PTC Therapeutics Announces First-Ever Winners of Its PRIORITY Program To Support Research Projects In Rare, Genetic Disorders

May 20, 2019

- $545,000 awarded to help improve Duchenne muscular dystrophy diagnosis and screening across the globe -

SOUTH PLAINFIELD, N.J., May 20, 2019 /PRNewswire/ -- PTC Therapeutics, Inc. (NASDAQ: PTCT) today announced the three funding recipients from PRIORITY, an annual program to support outstanding clinical research by the rare genetic disorders community. This year's funding will accelerate innovative research projects to improve Duchenne muscular dystrophy screening in infants and support earlier diagnosis of this progressive, muscle-wasting disease.

"We were overwhelmed to receive so many outstanding applications in the first year of PRIORITY and to see such a breadth of ideas to improve the screening and diagnosis of Duchenne," said Dr. Claudio Santos, Senior Vice President, Global Medical Affairs, PTC Therapeutics, Inc. "We are thrilled that the PRIORITY program will help bring these research projects to fruition and ultimately help patients receive the best care as early as possible."

Award entries were submitted from ten countries and evaluated by an independent panel of Duchenne experts from around the world. This year's PRIORITY recipients and their winning proposals are:

- Karolinska Institutet, Baltic/Nordic, Sweden: Serum-CK-based screening project for children presenting with early symptoms of Duchenne
- Department of Clinical & Experimental Medicine, Messina University, Italy: Pilot study in Sicily of two-tier infant screening for Duchenne through CK testing (boys aged 6-42 months), followed by dystrophin gene testing
- Italian Society of Pediatric Doctors (SIMPE), Italy: Duchenne education and early monitoring for neuromuscular delays using a computer management system already being used by pediatricians who are members of the Pediatric Epidemiology and Research Network (REPER) to communicate with parents

"The quantity and quality of the submissions we received highlight the unmet need for improved screening and earlier diagnosis in Duchenne," said Dr. Craig Campbell, Pediatric Neurologist at the Children's Hospital - London Health Science Centre, Canada, who is part of the expert Review Committee. "The proposals were inventive, smart and sustainable; we hope that PRIORITY can act as a springboard to help the research community turn ideas into solutions for patients and their families in the future."

Duchenne muscular dystrophy is a rare disease and affects approximately 1 in every 3,500 live male births, according to Parent Project Muscular Dystrophy and approximately 1 in every 5,000 live male births according to Ryder (2017) in the European Journal of Human Genetics. Despite improvements in clinical care over the last 20 years, there is a delay in the diagnosis of children with Duchenne (mean age of diagnosis averaging between 4–4.5 years) and many children are diagnosed much later, when the benefits of treatment in delaying loss of ambulation and extending quality of life are not as optimal.[1,2,3,4] Following diagnosis, interventions can be made to extend the child's life expectancy and enhance their quality of life.

For more information about PRIORITY and each recipient, please visit the PRIORITY page on the PTC website here.

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.

For More Information:

Investors:
Emily Hill
+ 1 (908) 912-9327
ehill@ptcbio.com

Media:
Jane Baj
+1 (908) 912-9167

jbaj@ptcbio.com

Forward-Looking Statements:
All statements, other than those of historical fact, contained in this release are forward-looking statements, including statements regarding PTC's strategy, future operations, future financial position, future revenues or 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 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.

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.

References

1. Van Ruiten HJ et al. State-Of-The-Art Advances in Muscular Dystrophy. *European Medical Journal*. 2017 2(1):90-99.

2. Ryder S et al. The burden, epidemiology, costs and treatment for Duchenne muscular dystrophy: an evidence review. *Orphanet Journal of Rare Diseases*. 2017;12(1):79.

3. Van Ruiten HJ et al. Improving recognition of Duchenne muscular dystrophy: a retrospective case note review. *Archives of Disease in Childhood.* 2014;99(12):1074–7.

4. Ciafaloni E et al. Delayed diagnosis in Duchenne muscular dystrophy: data from the Muscular Dystrophy Surveillance, Tracking and Research Network (MDSTARmet). *The Journal of Pediatrics*. 2009; 155(3):380-5.