

# PTC Therapeutics Deepens Financial and Commercial Expertise of Board with Two New Appointments

December 18, 2018

SOUTH PLAINFIELD, N.J., Dec. 18, 2018 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the appointment of Emma Reeve and Stephanie Okey to the Company's Board of Directors. Ms. Reeve currently serves as the Chief Financial Officer of Constellation Pharmaceuticals, Inc., a publicly-traded company, and will enhance the financial expertise of the Board. Ms. Okey most recently served as the Senior Vice President, Head of North America, Rare Diseases and U.S. General Manager, Rare Disease Business Unit at Genzyme and brings decades of experience launching and commercializing rare and orphan disease products.

"We are very pleased to welcome Emma and Stephanie to PTC's Board of Directors," said Stuart W. Peltz, Ph.D., Chief Executive Officer of PTC Therapeutics. "As PTC continues to launch and commercialize products globally, Emma and Stephanie will provide the global experience and strategic leadership that will be vital to the growth of PTC. Emma's diverse global experience and deep knowledge of financial strategies, such as international reimbursement policies, and Stephanie's extensive rare disease commercial experience will assist in the delivery of therapies to the patients that urgently need them."

Ms. Reeve has over 20 years of global financial experience across pharmaceutical, medical device and bio-pharma companies. As Chief Financial Officer of Constellation Pharmaceuticals, Ms. Reeve ensured the Company was well-capitalized, and oversaw Constellation's initial public offering in July 2018. Prior to Constellation, Ms. Reeve acted as interim Chief Financial Officer and Corporate Controller of Parexel, a global biopharmaceutical services company, where she was responsible for all aspects of finance, investor relations, procurement and facilities, leading a team of over 950 people. Ms. Reeve served as Chief Financial Officer of Inotek Pharmaceuticals from 2005 to 2006 and of Aton Pharma from 2001 to 2003. She held senior finance roles at Bristol-Myers Squibb, Merck and Novartis.

Ms. Okey brings nearly three decades of biotech experience, launching and commercializing rare and orphan disease products, as well as large market products. During her time at Genzyme, Ms. Okey oversaw 500 employees and was responsible for six business units: Gaucher Disease and MPS1, Fabry Disease, Pompe and Neuromuscular Diseases, Cardiovascular Diseases, Endocrine Diseases and Leukine and Immunologic Diseases. She has successfully launched 13 therapeutic agents, 9 of which were in the orphan disease space. Ms. Okey is highly knowledgeable about complex approval processes, healthcare stake holder systems and markets. Prior to her 19-year tenure at Genzyme, Ms. Okey held roles at MedImmune, Genentech and Bristol-Myers Squibb.

"I am delighted to join the board of PTC at this exciting time, when the Company has the potential for several global regulatory filings in 2019 including a BLA in AADC," said Ms. Reeve. "PTC is well-positioned to leverage its global footprint and strong financial profile as it drives the development and commercialization of its pipeline of therapeutics for rare disorders."

"PTC is aligned with my personal passion to provide therapies to patients and their families in urgent need of treatments, especially in the rare disease space," said Ms. Okey. "I am excited to add my commercial experience to the existing foundation for growth of PTC and join its distinguished Board."

## 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. Founded 20 years ago, PTC Therapeutics has successfully launched two rare disorder products and has a global commercial footprint. This success 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 statements regarding: the future expectations, plans and prospects for PTC; expectations with respect to PTC's recently acquired gene therapy platform, including any potential regulatory submissions; PTC's expectations with respect to the licensing and potential commercialization of Tegsedi and Waylivra; expansion of commercialization of Translarna and Emflaza; advancement of PTC's joint collaboration program in SMA; 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 Emflaza and Translarna and any other product candidates for which PTC may commercialize in the future; whether, and to what extent, third party payors impose additional requirements before approving Emflaza prescription reimbursement; PTC's ability to complete any dystrophin study necessary in order to resolve the matters set forth in the denial to the Complete Response letter it received from the FDA in connection with its new drug application for Translarna for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD), and PTC's ability to perform 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, placebocontrolled 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 potential financial impact or PTC's ability to realize the anticipated benefits of the acquisition of Agilis and its gene therapy platform, including with respect to the business of Agilis and expectations with respect to the potential achievement of development, regulatory and sales milestones and contingent payments to the former Agilis equityholders with respect thereto and PTC's ability to obtain marketing approval of PTC-AADC and other product candidates acquired from Agilis, will not be realized or will not be realized within the expected time period; 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 LATAM 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; PTC's ability to realize the anticipated benefits of the acquisition of Emflaza, including the possibility that the expected benefits from the acquisition will not be realized or will not be realized within the expected time period; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to the acquisition of Emflaza or the acquisition of its gene therapy pipeline, as well as other 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 Translarna, Emflaza, PTC-AADC, Tegsedi, Waylivra or any of PTC's other product candidates; 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; PTC's scientific approach and general development progress; PTC's ability to satisfy its obligations under the terms of the senior secured term loan facility with MidCap Financial; 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 Annual Report on Form 10-K for the year ended December 31, 2017, Quarterly Reports on Form 10-Q for the periods ended March 31, 2018, June 30, 2018 and September 30, 2018 and Exhibit 99.2 to PTC's Current Report on Form 8-K filed on August 24, 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 Translarna, Emflaza, PTC-AADC, 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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