PTC Therapeutics Announces Agreement to Monetize a Portion of the Risdiplam Royalty Stream for $650 Million

July 20, 2020

- Strategic partnership with Royalty Pharma plc. enables PTC to strengthen and advance its diversified rare disorders portfolio -

- Conference call scheduled for 8:30 am ET -

SOUTH PLAINFIELD, N.J., July 20, 2020 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced an agreement to monetize a portion of the risdiplam royalty stream for $650 million from Royalty Pharma plc. The capital from the collaboration will enable PTC to further develop and expand its innovative rare disorder portfolio, particularly its validated splicing, Bio-e and gene therapy platforms.

"The discovery, development and expected commercialization of risdiplam exemplifies how PTC's strengths in novel scientific approaches to diseases with high unmet needs can generate value for the benefit of all of our stakeholders," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "Today's announcement of our strategic partnership with Royalty Pharma brings forward significant, non-dilutive capital to drive further innovation and growth across our robust and diverse rare disorder portfolio."

Under the terms of the royalty purchase agreement, PTC Therapeutics will receive $650 million in upfront cash from Royalty Pharma in return for approximately 43% of the risdiplam royalties, up to a specified amount. PTC Therapeutics maintains the majority of the risdiplam royalty stream and retains all economics associated with up to approximately $400 million in remaining regulatory and sales milestones.

Pablo Legorreta, Royalty Pharma's Founder and Chief Executive Officer, stated, "Risdiplam is consistent with our focus on high value, differentiated therapeutics addressing diseases with high unmet medical need. We recognize the value and importance of an oral therapy for the treatment of all types of SMA. We are delighted to partner with PTC and to help fund their innovative pipeline of treatments for rare diseases."

Wilmer Cutler Pickering Hale and Dorr LLP acted as legal advisor to PTC Therapeutics on the transaction. Goodwin Proctor, Dechert and Maiwald acted as legal advisors to Royalty Pharma.

The risdiplam New Drug Application (NDA) for the treatment of Types 1, 2 and 3 spinal muscular atrophy (SMA) is under Priority Review by the U.S Food and Drug Administration, with a PDUFA date of August 24, 2020. A Marketing Authorization Application (MAA) is planned to be submitted to the European Medicines Agency (EMA), as well as filings in other international markets. The risdiplam SMA program is a collaboration between PTC, the SMA Foundation, and Roche.

Today's Conference Call:
Today's conference call will take place at 8:30 am ET and can be accessed by dialing (877) 303-9216 (domestic) or (973) 935-8152 (international) five minutes prior to the start of the call and providing the passcode 3492277. 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 PTC's website for 30 days following the call.

About Risdiplam
Risdiplam is an investigational survival motor neuron 2 (SMN2) splicing modifier for SMA and is an orally administered liquid. It is designed to increase and sustain SMN protein levels both throughout the central nervous system and in peripheral tissues of the body. Risdiplam is being studied in the broadest clinical trial program in SMA, with patients ranging from birth to 60 years old, and includes patients previously treated with other SMA-targeting therapies. The clinical trial population represents the diverse, real-world spectrum of people living with this disease. The risdiplam clinical development program was designed with the aim of enabling access for all appropriate patients.

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 historical fact, are forward-looking statements, including statements regarding: the future expectations, plans and prospects for PTC; advancement of PTC's joint collaboration program in SMA, including any potential regulatory submissions, regulatory approvals or commercial prospects; PTC's strategy, future operations, future financial position, future revenues and, projected costs; PTC's expected use of proceeds from the agreement with Royalty Pharma; potential royalties and potential regulatory and sales milestone payments; 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 enrollment, conduct, and results of 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 regards to risdiplam; the eligible patient base and commercial potential of risdiplam or any of PTC's other product candidates; 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 or product candidate will receive or maintain regulatory approval in any territory, or prove to be commercially successful, including risdiplam.

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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