PTC Therapeutics Provides Corporate Update and Reports Third Quarter Financial Results

October 26, 2023

– Third quarter 2023 total revenues of $197 million and remain on target for 2023 revenue guidance –

– PTC strengthened its financial position following recent restructuring initiatives and Royalty Pharma transaction –

– PTC provides regulatory updates for pipeline programs –

SOUTH PLAINFIELD, N.J., Oct. 26, 2023 /PRNewswire/ -- PTC Therapeutics, Inc., (NASDAQ: PTCT) today announced a corporate update and financial results for the third quarter ending September 30, 2023.

"I am very proud of the progress all of our teams made this quarter," said Matthew Klein, M.D., Chief Executive Officer, PTC Therapeutics, Inc. "The recent Royalty Pharma transaction and restructuring initiatives that we implemented have put the company on a strong financial footing. We believe we are well positioned to deliver on our most promising opportunities for growth, including the potential sepiapterin revenue opportunity of more than $1 billion and the PTC518 HD program."

Key Corporate Updates:

- Finalized strategic partnership transaction with Royalty Pharma, in which Royalty Pharma acquired additional royalties of Evrysdi for $1.0 billion upfront. The agreement included options for PTC to sell the remainder of its royalties of Evrysdi for up to $500 million or for Royalty Pharma to acquire half of such retained royalties for up to $250 million at a later date, less royalties received by PTC. PTC maintains all economics associated with up to $250 million in the remaining commercial sales milestones associated with Evrysdi global net sales.
- Third quarter 2023 revenue for the DMD franchise was $136 million, supporting increasing 2023 DMD franchise revenue guidance to between $565 million and $595 million.
    - Translarna™ (ataluren) quarterly net product revenue was $69 million, with new patients in existing geographies and continued geographic expansion.
    - Emflaza® (deflazacort) quarterly net product revenue was $67 million, with new patient starts and high compliance.

Key Clinical and Regulatory Updates:

- For Translarna, following the negative opinion from the CHMP, the CHMP gave PTC the option to request re-examination of both opinions or only one opinion. PTC decided to pursue re-examination of the negative opinion on renewal of the conditional authorization only. In accordance with EMA guidelines, PTC expects the opinion from the re-examination procedure in late January 2024, with ratification of that opinion by the European Commission 67 days later.
- For the United States, a type C meeting with the FDA for Translarna is scheduled for the fourth quarter of 2023.
- PTC held a pre-NDA meeting in the third quarter with the FDA for sepiapterin in PKU to discuss the NDA submission. At the meeting, the FDA stated that the sepiapterin clinical safety and efficacy data supported NDA submission for the treatment of pediatric and adult PKU patients. It was requested that PTC complete an additional 26-week nonclinical mouse study to assess sepiapterin carcinogenicity potential prior to NDA submission. This nonclinical study was not initially required when sepiapterin was acquired from Censa, as the NDA submission was planned under the Section 505(b)(2) pathway. With PTC's decision to file under the Section 505(b)(1) pathway, the 26-week study is considered a required NDA component needed to inform labeling and is typically completed prior to submission. PTC will continue to discuss with the FDA the potential to submit the mouse study results during the NDA review process. PTC now expects the NDA submission to occur no later than the third quarter of 2024; the submission could be submitted during the second quarter of 2024 if the nonclinical study report can be submitted during the review process.
- PTC expects to submit an MAA to the EMA for sepiapterin for the treatment of PKU in the first half of 2024.
- Enrollment in the PIVOT-HD study for PTC518 for Huntington's disease continues outside of the US for both the stage 2 and early stage 3 cohorts. PTC expects the next data update to occur in the first half of 2024. This update will include 12-month data on the initial group of subjects for which data was reported in June of this year.
- PTC had a type A meeting with the FDA to discuss the clinical safety data needed to enable enrollment of the PIVOT-HD trial at US study sites. At the meeting, the FDA stated that the existing three months of safety data could support 12-week dosing at 5mg and 10mg dose levels and that six months of clinical safety data demonstrating a similar favorable safety profile could support 12-month dosing in PIVOT-HD.
- PTC had a type C written-response-only meeting with FDA for vatiquinone for Friedreich ataxia to determine whether the data from the MOVE-FA study would be sufficient to support an NDA for accelerated approval. In their written response, the FDA stated that while they see the value of upright stability as a clinically meaningful endpoint, they believed a

confirmatory study would likely be needed to support NDA submission. PTC has requested a follow-up live meeting to address the issues raised by the FDA.
- PTC is participating in a scientific advice procedure with the EMA to determine if the MOVE-FA data could support a conditional marketing authorization application in the EEA. PTC expects to have the outcome of this procedure in the first quarter of 2024.
- PTC had an informal meeting with the FDA for Upstaza for AADC deficiency. The FDA stated that the data PTC has provided to support comparability between the clinical drug product and the intended commercial drug product were still not sufficient. The FDA did say that the available data from the ongoing clinical study in the US assessing the safety of the drug delivery cannula could be used to support a BLA for accelerated approval based on biomarker data demonstrating a treatment-related increase in *de novo* dopamine production. The FDA suggested that PTC conduct a pre-BLA meeting prior to BLA submission to review BLA contents. This meeting has been scheduled for December 2023, and pending the outcome, PTC expects to submit the BLA shortly thereafter.

Third Quarter 2023 Financial Highlights:

- Total revenues were $196.6 million for the third quarter of 2023, compared to $217.1 million for the third quarter of 2022.
- Total revenues include net product revenue across the commercial portfolio of $144.0 million for the third quarter of 2023, compared to $134.2 million for the third quarter of 2022. Total revenues also include collaboration, royalty and manufacturing revenue of $52.5 million in the third quarter of 2023, compared to $82.9 million for the third quarter of 2022.
- Translarna net product revenue was $69.0 million for the third quarter of 2023, compared to $76.6 million for the third quarter of 2022. These results were due to new patients in existing geographies and continued geographic expansion, while the quarter over quarter decrease was due to the timing of bulk government orders.
- Emflaza net product revenue was $67.4 million for the third quarter of 2023, compared to $54.8 million for the third quarter of 2022. These results reflect new patient starts and high compliance.
- Roche reported Evrysdi 2023 year-to-date sales of approximately CHF 1,065 million, resulting in royalty revenue of $50.2 million to PTC for the third quarter of 2023, as compared to $32.9 million for the third quarter of 2022.
- Based on U.S. GAAP (Generally Accepted Accounting Principles), GAAP R&D expenses were $164.2 million for the third quarter of 2023, compared to $165.5 million for the third quarter of 2022.
- Non-GAAP R&D expenses were $150.2 million for the third quarter of 2023, excluding $14.0 million in non-cash, stock-based compensation expense, compared to $150.4 million for the third quarter of 2022, excluding $15.1 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $80.9 million for the third quarter of 2023, compared to $80.1 million for the third quarter of 2022.
- Non-GAAP SG&A expenses were $67.9 million for the third quarter of 2023, excluding $13.0 million in non-cash, stock-based compensation expense, compared to $66.5 million for the third quarter of 2022, excluding $13.6 million in non-cash, stock-based compensation expense.
- During the third quarter of 2023, PTC incurred additional reductions in workforce as part of the continued strategic portfolio prioritization, which resulted in a one-time charge of approximately $22.6 million recorded to R&D and SG&A expense.
- The change in the fair value of deferred and contingent consideration was a loss of $1.5 million for the third quarter of 2023, compared to a gain of $5.3 million for the third quarter of 2022.
- The net loss was $133.0 million for the third quarter of 2023, compared to a net loss of $109.3 million for the third quarter of 2022.
- Cash, cash equivalents, and marketable securities was $294.8 million on September 30, 2023, compared to $410.7 million at December 31, 2022.
- Shares issued and outstanding as of September 30, 2023, were 75,459,022.

PTC Updates Full Year 2023 Financial Guidance as Follows:

- PTC anticipates total revenues for full-year 2023 to be between $940 million and $1.0 billion.
- PTC anticipates net product revenue for the DMD franchise for full-year 2023 to be between $565 million and $595 million.
- PTC anticipates GAAP R&D and SG&A expenses for full-year 2023 to be between $915 million and $965 million.
- PTC anticipates Non-GAAP R&D and SG&A expenses for full year 2023 to be between $810 million and $860 million, excluding estimated non-cash stock-based compensation expense of $105 million.
- PTC expects to incur $37 million of one-time expenses related to the achievement of clinical success-based milestones from previous acquisitions and expenses associated with a rights exchange agreement, which have already been paid in equity and cash.

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 R&D and SG&A expense 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 September 30,		Nine Months Ended September 30,	
	2023	**2022**	**2023**	**2022**
Revenues:				
Net product revenue	$ 144,038	$ 134,186	$ 506,187	$ 407,720
Collaboration revenue	-	50,017	6	50,024
Royalty revenue	50,173	32,924	117,857	73,645
Manufacturing revenue	2,365	-	6,716	-
Total revenues	196,576	217,127	630,766	531,389
Operating expenses:				
Cost of product sales, excluding amortization of acquired intangible assets	9,493	14,011	36,368	33,785
Amortization of acquired intangible asset	58,649	31,023	145,461	80,790
Research and development (1)	164,212	165,462	545,210	462,802
Selling, general and administrative (2)	80,886	80,118	256,249	233,280
Change in the fair value of deferred and contingent consideration	1,500	(5,300)	(125,000)	(32,200)
Intangible asset impairment	-	-	217,800	-
Total operating expenses	314,740	285,314	1,076,088	778,457
Loss from operations	(118,164)	(68,187)	(445,322)	(247,068)
Interest expense, net	(28,160)	(20,880)	(84,905)	(66,371)
Other expense, net	(20,266)	(38,141)	(8,832)	(84,355)
Loss before income tax benefit	(166,590)	(127,208)	(539,059)	(397,794)
Income tax benefit	33,620	17,893	68,247	9,666
Net loss attributable to common stockholders	$ (132,970)	$ (109,315)	$ (470,812)	$ (388,128)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	75,377,997	71,654,671	74,618,611	71,415,849
Net loss per share—basic and diluted (in dollars per share)	$ (1.76)	$ (1.53)	$ (6.31)	$ (5.43)
(1) Research and development reconciliation				
GAAP research and development	$ 164,212	$ 165,462	$ 545,210	$ 462,802
Less: share-based compensation expense	13,986	15,063	44,828	41,896
Non-GAAP research and development	$ 150,226	$ 150,399	$ 500,382	$ 420,906
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 80,886	$ 80,118	$ 256,249	$ 233,280
Less: share-based compensation expense	12,956	13,607	40,300	41,093
Non-GAAP selling, general and administrative	$ 67,930	$ 66,511	$ 215,949	$ 192,187

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

	September 30, 2023	December 31, 2022
Cash, cash equivalents and marketable securities	$ 294,810	$ 410,705
Total Assets	**$ 1,259,885**	**$ 1,705,619**
Total debt	$ 573,174	$ 571,722
Total deferred revenue	1,224	1,351

Total liability for sale of future royalties	763,318	757,886
Total liabilities	**$ 1,930,695**	**$ 2,052,705**
Total stockholders' deficit (75,459,022 and 73,104,692 common shares issued and outstanding at September 30, 2023 and December 31, 2022, respectively)	$ (670,810)	$ (347,086)
Total liabilities and stockholders' deficit	**$ 1,259,885**	**$ 1,705,619**

PTC Therapeutics, Inc.
Reconciliation of GAAP to Non-GAAP Projected Full Year 2023 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	105,000	105,000
Projected non-GAAP R&D and SG&A expense	$ 810,000	$ 860,000

Acronyms:
BLA: Biologics License Application
CHF: Confoederatio Helvetica Francs (Swiss francs)
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
EMA: European Medicines Agency
FA: Friedreich Ataxia
FDA: U.S. Food and Drug Administration
GAAP: Generally Accepted Accounting Principles
HD: Huntington's Disease
NDA: New Drug Application
PKU: Phenylketonuria
R&D: Research and Development
SG&A: Selling, General and Administrative
SMA: Spinal Muscular Atrophy
STRIDE: Strategic Targeting of Registries and International Database of Excellence

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 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 2023 Revenue Guidance as Follows", including with respect to (i) 2023 total revenue guidance, (ii) 2023 net product revenue guidance for the DMD franchise, (iii) 2023 GAAP and non-GAAP R&D and SG&A expense guidance and (iv) 2023 acquisition related one-time 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, commercialization and other matters with respect to its products

and product candidates; PTC's strategy, future operations, future financial position, future revenues, projected costs; the extent, timing and financial aspects of our strategic pipeline prioritization and reductions in workforce; 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: 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; 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 the re-examination process of the Committee for Medicinal Products for Human Use's negative opinion 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 renewal of the conditional marketing authorization for Translarna for the treatment of nmDMD in the EEA and to support a marketing approval for Translarna for the treatment of nmDMD in the United States; whether investigators agree with PTC's interpretation of the results of clinical trials and the totality of clinical data from our trials in Translarna; expectations with respect to Upstaza, including any regulatory submissions and potential approvals, commercialization, manufacturing capabilities, 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; expectations with respect to the commercialization of Tegsedi and Waylivra; the timing of and actual expenses incurred in connection with the discontinuation of PTC's preclinical and early research programs in gene therapy and reductions in workforce, which may be in different periods and may be materially higher than estimated; the savings that may result from the discontinuation of PTC's strategic pipeline prioritization and reductions in workforce, which may be materially less than expected; 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; the potential financial impact and benefits of PTC's leased biologics manufacturing facility; PTC's ability to satisfy its obligations under the terms of its lease agreements, including 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, 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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