial portfolio of $107.3 million for the fourth quarter of 2020 and $333.4 million for full year 2020, and collaboration and royalty revenue of $11.6 million for the fourth quarter of 2020 and $47.4 million for full year 2020.
- Translarna net product revenues were $69.4 million for the fourth quarter of 2020, compared to $48.4 million for the fourth quarter of 2019. Translarna net product revenues were $191.9 million for the full year 2020, compared to $190.0 million for the full year 2019. In October 2020, PTC secured a purchase agreement for Translarna with the Ministry of Health in Brazil specifying two shipments, both of which were fulfilled in 2020.
- Emflaza net product revenues were $36.8 million for the fourth quarter of 2020, compared to $32.7 million for the fourth quarter of 2019. Emflaza net product revenues were $139.0 million for the full year 2020, compared to $101.0 million for the full year 2019.
- Roche reported Evrysdi 2020 year-to-date sales of approximately CHF 55 million. During the third quarter of 2020, 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. Additionally, 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. The filing in Japan triggered a $7.5 million milestone payment to PTC in the fourth quarter of 2020. As a result, PTC recognized $35.0 million in the third quarter of 2020 and $7.5 million in the fourth quarter of 2020 of collaboration revenue associated with Roche milestone events.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $118 million for the fourth quarter of 2020, compared to $81.8 million for the fourth quarter of 2019. GAAP R&D expenses were $477.6 million for the full year 2020, compared to $257.4 million for the full year 2019. The increase in R&D expenses for the fourth quarter and full year 2020 reflects costs associated with advancing the gene therapy and Bio-e platforms, increased investment in research programs, and advancement of the clinical pipeline. Additionally, the increase in R&D expenses full year 2020 includes one-time charges in 2020 of $53.6 million related to the acquisition of Censa Pharmaceuticals, and $41.4 million related to the MassBiologics of the University of Massachusetts Medical School agreement for commercial manufacturing of our lead gene therapy program in AADC deficiency.
- Non-GAAP R&D expenses were $105.3 million for the fourth quarter of 2020, excluding $12.7 million in non-cash, stock-based compensation expense, compared to $76.2 million for the fourth quarter of 2019, excluding $5.6 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $438.9 million for the full year 2020, excluding $38.7 million in non-cash, stock-based compensation expense, compared to $236.6 million for the full year 2019, excluding $20.8 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $75.5 million for the fourth quarter of 2020, compared to $63.5 million for the fourth quarter of 2019. GAAP SG&A expenses were $245.2 million for the full year 2020, compared to $202.5 million for the full year 2019. The increase reflects continued investment to support PTC's commercial activities including the expanding commercial portfolio, and rent and related expenses associated with entering into a long-term lease for the Hopewell facility that commenced on July 1, 2020.
- Non-GAAP SG&A expenses were $66.8 million for the fourth quarter of 2020, excluding $8.7 million in non-cash, stock-based compensation expense, compared to $57.7 million for the fourth quarter of 2019, excluding $5.8 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $213.6 million for the full year 2020, excluding $31.6 million in non-cash, stock-based compensation expense, compared to $181.2 million for the full year 2019, excluding $21.3 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $6.3 million for the fourth quarter of 2020, compared to $12.4 million for the fourth quarter of 2019. Change in the fair value of deferred and contingent consideration was $23.3 million for the full year 2020, compared to $48.4 million for the full year 2019. The change is related to the fair valuation of the potential future consideration to be paid to former equity holders of Agilis Biotherapeutics, Inc. (Agilis), as a result of our merger with Agilis which closed in August 2018. Changes in the fair value were due to the re-calculation of discounted cash flows for the passage of time and changes to certain other estimated assumptions.
- Settlement of deferred and contingent consideration was $10.6 million for the full year 2020. The settlement of deferred and contingent consideration is related to a loss upon the settlement of the deferred and contingent consideration liabilities as a result of the rights exchange agreement with certain former shareholders of Agilis, whereby such former shareholders exchanged their pro rata share of specific future cash milestone payments in the aggregate amount of $225 million for a mixture of cash and equity of PTC. Under this agreement, which the former shareholders and PTC entered into on April 29, 2020, PTC has paid $36.9 million in cash and issued 2,821,176 shares of common stock in exchange for the cancellation and forfeiture of the participating shareholders' rights to receive (i) $174.0 million, in the aggregate, of potential milestone payments based on the achievement of certain regulatory milestones and (ii) $37.6 million, in the aggregate, of

$40.0 million in development milestone payments that would have been due upon the passing of the second anniversary of the closing of PTC's merger with Agilis, regardless of whether the milestones are achieved.

- Net loss was $74.4 million for the fourth quarter of 2020, compared to net loss of $77.7 million for the fourth quarter of 2019. Net loss was $438.2 million for the full year 2020, compared to net loss of $251.6 million for the full year 2019.
- Cash, cash equivalents, and marketable securities was $1.1 billion at December 31, 2020, compared to $686.6 million at December 31, 2019.
- Shares issued and outstanding as of December 31, 2020 were 69,718,096.

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 December 31,		Twelve Months Ended December 31,	
	2020	2019	2020	2019
Revenues:				
Net product revenue	$ 107,258	$ 81,407	$ 333,401	$ 291,306
Collaboration revenue	7,516	15,052	42,579	15,674
Royalty Revenue	4,090	-	4,786	-
Total revenues	118,864	96,459	380,766	306,980
Operating expenses:				
Cost of product sales	4,886	3,542	18,942	12,135
Amortization of acquired intangible asset	10,583	7,973	36,892	27,650
Research and development (1)	118,013	81,831	477,643	257,452
Selling, general and administrative (2)	75,456	63,497	245,164	202,541
Change in the fair value of deferred and contingent consideration	6,300	12,400	23,280	48,360
Settlement of deferred and contingent consideration	-	-	10,613	-
Total operating expenses	215,238	169,243	812,534	548,138
Loss from operations	(96,374)	(72,784)	(431,768)	(241,158)
Interest expense, net	(24,292)	(5,463)	(56,352)	(12,491)
Other income (expense), net	58,946	11,214	85,188	13,723
Loss before income tax expense	(61,720)	(67,033)	(402,932)	(239,926)
Income tax expense	(12,634)	(10,644)	(35,228)	(11,650)
Net loss attributable to common stockholders	$ (74,354)	$ (77,677)	$ (438,160)	$ (251,576)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	68,886,219	56,570,002	66,027,908	58,863,185
Net loss per share—basic and diluted (in dollars per share)	$ (1.08)	$ (1.37)	$ (6.64)	$ (4.27)
(1) Research and development reconciliation				
GAAP research and development	$ 118,013	$ 81,831	$ 477,643	$ 257,452

Less: share-based compensation expense	12,755	5,645	38,716	20,836
Non-GAAP research and development	$ 105,258	$ 76,186	$ 438,927	$ 236,616
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 75,456	$ 63,497	$ 245,164	$ 202,541
Less: share-based compensation expense	8,661	5,821	31,609	21,298
Non-GAAP selling, general and administrative	$ 66,795	$ 57,676	$ 213,555	$ 181,243

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

	December 31, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 1,103,650	$ 686,563
Total Assets	**$ 2,208,278**	**$ 1,623,782**
Total debt	$ 309,145	$ 313,859
Total deferred revenue	4,151	11,657
Total liability for sale of future royalties	679,762	-
Total liabilities	**$ 1,726,296**	**$ 1,029,452**
Total stockholders' equity (69,718,096 and 61,935,870 common shares issued and outstanding at December 31, 2020 and December 31, 2019 respectively)	$ 481,982	$ 594,330
Total liabilities and stockholders' equity	**$ 2,208,278**	**$ 1,623,782**

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:
Today's conference call will take place at 4:30 pm ET and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 2174406. 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 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 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 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 any 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; 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 fo 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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