PTC Therapeutics Reports Second Quarter 2020 Financial Results and Provides a Corporate Update

August 5, 2020

- Strong Emflaza® 2Q 2020 performance with 30% YoY net product revenue increase -

- Initiated PTC299 trial in COVID-19 in multiple countries -

- Initiated clinical trial with PTC857, identified from Bio-e platform -

- Greater than $1B in cash to accelerate pipeline growth -

SOUTH PLAINFIELD, N.J., Aug. 5, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced a corporate update and reported financial results for the second quarter ending June 30, 2020.

"We are pleased with the progress in our pipeline and the strong commercial performance of our global Duchenne franchise," said Stuart W. Peltz, Ph.D., CEO of PTC Therapeutics. "Our growing revenue stream allows us to continue to invest in the development of treatments leveraging our novel technology platforms. We have a robust pipeline and with more than $1 billion in cash are well positioned to advance multiple differentiated therapies for patients living with rare disorders, not only in the near term, but also for many years to come."

Key Second Quarter and Other Corporate Updates:

- Our Duchenne muscular dystrophy franchise had strong performance in the second quarter. The greater than 30% year-over-year increase in second quarter sales for Emflaza was driven by ongoing improvements in our commercial business. New Duchenne patients continue to be identified in Europe, LATAM and other key markets for Translarna ™ despite the challenges of COVID-19.
- As part of the annual renewal process for Translarna, the European Medicines Agency (EMA) confirmed its risk/benefit profile for the sixth consecutive year. In addition, PTC previously announced that the Committee for Medicinal Products for Human Use (CHMP) of the EMA recommended to remove the statement "efficacy has not been demonstrated in non-ambulatory patients" from the SmPC for Translarna. This label change enables healthcare professionals to use their clinical judgement to make treatment decisions for their patients on Translarna who have lost ambulation and should support reimbursement agencies granting continued access to Translarna.
- In the second quarter, PTC strengthened its pipeline and platforms with the acquisition of a late-stage asset for phenylketonuria (PKU). PTC923 is a clinical-stage investigational therapy for inborn errors of metabolism, including PKU and other diseases associated with defects in the tetrahydrobiopterin (BH4) biochemical pathways diagnosed at birth. PTC is currently conducting nonclinical studies to support the long-term dosing for the planned phase 3 trial in PKU in 2021.
- The U.S. Food and Drug Administration (FDA) PDUFA date for risdiplam is August 24, 2020. In addition, a submission of a marketing authorization application (MAA) to EMA for risdiplam is imminent. The filing of the MAA would trigger a $15 million milestone payment to PTC from Roche. Roche has submitted applications for approval in Brazil, Chile, China, Indonesia, Russia, South Korea and Taiwan.
- PTC recently announced an agreement to monetize a portion of the rights to the risdiplam royalty stream for a $650 million up front payment from Royalty Pharma plc, providing PTC with significant non-dilutive capital. PTC retains nearly 60% of the risdiplam royalty stream up until a $1.3 billion threshold is reached, after which PTC retains 100% of the risdiplam royalty stream. PTC also retains all economics associated with up to approximately $400 million in remaining regulatory and sales milestones.
- Launch activities for Tegsedi® continue to focus on patient finding. Pricing discussions in Brazil are ongoing and are expected to be completed by the end of the year.
- The submission of an application for marketing authorization for Waylivra® in patients with familial chylomicronemia syndrome (FCS) has been filed with ANVISA and a decision is expected in 2021. Patient finding and early access programs are ongoing.
- PTC continues to develop manufacturing capabilities for its gene therapy products. It has expanded its collaboration with MassBiologics for the manufacturing of the aromatic L-amino acid decarboxylase deficiency (AADCd) commercial supply. In addition, PTC gained occupancy under its lease of ~220,000 square feet at the former BMS site in Hopewell, NJ. Manufacturing of clinical materials for its gene therapy programs at this site is expected to initiate in 2021.
- PTC recently launched a global internship program, the Talent Pipeline Program (TPP), aimed at providing recent graduates, especially those in minority communities, with real-world experience in the biopharmaceutical industry and related professions, including research, finance, commercial, compliance, quality, legal, information technology, and

communications. The TPP will provide approximately 30 interns with a one-year paid program with real-world training experience.

Updates in PTC's Diverse Product Pipeline

- In its Bio-e platform, PTC expects to initiate potential registrational trials with vatiquinone, formerly known as PTC743, in refractory mitochondrial epilepsy in the third quarter of 2020 and in Friedreich ataxia in the fourth quarter of 2020.
- The first subject has been dosed in the Phase 1 trial for PTC857, PTC's second compound from the Bio-e platform. Data from the single ascending dose and multiple ascending dose studies is expected by the end of 2020.
- PTC recently initiated a Phase 2/3 clinical trial for PTC299 for COVID-19 called FITE-19. PTC expects Stage 1 to be completed in the second half of 2020 and anticipates reporting top-line results from both stages in the first half of 2021. Multiple sites have been opened in the U.S., Brazil, Spain and Australia with additional countries for Stage 2 expected to initiate in the coming months.
- In the novel splicing platform, the Huntington disease program remains on track for the initiation of first-in-human studies in 2020.
- PTC expects to initiate submission of the biologics license application (BLA) to the U.S. FDA in the second half of 2020 for its AADCd gene therapy program.
- Due to COVID-19 related delays, PTC now expects the final opinion from the CHMP on the MAA for the AADCd program in the first quarter of 2021.
- Given the evolving COVID-19 situation in the U.S., the final study muscle biopsies have not yet been collected from 8 remaining boys in the Translarna (ataluren) dystrophin study. PTC is continuously monitoring the situation to determine when it will be possible to safely obtain the final biopsies and is exploring all potential options in order to have a data read out by the end of 2020. All patients in the study remain on Translarna until they are able to complete the final study visit.

Financial Highlights:

- Total net product revenues were $75.2 million across our commercial portfolio for the second quarter of 2020, compared to total net product revenues of $85.5 million for the second quarter of 2019.
- Translarna net product revenues were $38.6 million for the second quarter of 2020, compared to $57.8 million for the second quarter of 2019. Revenues for the second quarter of 2020 were impacted by the timing of a group purchase order from Brazil, which is the primary driver for the year-over-year decrease, as the second quarter of 2019 included a significant group purchase order from Brazil. Due to the impact of the pandemic, there was an administrative delay by the Brazilian Ministry of Health in receiving the centralized Translarna group purchase order. PTC anticipates a group purchase order from Brazil later this year.
- Emflaza net product revenues were $36.2 million for the second quarter of 2020, compared to $27.6 million for the second quarter of 2019. Growth in net product revenues was driven by new patient prescriptions and continued operational improvements and efficiencies in our commercial business.
- Generally accepted accounting principles in the U.S. (GAAP) research and development (R&D) expenses were $176.5 million for the second quarter of 2020, compared to $60.0 million for the second quarter of 2019. The increase in R&D expenses includes one-time charges of $53.6 million related to the acquisition of Censa Pharmaceuticals, and $41.2 million related to the MassBiologics of the University of Massachusetts Medical School agreement for commercial manufacturing of our lead gene therapy program in AADCd.
- Non-GAAP R&D expenses were $168.0 million for the second quarter of 2020, excluding $8.6 million in non-cash, stock-based compensation expense, compared to $54.5 million for the second quarter of 2019, excluding $5.5 million in non-cash, stock-based compensation expense.
- GAAP selling, general and administrative (SG&A) expenses were $53.7 million for the second quarter of 2020, compared to $49.2 million for the second quarter of 2019. The increase reflects continued investment to support our commercial activities including our expanding commercial portfolio.
- Non-GAAP SG&A expenses were $45.3 million for the second quarter of 2020, excluding $8.3 million in non-cash, stock-based compensation expense, compared to $43.8 million for the second quarter of 2019, excluding $5.4 million in non-cash, stock-based compensation expense.
- Change in the fair value of deferred and contingent consideration was $7.7 million for the second quarter of 2020, compared to $5.3 million for the second quarter of 2019. 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 second quarter of 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 the Merger, regardless of whether the milestones are achieved.

- Net loss was $181.4 million for the second quarter of 2020, compared to net loss of $41.8 million for the second quarter of 2019.
- Cash, cash equivalents and marketable securities were $498.9 million as of June 30, 2020, compared to $686.6 million as of December 31, 2019.
- Shares issued and outstanding as of June 30, 2020 were 67,240,679.

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 June 30,		Six Months Ended June 30,	
	2020	2019	2020	2019
Revenues:				
Net product revenue	$ 75,239	$ 85,476	$ 143,435	$ 138,530
Collaboration and grant revenue	—	46	63	575
Total revenues	75,239	85,522	143,498	139,105
Operating expenses:				
Cost of product sales	5,304	3,211	9,389	5,587
Amortization of acquired intangible asset	8,731	6,575	16,679	12,652
Research and development (1)	176,525	59,979	266,632	112,544
Selling, general and administrative (2)	53,659	49,215	111,869	89,760
Change in the fair value of deferred and contingent consideration	7,680	5,300	8,580	26,460
Settlement of deferred and contingent consideration	10,613	—	10,613	—
Total operating expenses	262,512	124,280	423,762	247,003
Loss from operations	(187,273)	(38,758)	(280,264)	(107,898)
Interest expense, net	(5,379)	(2,074)	(11,021)	(4,362)
Other income (expense), net	11,309	(183)	(2,523)	(292)
Loss before income tax expense	(181,343)	(41,015)	(293,808)	(112,552)
Income tax expense	(84)	(774)	(306)	(1,350)
Net loss attributable to common stockholders	$ (181,427)	$ (41,789)	$ (294,114)	$ (113,902)
Weighted-average shares outstanding:				
Basic and diluted (in shares)	65,150,780	55,912,748	63,769,958	57,113,141
Net loss per share—basic and diluted (in dollars per share)	$ (2.78)	$ (0.75)	$ (4.61)	$ (1.99)
(1) Research and development reconciliation				
GAAP research and development	$ 176,525	$ 59,979	$ 266,632	$ 112,544
Less: share-based compensation expense	8,562	5,516	16,741	10,203
Non-GAAP research and development	$ 167,963	$ 54,463	$ 249,891	$ 102,341
(2) Selling, general and administrative reconciliation				
GAAP selling, general and administrative	$ 53,659	$ 49,215	$ 111,869	$ 89,760
Less: share-based compensation expense	8,348	5,404	15,389	9,981
Non-GAAP selling, general and administrative	$ 45,311	$ 43,811	$ 96,480	$ 79,779

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

	June 30, 2020	December 31, 2019
Cash, cash equivalents and marketable securities	$ 498,891	$ 686,563
Total Assets	**$ 1,476,637**	**$ 1,623,782**

Total debt	$ 315,362	$ 313,859
Total deferred revenue	7,702	11,657
Total liabilities	**$ 904,143**	**$ 1,029,452**
Total stockholders' equity (67,240,679 and 61,935,870 common shares issued and outstanding at June 30, 2020 and December 31, 2019 respectively)	$ 572,494	$ 594,330
Total liabilities and stockholders' equity	**$ 1,476,637**	**$ 1,623,782**

Today's Conference Call and Webcast Reminder:
Today's conference call will take place 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 5897698. 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. 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 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 potential regulatory submissions and manufacturing capabilities; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, commercialization or royalty or milestone payments; PTC's expected use of proceeds from the agreement with Royalty Pharma; PTC's expectations with respect to the licensing, regulatory submissions and commercialization of its products and product candidates; the timing with respect to orders for PTC's products; expectations with respect to the impacts of the COVID-19 pandemic and related response measures; 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: 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 potential 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 potential regulatory submissions and potential commercialization with respect to risdiplam; PTC's ability to complete a dystrophin study necessary to support a re-submission of its Translarna NDA for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD) to the FDA, and PTC's ability to perform any necessary additional clinical trials, non-clinical studies, and CMC assessments or analyses at significant cost; 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 enroll, 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 for COVID-19; expectations with respect to the COVID-19 pandemic and related response measures and their effects on PTC's business, operations, clinical trials, potential regulatory submissions and approvals, and PTC's collaborators, contract research organizations, suppliers and manufacturers; 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, risdiplam, 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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