PTC Therapeutics Provides a Corporate Update and Reports Fourth Quarter and Full Year 2021 Financial Results

February 22, 2022

- $539 million 2021 total revenue representing impressive 41% year-over-year growth -
- Continued pipeline progress with five registration-directed clinical trials ongoing -
- $700-750 million 2022 total revenue guidance reaffirmed -

SOUTH PLAINFIELD, N.J., Feb. 22, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the fourth quarter and full year ending December 31, 2021.

"It is gratifying to see that after two years of investment in the innovation revenue cycle we see substantial progress across the robust pipeline and commercial revenues exceeding half a billion dollars," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "2022 is expected to be a transformational year for PTC and I look forward to continued growth."

Key 2021 Corporate Updates:

- Strong revenue growth continued in the Duchenne muscular dystrophy (DMD) franchise, with total net product revenue of $423 million for Translarna™ (ataluren) and Emflaza® (deflazacort) in 2021.
  - Translarna total net product revenue of $236 million was due to treatment of new patients, continued high compliance, and geographic expansion.
  - Emflaza total net product revenue of $187 million was driven by continued new prescriptions, continued high compliance, and more favorable access.
- Evrysdi® (risdiplam) 2021 revenue of over $500 million resulted in a $25 million sales-based milestone to PTC. Evrysdi is now the most prescribed Spinal Muscular Atrophy (SMA) therapy in the U.S. and growth continues in markets outside of the U.S. Evrysdi is a product of the SMA collaboration between PTC, the SMA Foundation and Roche.
- Waylivra® (volanesorsen) and Tegsedi® (inotersen**)** both received Category 1 classification from Câmara de Regulação do Mercado de Medicamentos - CMED (Drug Market Regulation Chamber) in Brazil. This allows for pricing in line with international markets.
- PTC submitted an application to the Brazilian Health Regulatory Agency, Agência Nacional de Vigilância Sanitária (ANVISA), for approval of Waylivra for the treatment of familial partial lipodystrophy (FPL). If approved, Waylivra would be the first treatment in the world approved for FPL.
- PTC successfully advanced the clinical pipeline in 2021:
  - Phase 1 healthy volunteer trial of PTC518, the next compound from the validated splicing platform, which is being developed for Huntington's disease (HD).
  - Phase 1 healthy volunteer trial of PTC857, the second compound from the Bio-e platform, which is being developed for amyotrophic lateral sclerosis (ALS).
  - Phase 1b studies of unesbulin in both leiomyosarcoma and diffuse intrinsic pontine glioma (DIPG), two rare oncology indications.
- Enrollment is complete in the MOVE-FA registration-directed trial of vatiquinone in Friedreich ataxia, with results expected in the second quarter of 2023.

2022 Potential Value-Creating Milestones:

- Results are expected mid-year for Translarna Study 041.
- Results are expected from the Phase 3 APHENITY trial of PTC923 in phenylketonuria (PKU) by the end of 2022.
- Results from the Phase 2/3 MIT-E study of vatiquinone in mitochondrial disease associated seizures are expected in the fourth quarter of 2022.
- An opinion on our marketing authorization application (MAA) for PTC-AADC is expected in April 2022 from the European Medicine Agency's Committee for Medicinal Products for Human Use (CHMP).
- PTC remains on track to submit the biologics license application for PTC-AADC to the Food and Drug Administration in the second quarter of 2022.
- PIVOT-HD, the Phase 2 study of PTC518 in HD is expected to initiate in the first quarter of 2022.
- The CardinALS registration-directed Phase 2 study of PTC857 in ALS is expected to initiate in the second quarter of 2022.

Fourth Quarter and Full-Year 2021 Financial Highlights:

- Total revenues were $165.2 million for the fourth quarter of 2021, compared to $118.9 million for the fourth quarter of 2020. Total revenues were $538.6 million for the full year 2021, compared to $380.8 million for the full year 2020.
- Total revenue includes net product revenue across the commercial portfolio of $118.9 million for the fourth quarter of 2021 and $428.9 million for full year 2021, compared to $107.3 million for the fourth quarter of 2020 and $333.4 million for full year 2020. Total revenue also includes collaboration and royalty revenue of $46.3 million in the fourth quarter of 2021 and $109.7 million for the full year 2021, compared to $11.6 million for the fourth quarter of 2020 and $47.4 million for the full year 2020.
- Translarna net product revenues were $69.7 million for the fourth quarter of 2021, compared to $69.4 million for the fourth quarter of 2020. Translarna net product revenues were $236.0 million for the full year 2021, compared to $191.9 million for the full year 2020. These results were driven by treatment of new patients, continued high compliance, and geographic expansion.
- Emflaza net product revenues were $47.5 million for the fourth quarter of 2021, compared to $36.8 million for the fourth quarter of 2020. Emflaza net product revenues were $187.3 million for the full year 2021, compared to $139.0 million for the full year 2020. These results were driven by continued new prescriptions, continued high compliance, and more favorable access.
- Roche reported Evrysdi full year 2021 sales of approximately CHF 602 million, resulting in full year 2021 royalty revenue of $54.6 million to PTC, as compared to $4.8 million for the full year 2020. In the first quarter of 2021 the first commercial sale of Evrysdi in the European Union triggered a $20 million milestone payment to PTC, in the third quarter of 2021 the first commercial sale of Evrysdi in Japan triggered a $10 million milestone payment from Roche to PTC, and in the fourth quarter of 2021 PTC recorded its first sales milestone of $25 million for the achievement of $500 million in worldwide annual net sales from Evrysdi. All of these 2021 achievements were reported as collaboration revenue. For the full the year 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. PTC recognized $25 million of collaboration revenue associated with Roche milestone events in the fourth quarter of 2021 and $55 million for the full year 2021, as compared to $7.5 million in the fourth quarter of 2020 and $42.6 million for the full year 2020.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $149.8 million for the fourth quarter of 2021, compared to $118 million for the fourth quarter of 2020. GAAP R&D expenses were $540.7 million for the full year 2021, compared to $477.6 million for the full year 2020. The increase reflects additional investment in research programs and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $136.4 million for the fourth quarter of 2021, excluding $13.4 million in non-cash, stock-based compensation expense, compared to $105.3 million for the fourth quarter of 2020, excluding $12.7 million in non-cash, stock-based compensation expense. Non-GAAP R&D expenses were $487.1 million for the full year 2021, excluding $53.6 million in non-cash, stock-based compensation expense, compared to $438.9 million for the full year 2020, excluding $38.7 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $86.5 million for the fourth quarter of 2021, compared to $75.5 million for the fourth quarter of 2020. GAAP SG&A expenses were $285.8 million for the full year 2021, compared to $245.2 million for the full year 2020. The increase reflects our continued investment to support commercial activities including expanding our commercial portfolio.
- Non-GAAP SG&A expenses were $73.7 million for the fourth quarter of 2021, excluding $12.8 million in non-cash, stock-based compensation expense, compared to $66.8 million for the fourth quarter of 2020, excluding $8.7 million in non-cash, stock-based compensation expense. Non-GAAP SG&A expenses were $235.9 million for the full year 2021, excluding $49.9 million in non-cash, stock-based compensation expense, compared to $213.6 million for the full year 2020, excluding $31.6 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was a gain of $12.1 million for the fourth quarter of 2021, compared to a loss of $6.3 million for the fourth quarter of 2020. Change in the fair value of deferred and contingent consideration was a gain of $0.5 million for the full year 2021, compared to a loss of $23.3 million for the full year 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.
- 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 $143.3 million for the fourth quarter of 2021, compared to net loss of $74.4 million for the fourth quarter of 2020. Net loss was $523.9 million for the full year 2021, compared to net loss of $438.2 million for the full year 2020.
- Cash, cash equivalents, and marketable securities were $773.4 million on December 31, 2021, compared to $1.1 billion at December 31, 2020.
- Shares issued and outstanding as of December 31, 2021 were 70,828,226.

PTC Reaffirms 2022 Financial Guidance:

- PTC anticipates total revenues for the full year 2022 to be between $700 and $750 million.
- PTC anticipates net product revenues for the DMD franchise for the full year 2022 to be between $475 and $495 million.
- PTC anticipates GAAP R&D and SG&A expense for the full year 2022 to be between $915 and $965 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2022 to be between $800 and $850 million, excluding estimated non-cash, stock-based compensation expense of $115 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 December 31,		Twelve Months Ended December 31,	
	2021	2020	2021	2020
Revenues:				
Net product revenue	$ 118,905	$ 107,258	$ 428,904	$ 333,401
Collaboration revenue	25,029	7,516	55,046	42,579
Royalty revenue	21,294	4,090	54,643	4,786
Total revenues	165,228	118,864	538,593	380,766
Operating expenses:				
Cost of product sales	9,327	4,886	32,328	18,942
Amortization of acquired intangible asset	16,340	10,583	54,751	36,892
Research and development (1)	149,844	118,013	540,684	477,643
Selling, general and administrative (2)	86,548	75,456	285,773	245,164
Change in the fair value of deferred and contingent consideration	(12,100)	6,300	(500)	23,280
Settlement of deferred and contingent consideration	-	-	-	10,613
Total operating expenses	249,959	215,238	913,036	812,534
Loss from operations	(84,731)	(96,374)	(374,443)	(431,768)
Interest expense, net	(22,502)	(24,292)	(86,022)	(56,352)
Other (expense) income, net	(31,375)	58,946	(57,875)	85,188
Loss before income tax expense	(138,608)	(61,720)	(518,340)	(402,932)
Income tax expense	(4,657)	(12,634)	(5,561)	(35,228)
Net loss attributable to common stockholders	$ (143,265)	$ (74,354)	$ (523,901)	$ (438,160)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	70,669,797	68,886,219	70,466,393	66,027,908
Net loss per share—basic and diluted (in dollars per share)	$ (2.03)	$ (1.08)	$ (7.43)	$ (6.64)
(1) Research and development reconciliation				
GAAP research and development	$ 149,844	$ 118,013	$ 540,684	$ 477,643
Less: share-based compensation expense	13,416	12,755	53,632	38,716
Non-GAAP research and development	$ 136,428	$ 105,258	$ 487,052	$ 438,927
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 86,548	$ 75,456	$ 285,773	$ 245,164
Less: share-based compensation expense	12,819	8,661	49,881	31,609
Non-GAAP selling, general and administrative	$ 73,729	$ 66,795	$ 235,892	$ 213,555

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

	December 31, 2021	December 31, 2020
Cash, cash equivalents and marketable securities	$ 773,376	$ 1,103,650
Total Assets	**$ 1,938,056**	**$ 2,208,278**
Total debt	$ 431,434	$ 309,145
Total deferred revenue	-	4,151
Total liability for sale of future royalties	733,985	679,762
Total liabilities	**$ 1,936,618**	**$ 1,726,296**
Total stockholders' equity (70,828,226 and 69,718,096 common shares issued and outstanding at December 31, 2021 and December 31, 2020 respectively)	$ 1,438	$ 481,982
Total liabilities and stockholders' equity	**$ 1,938,056**	**$ 2,208,278**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2022 R&D and SG&A Expense
(In thousands)

	Low End of Range	High End of Range
Projected GAAP R&D and SG&A Expense	$ 915,000	$ 965,000
Less: projected non-cash, stock-based compensation expense	115,000	115,000
Projected non-GAAP R&D and SG&A expense	$ 800,000	$ 850,000

Today's Conference Call and Webcast Reminder:

PTC will host a conference call to discuss the fourth quarter and full year 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 6884452. 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 2022 Financial Guidance", including with respect to (i) 2022 total revenue guidance, (ii) 2022 net product revenue guidance for the DMD franchise and (iii) 2022 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; PTC's ability to utilize results from 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, to support a marketing approval for Translarna for the treatment of nmDMD in the United States; expectations with respect to the commercialization of Evrysdi under our SMA collaboration; PTC's ability to maintain its marketing authorization of Translarna for the treatment of nmDMD in Brazil, Russia, the European Economic Area (EEA) and other regions, 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 complete Study 041, 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 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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