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

May 3, 2022

- $149 million total revenue representing impressive 26% year-over-year growth –

- Initiation of PIVOT-HD Phase 2 trial of PTC518 in Huntington's disease –

- CHMP opinion on AADC gene therapy expected in May; ready to execute on potential launch –

SOUTH PLAINFIELD, N.J., May 3, 2022 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and financial results for the first quarter ending March 31, 2022.

"We are continuing to build a robust pipeline of potential new therapeutics that at steady state we anticipate delivering a new product every 2-3 years," said Stuart W. Peltz, Ph.D., Chief Executive Officer, PTC Therapeutics, Inc. "With multiple ongoing registration-directed trials, we are well on the way to fulfill this vision."

Key First Quarter Corporate Updates:

- The Duchenne muscular dystrophy (DMD) franchise continued to show strong growth, with total net product revenue of $128 million for Translarna™ (ataluren) and Emflaza® (deflazacort) in the first quarter of 2022.
  - Translarna total net product revenue of $79 million, with growth coming from new patients in existing markets and continued geographic expansion as PTC drives its robust and globally diversified business.
  - Emflaza total net product revenue of $49 million, with growth driven by continued increases in new patients and high compliance and appropriate weight-based dosing.
- Evrysdi® (risdiplam) net sales in the first quarter of 2022 resulted in $19 million in royalty revenue to PTC. Evrysdi is a product of the SMA collaboration among PTC, the SMA Foundation and Roche.

First Quarter Clinical Updates:

- The PIVOT-HD Phase 2 study of PTC518 for the treatment of Huntington's disease was initiated in the first quarter of 2022.
- In April, Scientific Advisory Group and Oral Explanation meetings with the CHMP were successfully completed. The CHMP opinion on the PTC-AADC marketing authorization application is now expected in May 2022.
- PTC expects to submit the BLA for PTC-AADC in AADC deficiency to the FDA in the third quarter of 2022.
- PTC anticipates reporting results of Study 041 for ataluren by the end of the second quarter of 2022, after data analysis is completed.
- PTC continues to make progress in three additional ongoing registration-directed clinical studies:
  - The MIT-E Phase 2/3 vatiquinone trial for mitochondrial disease associated seizures, with results anticipated in the fourth quarter of 2022.
  - The MOVE-FA Phase 3 vatiquinone trial for Friedreich ataxia, with results anticipated in the second quarter of 2023.
  - The APHENITY Phase 3 trial of PTC923 for PKU, with results anticipated by the end of this year.
- Two additional registration-directed trials were initiated:
  - The CardinALS Phase 2 trial of PTC857 for the treatment of ALS.
  - The SUNRISELMS Phase 2 trial of unesbulin for the treatment of leiomyosarcoma.

First Quarter 2022 Financial Highlights:

- Total revenues were $148.7 million for the first quarter of 2022, compared to $117.9 million for the first quarter of 2021.
- Total revenues included net product revenue across the commercial portfolio of $129.8 million for the first quarter of 2022, compared to $91.3 million for the first quarter of 2021. Total revenues also included collaboration and royalty revenue of $18.9 million in the first quarter of 2022, compared to $26.7 million for the first quarter of 2021.
- Translarna net product revenues were $79.2 million for the first quarter of 2022, compared to $46.5 million for the first quarter of 2021. These results reflect an increase in net product sales in existing markets as well as continued geographic expansion.
- Emflaza net product revenues were $48.6 million for the first quarter of 2022, compared to $43.5 million for the first quarter

of 2021. These results reflect continued addition of new patients, continued high compliance and appropriate weight-based dosing.

- Roche reported Evrysdi sales of approximately CHF 226 million for the first quarter of 2022, resulting in royalty revenue of $18.9 million to PTC in the first quarter of 2022, as compared to $6.7 million for the first quarter of 2021. In the first quarter of 2021, the first commercial sale of Evrysdi in the EU triggered a $20 million milestone payment to PTC. This 2021 achievement was reported as collaboration revenue.
- Based on U.S. GAAP (Generally Accepted Accounting Principles) R&D expenses were $140.1 million for the first quarter of 2022, compared to $134.5 million for the first quarter of 2021. The increase reflects additional investment in research programs and advancement of the clinical pipeline.
- Non-GAAP R&D expenses were $127.0 million for the first quarter of 2022, excluding $13.0 million in non-cash, stock-based compensation expense, compared to $120.8 million for the first quarter of 2021, excluding $13.7 million in non-cash, stock-based compensation expense.
- GAAP SG&A expenses were $73.3 million for the first quarter of 2022, compared to $61.1 million for the first quarter of 2021. The increase reflects our continued investment to support commercial activities, including expanding our commercial portfolio.
- Non-GAAP SG&A expenses were $59.7 million for the first quarter of 2022, excluding $13.6 million in non-cash, stock-based compensation expense, compared to $49.1 million for the first quarter of 2021, excluding $12.0 million in non-cash, stock-based compensation expense.
- The change in the fair value of deferred and contingent consideration was $11.7 million for the first quarter of 2022, compared to $0.1 million for the first 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.
- The net loss was $126.7 million for the first quarter of 2022, compared to a net loss of $128.6 million for the first quarter of 2021.
- Cash, cash equivalents, and marketable securities were $587.8 million on March 31, 2022, compared to $773.4 million at December 31, 2021.
- Shares issued and outstanding as of March 31, 2022 were 71,337,041.

PTC Reaffirms 2022 Financial Guidance:

- PTC anticipates total revenues for full year 2022 to be between $700 and $750 million.
- PTC anticipates net product revenues for the DMD franchise for full year 2022 to be between $475 and $495 million.
- PTC anticipates GAAP R&D and SG&A expenses for full year 2022 to be between $915 and $965 million.
- PTC anticipates Non-GAAP R&D and SG&A expense for 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 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 March 31,	
	2022	**2021**
Revenues:		
Net product revenue	$ 129,832	$ 91,280
Collaboration revenue	7	20,007
Royalty revenue	18,896	6,655
Total revenues	148,735	117,942
Operating expenses:		

Cost of product sales, excluding amortization of acquired intangible asset	10,135	9,104
Amortization of acquired intangible asset	23,473	11,278
Research and development (1)	140,078	134,513
Selling, general and administrative (2)	73,271	61,095
Change in the fair value of deferred and contingent consideration	(11,700)	100
Total operating expenses	235,257	216,090
Loss from operations	(86,522)	(98,148)
Interest expense, net	(23,514)	(19,159)
Other (expense) income, net	(11,855)	(10,884)
Loss before income tax expense	(121,891)	(128,191)
Income tax expense	(4,835)	(451)
Net loss attributable to common stockholders	$ (126,726)	$ (128,642)
Weighted-average shares outstanding:		
Basic and diluted (in shares)	71,215,105	70,188,602
Net loss per share—basic and diluted (in dollars per share)	$ (1.78)	$ (1.83)
(1) Research and development reconciliation		
GAAP research and development	$ 140,078	$ 134,513
Less: share-based compensation expense	13,034	13,725
Non-GAAP research and development	$ 127,044	$ 120,788
(2) Selling, general and administrative reconciliation		
GAAP selling, general and administrative	$ 73,271	$ 61,095
Less: share-based compensation expense	13,555	11,982
Non-GAAP selling, general and administrative	$ 59,716	$ 49,113

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

	March 31, 2022	**December 31, 2021**
Cash, cash equivalents and marketable securities	$ 587,793	$ 773,376
Total Assets	**$ 1,799,591**	**$ 1,938,056**
Total debt	$ 431,897	$ 431,434
Total liability for sale of future royalties	744,746	733,985
Total liabilities	**$ 1,890,172**	**$ 1,936,618**
Total stockholders' (deficit) equity (71,337,041 and 70,828,226 common shares issued and outstanding at March 31, 2022 and December 31, 2021 respectively)	$ (90,581)	$ 1,438
Total liabilities and stockholders' (deficit) equity	**$ 1,799,591**	**$ 1,938,056**

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

Acronyms:

ALS: Amyotrophic Lateral Sclerosis
BLA: Biologics License Application
CHMP: Committee for Medicinal Products for Human Use
DMD: Duchenne Muscular Dystrophy
EMA: European Medicines Agency
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:

PTC will host a conference call to discuss the first quarter 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 9963177. 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.

For More Information:

Investors

Kylie O'Keefe

+1 (908) 300-0691

kokeefe@ptcbio.com

Media

Jeanine Clemente

+1 (908) 912-9406

jclemente@ptcbio.com

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 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.

View original content:https://www.prnewswire.com/news-releases/ptc-therapeutics-provides-a-corporate-update-and-reports-first-quarter-financial-results-301538794.html

SOURCE PTC Therapeutics, Inc.