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PTC Therapeutics and Parent Project Muscular Dystrophy Advance Drug Discovery Program to Improve Heart Function in Duchenne/Becker Muscular Dystrophy Patients

- PPMD awards $250,000 grant towards research program-

SOUTH PLAINFIELD, NJ AND HACKENSACK, NJ – FEBRUARY 1, 2011 – PTC Therapeutics, Inc. (PTC) and Parent Project Muscular Dystrophy (PPMD) today announced an expansion of their collaboration to discover new treatments for patients with Duchenne/Becker muscular dystrophy (DBMD). PPMD has awarded PTC a $250,000 grant, with the potential of additional funding, to advance drug discovery efforts in identifying a new treatment that improves heart function in patients with DBMD.

PPMD's President Pat Furlong explained the decision for the grant, "The fight to end Duchenne and Becker muscular dystrophies must involve a comprehensive approach, considering all of the critical muscles that make up the human body. The heart is a muscle too, and Parent Project Muscular Dystrophy has made cardiac health a top priority within our research investment for 2011. PTC Therapeutics understood the importance of cardiac health from the moment we agreed to collaborate with them, now over ten years ago. PTC continues to make our community's priorities their own and we are hopeful their approach to increase the level of SERCA2a protein will translate into healthier hearts for our sons."

Heart failure is a leading cause of death in patients with advanced DBMD. Through the course of the disease, heart function is progressively compromised by calcium overload and inadequate transport, which leads to cell imbalance, dysfunction and death. SERCA2a is a calcium ion pump found in heart tissue. The goal of this research program is to identify new medicines that increase the amount of SERCA2a in the heart, resulting in improvements in calcium ion transport and heart function. Using its proprietary drug discovery technology, PTC has identified early stage compounds that increase levels of SERCA2a protein. The new PPMD grant will support medicinal chemistry efforts to improve the chemical, biological and pharmaceutical properties of these early molecules with the goal of identifying a development candidate for clinical studies.

"We are pleased to continue and expand our collaboration with PPMD in our ongoing effort to bring new treatments to Duchenne and Becker muscular dystrophy patients," stated Stuart W. Peltz, Ph.D., President & CEO of PTC Therapeutics. "Our commitment to DBMD patients started more than a decade ago when we initiated our nonsense suppression program. Those efforts led to ataluren, which has the potential to be the first therapy to treat the underlying cause of nonsense mutation DBMD. Our experience developing ataluren will be beneficial to the SERCA2a and our other neuromuscular programs as we move forward in development. Our goal is to develop safe and effective treatments for all patients with DBMD."

ABOUT PARENT PROJECT MUSCULAR DYSTROPHY
Parent Project Muscular Dystrophy (PPMD) is a national not-for-profit organization founded in 1994 by parents of children with Duchenne and Becker muscular dystrophy. The organization's mission is to improve the treatment, quality of life and long-term outlook for all individuals affected by Duchenne muscular dystrophy through research, advocacy, education and compassion. PPMD is headquartered in Middletown, Ohio with offices in Hackensack, New Jersey. For more information, visit www.parentprojectmd.org.

ABOUT PTC THERAPEUTICS, INC.
PTC is a biopharmaceutical company focused on the discovery, development and commercialization of orally administered small-molecule drugs that target post-transcriptional control processes. Post-transcriptional control processes regulate the rate and timing of protein production and are of central importance to proper cellular function. PTC's internally discovered pipeline addresses multiple therapeutic areas, including rare genetic disorders, oncology and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and is the basis for collaborations with leading biopharmaceutical companies. For more information, visit the company's web site at www.ptcbio.com.

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