PTC Therapeutics Launches New Program to Support Research Projects in Rare, Genetic Disorders

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- $500,000 funding to improve Duchenne muscular dystrophy diagnosis and screening in 2019 -

SOUTH PLAINFIELD, N.J., Jan. 31, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today launched PRIORITY, an annual funding program for innovative research. In its inaugural year, PRIORITY will fund programs designed to improve Duchenne screening in infants and support earlier diagnosis of this progressive, muscle-wasting disease.

"As a research-driven, patient-centric company, it's important to PTC that we support the efforts of clinicians and researchers who share our passion for improving the clinical care of people with rare, genetic disorders," said Dr. Claudio Santos, Senior Vice President, Global Medical Affairs, PTC Therapeutics. "Our hope is that each year, the PRIORITY program will help accelerate research into innovative, practicable, and sustainable solutions to the many challenges posed by these disorders. In 2019, we are excited to be creating opportunities for improved screening and diagnosis of Duchenne to ensure patients can receive the best care as early as possible."

The 2019 PRIORITY program invites funding proposals from clinicians in the fields of neurology, genetics, and pediatrics for submission between now and March 31st, 2019. A fund of $500,000 will be divided among successful applicants. PRIORITY grant recipients will be announced in April 2019.

Funding applications will be evaluated by an external Review Committee comprised of independent Duchenne experts from around the world.

"This year's program will recognize innovative initiatives to improve screening for Duchenne in infants that will ultimately lead to a much earlier diagnosis of this progressive disease," said Dr. Yann Péréon, Neurologist at University Hospital – CHU Nantes, France, who is part of the Review Committee. "Earlier diagnosis is critical to ensure patients get timely access to the best care, and to allow families to plan for the future, and we encourage the neuromuscular community to be inventive, smart and sustainable in their proposals."

Duchenne muscular dystrophy is a rare disease and affects approximately 1 in every 3,600–6,000 live male births.[1] Despite improvements in clinical care over the last 20 years, there remains a delay in the diagnosis of children with Duchenne (mean age of diagnosis averaging between 4–4.5 years, with many diagnoses occurring much later). This delay reduces the benefits of post-diagnosis interventions that can delay loss of ambulation and extend quality of life.[2,3,4,5]

For more information around the criteria for applications, please visit the PRIORITY page on the PTC website: www.ptcbio.com/priority

About Duchenne Muscular Dystrophy
Primarily affecting males, Duchenne muscular dystrophy (Duchenne) is a rare 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. It is a progressive muscle disorder caused by the lack of functional dystrophin protein. Dystrophin is critical to the structural stability of all muscles, including skeletal, diaphragm, and heart muscles. 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. More information on the signs and symptoms of Duchenne can be found at: www.duchenneandyou.com

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 our continued 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, other than those of historical fact contained in this release are forward-looking statements, including statements regarding the future expectations, plans and prospects for PTC. Other forward-looking statements may be identified by the words "plan," "guidance," "anticipate," "believe," "estimate,"

"expect," "intend," "may," "predict," "project," "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 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. 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 release except as required by law.

References

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