Translarna™ (ataluren) is the First Therapy Approved in Brazil for Duchenne Muscular Dystrophy

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- Ambulatory Duchenne patients who are 5 years and older with a nonsense mutation can now access a treatment that targets the underlying cause -

SOUTH PLAINFIELD, N.J., April 29, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced that Translarna ™(ataluren) has been granted marketing approval from the Brazilian National Health Surveillance Agency (ANVISA) under rare diseases procedure, for the treatment of ambulatory children five years and older with Duchenne muscular dystrophy caused by a nonsense mutation. Patients now can have access to a treatment that targets the underlying cause.

"The regulatory approval from the Brazilian authorities will accelerate access to Translarna for the many patients who have been waiting for treatment," stated Eric Pauwels, Senior Vice President and General Manager of the Americas of PTC Therapeutics, Inc. "We are committed to working quickly to make Translarna available to all patients in Brazil who may benefit."

Primarily affecting males, Duchenne muscular dystrophy is a rare, irreversible, and fatal genetic disorder that results in progressive muscle weakness from early childhood and leads to premature death in the mid-twenties due to heart and respiratory failure. Patients with Duchenne can lose the ability to walk as early as age ten, followed by loss of the use of their arms. Duchenne patients subsequently experience life-threatening lung complications, requiring the need for ventilation support, and heart complications in their late teens and twenties.

"Muscle damage in Duchenne starts very young, so early diagnosis and treatment is critical to maintain muscle function and delay disease progression," stated Dr. Alexandra Prufer, Associate Professor of Pediatric Neurology, Department of Pediatrics, Medical School, The Federal University of Rio de Janeiro. "Treatment measures should be started once children are diagnosed, when there is the most amount of muscle to effect."

A**bout Translarna**
Ataluren, discovered and developed by PTC Therapeutics, Inc., is a protein restoration therapy designed to enable the formation of a functioning protein in patients with genetic disorders caused by a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. The resulting disorder is determined by which protein cannot be expressed in its entirety and is no longer functional, such as dystrophin in Duchenne muscular dystrophy. Translarna, tradename ataluren, is licensed in the European Economic Area for the treatment of nonsense mutation Duchenne muscular dystrophy in ambulatory patients aged two years and older. Ataluren is an investigational new drug in the United States.

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 statements regarding: the future expectations, plans and prospects for PTC; expansion of commercialization of Translarna; 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 that 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 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 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 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 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 with regards to risdiplam; significant transaction costs, unknown liabilities, the risk of litigation and/or regulatory actions related to 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, risdiplam or any of PTC's other product candidates; 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, 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, Waylivra or 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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