PTC to Expand Product Portfolio in Latin America with WAYLIVRA™

March 4, 2019

WAYLIVRA, the first and only therapy for the treatment of familial chylomicronemia syndrome (FCS), has received a positive opinion from the CHMP

SOUTH PLAINFIELD, N.J., March 4, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that its partner, Akcea Therapeutics, affiliate of Ionis Pharmaceuticals, received a positive opinion for WAYLIVRA™ by the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). PTC plans to initiate activities to bring WAYLIVRA to patients in Latin America following European Commission ratification.

"We are excited that the CHMP gave a positive opinion to WAYLIVRA™," said Stuart Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "Patients with FCS have no effective treatment. We, along with Akcea, look forward to bringing WAYLIVRA to those suffering from FCS. We have the team in place to bring WAYLIVRA to people with FCS in Latin America as quickly as possible."

Upon ratification of the positive option of the CHMP by the European Commission, PTC will pay Akcea the balance of the up-front licensing fee. WAYLIVRA was discovered by Ionis Pharmaceuticals and co-developed by Ionis and Akcea.

About PTC Therapeutics, Inc.
PTC is a science-led, 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 pipeline of transformative medicines and our mission to provide access to best-in-class treatments for patients who have an unmet medical need.

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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 future expectations, plans and prospects for PTC; the timing and outcome of the regulatory process for WAYLIVRA™, including the final determination by the European Commission with respect to the marketing authorization for WAYLIVRA; PTC's expectations with respect to the licensing and potential commercialization of WAYLIVRA, including any milestone and royalty payments to be made by PTC to Akcea Therapeutics, Inc.; 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," "vision," "project," 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 any products or product candidates that PTC may commercialize; expectations with respect to the potential financial impact and benefits of the collaboration and licensing agreement with Akcea Therapeutics, Inc., including with respect to the timing of regulatory approval of TEGSEDI™ and WAYLIVRA in countries in Latin America and the Caribbean, the commercialization of TEGSEDI and WAYLIVRA, and PTC's expectations with respect to contingent payments to Akcea based on net sales and the potential achievement of regulatory milestones; the eligible patient base and commercial potential of TEGSEDI and WAYLIVRA or any of PTC's other product or product candidates; and the factors discussed in the "Risk Factors" section of PTC's Annual Report on Form 10-K for the year ended December 31, 2018, 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 TEGSEDI, WAYLIVRA or any other product or product candidate.

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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