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

October 27, 2022

- Total quarterly revenue of $217 million, representing 57% year-over-year growth -
- 2022 DMD revenue guidance and low end of total revenue guidance increased -
- Strategic financing with Blackstone with up to $1 billion of low-cost, low-dilution capital -
- Pipeline of multiple innovative compounds continues to advance in clinical development -

SOUTH PLAINFIELD, N.J., Oct. 27, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the third quarter ending September 30, 2022.

"Our mission is to build an enduring biopharmaceutical company that treats diseases with significant unmet medical needs," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "We continue to achieve our ambitious goals for 2022 of generating strong revenue growth while advancing our broad and deep pipeline to continue to fulfill this vision."

Key Corporate Updates:

- The Duchenne muscular dystrophy (DMD) franchise continued to show strong growth, with third quarter total net product revenue of $131 million, or 15% year-over-year growth.
  - Translarna™ (ataluren) total net product revenue was $77 million, with growth coming from new patients in existing geographies and continued geographic expansion
  - Emflaza® (deflazacort) total net product revenue was $55 million, driven by new patient starts, broader access, continued high compliance and appropriate weight-based dosing
- Evrysdi milestone of $50 million from Roche achieved for surpassing annual net sales of $750 million.
- Strategic financing with Blackstone to grow pipeline. As part of the partnership, Blackstone provides PTC with an initial $350 million in capital at close, with an option for an additional $650 million in funding.

Key Clinical and Regulatory Updates:

- PTC submitted a type II variation to EMA to convert the conditional marketing authorization for Translarna to a standard marketing authorization. PTC expects a CHMP opinion in the first half of 2023.
- PTC submitted a meeting request to the FDA to gain clarity on the regulatory pathway for the NDA for Translarna in the US. While the FDA has provided initial written feedback that Study 041 does not provide substantial evidence of effectiveness, PTC is planning follow up discussions with the agency to understand whether the evidence in the ITT population in Study 041 along with confirmatory evidence from other studies could support approval.
- PTC held a type C meeting with the FDA to discuss the details of a potential submission package for Upstaza. The FDA asked for additional bioanalytical data in support of comparability between the drug product used in the clinical studies and the commercial drug product. PTC is currently working with the FDA to address this request and expects to submit a BLA for Upstaza in the first half of 2023.
- PTC continues to make progress in additional ongoing registration-directed clinical studies:
  - The APHENITY Phase 3 trial of sepiapterin (PTC923) for PKU, with results anticipated by the end of the fourth quarter
  - The MIT-E Phase 2/3 vatiquinone trial for mitochondrial disease associated seizures, with results anticipated in the first quarter of 2023
  - The MOVE-FA Phase 3 vatiquinone trial for Friedreich ataxia, with results anticipated in the second quarter of 2023
- Enrollment in the PIVOT-HD Phase 2 trial of PTC518 for Huntington's disease is active and ongoing at study sites in Europe and Australia. Enrollment in the US is paused as the FDA has requested additional data to support trial conduct. Data from the first 12 weeks of the placebo-controlled trial anticipated in the first half of 2023.

Third Quarter 2022 Financial Highlights:

- Total revenues were $217.1 million for the third quarter of 2022, compared to $138.7 million for the third quarter of 2021.
- Total revenue includes net product revenue across the commercial portfolio of $134.2 million for the third quarter of 2022, compared to $115.6 million for the third quarter of 2021. Total revenue also includes royalty and collaboration revenue of $82.9 million in the third quarter of 2022, compared to $23.1 million for the third quarter of 2021.
- Translarna net product revenues were $76.6 million for the third quarter of 2022, compared to $67.2 million for the third quarter of 2021. These results reflect an increase in net product sales from new patients in existing markets as well as continued geographic expansion.
- Emflaza net product revenues were $54.8 million for the third quarter of 2022, compared to $47.1 million for the third quarter of 2021. These results reflect new patient starts, broader access, continued high compliance and appropriate weight-based dosing.
- Roche reported Evrysdi 2022 year to date sales of approximately CHF 793 million, resulting in royalty revenue of $32.9 million to PTC in the third quarter of 2022, as compared to $13.1 million for the third quarter of 2021. Also in the third quarter of 2022, PTC recorded a sales milestone of $50 million for the achievement of $750 million in worldwide annual net sales from Evrysdi. This sales milestone was recorded as collaboration revenue.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $165.5 million for the third quarter of 2022, compared to $130.8 million for the third quarter of 2021. The increase reflects additional investment in research programs and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $150.4 million for the third quarter of 2022, excluding $15.1 million in non-cash, stock-based compensation expense, compared to $117.8 million for the third quarter of 2021, excluding $13.0 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $80.1 million for the third quarter of 2022, compared to $69.3 million for the third quarter of 2021. The increase reflects our continued investment to support commercial activities, including expanding our commercial portfolio.
- Non-GAAP SG&A expenses were $66.5 million for the third quarter of 2022, excluding $13.6 million in non-cash, stock-based compensation expense, compared to $56.4 million for the third quarter of 2021, excluding $12.8 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $5.3 million for the third quarter of 2022, compared to $10.8 million for the third quarter of 2021. 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 $109.3 million for the third quarter of 2022, compared to net loss of $133.6 million for the third quarter of 2021.
- Cash, cash equivalents, and marketable securities was $288.4 million as of September 30, 2022, compared to $773.4 million as of December 31, 2021.
- Shares issued and outstanding as of September 30, 2022, were 71,854,892.

PTC Updates Full Year 2022 Financial Guidance as Follows:

- PTC anticipates total revenues for the full year 2022 to be between $710 and $750 million, compared to previous guidance of between $700 and $750 million.
- PTC anticipates net product revenues for the DMD franchise for the full year 2022 to be between $490 and $500 million, compared to previous guidance of between $475 and $495 million.
- PTC anticipates GAAP R&D and SG&A expenses for the full year 2022 to be between $925 and $965 million, compared to previous guidance of between $915 and $965 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for the full year 2022 to be between $810 and $850 million, compared to previous guidance of between $800 and $850 million, both excluding estimated non-cash, stock-based compensation expense of $115 million.

Non-GAAP Financial Measures:

In this press release, the financial results 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.

Consolidated Statements of Operations
(In thousands, except share and per share data)

	Three Months Ended September 30,		Nine Months Ended September 30,	
	2022	2021	2022	2021
Revenues:				
Net product revenue	$ 134,186	$ 115,605	$ 407,720	$ 309,998
Collaboration revenue	50,017	10,011	50,024	30,018
Royalty revenue	32,924	13,127	73,645	33,348
Total revenues	217,127	138,743	531,389	373,364
Operating expenses:				
Cost of product sales	14,011	6,539	33,785	23,001
Amortization of acquired intangible asset, excluding amortization of acquired intangible assets	31,023	14,383	80,790	38,411
Research and development (1)	165,462	130,845	462,802	390,840
Selling, general and administrative (2)	80,118	69,252	233,280	199,225
Change in the fair value of deferred and contingent consideration	(5,300)	10,800	(32,200)	11,600
Total operating expenses	285,314	231,819	778,457	663,077
Loss from operations	(68,187)	(93,076)	(247,068)	(289,713)
Interest expense, net	(20,880)	(21,802)	(66,371)	(63,520)
Other expense, net	(38,141)	(18,782)	(84,355)	(26,499)
Loss before income tax expense	(127,208)	(133,660)	(397,794)	(379,732)
Income tax benefit (expense)	17,893	36	9,666	(904)
Net loss attributable to common stockholders	$ (109,315)	$ (133,624)	$ (388,128)	$ (380,636)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	71,654,671	70,585,938	71,415,849	70,397,846
Net loss per share—basic and diluted (in dollars per share)	$ (1.53)	$ (1.89)	$ (5.43)	$ (5.41)
(1) Research and development reconciliation				
GAAP research and development	$ 165,462	$ 130,845	$ 462,802	$ 390,840
Less: share-based compensation expense	15,063	13,048	41,896	40,216
Non-GAAP research and development	$ 150,399	$ 117,797	$ 420,906	$ 350,624
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 80,118	$ 69,252	$ 233,280	$ 199,225
Less: share-based compensation expense	13,607	12,823	41,093	37,061
Non-GAAP selling, general and administrative	$ 66,511	$ 56,429	$ 192,187	$ 162,164

PTC Therapeutics, Inc.
Summary Consolidated Balance Sheets

(in thousands, except share data)

	September 30, 2022	December 31, 2021
Cash, cash equivalents and marketable securities	$ 288,432	$ 773,376
Total Assets	**$ 1,576,398**	**$ 1,938,056**
Total debt	$ 282,749	$ 431,434
Total liability for sale of future royalties	758,146	733,985
Total liabilities	**$ 1,803,302**	$ **1,936,618**
Total stockholders' (deficit) equity (71,854,892 and 70,828,226 common shares issued and outstanding at September 30, 2022 and December 31, 2021, respectively)	$ (226,904)	$ 1,438
Total liabilities and stockholders' (deficit) equity	**$ 1,576,398**	**$ 1,938,056**

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	$ 925,000	$ 965,000
Less: projected non-cash, stock-based compensation expense	115,000	115,000
Projected non-GAAP R&D and SG&A expense	$ 810,000	$ 850,000

Acronyms:
AADC: Aromatic L-amino acid decarboxylase
ALS: Amyotrophic Lateral Sclerosis
DMD: Duchenne Muscular Dystrophy
FDA: U.S. Food and Drug Administration
PKU: Phenylketonuria
R&D: Research and Development
SG&A: Selling, General and Administrative
SMA: Spinal Muscular Atrophy

Today's Conference Call and Webcast Reminder:

To access the call by phone, please click here to register and you will be provided with dial-in details. To avoid delays, we recommend participants dial in to the conference call 15 minutes prior to the start of the call. The webcast conference call can be accessed on the Investor section of the PTC website at https://ir.ptcbio.com/events-presentations. A 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 2022 Financial Guidance as Follows", 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions, commercialization 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 Upstaza and other programs within PTC's gene therapy platform, including any regulatory submissions and potential approvals, commercialization, 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; 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; 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 and a conversion to a standard marketing authorization in the EEA; expectations with respect to the commercialization of Tegsedi and Waylivra; the 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 its lease agreements, including for its leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of the secured credit facility with Blackstone; 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, Upstaza, Evrysdi, Tegsedi or Waylivra.

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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