

FOR IMMEDIATE RELEASE

## **PTC THERAPEUTICS AWARDED GRANT FROM THE PARENT PROJECT MUSCULAR DYSTROPHY FOR DRUG DISCOVERY RESEARCH**

**SOUTH PLAINFIELD, NJ – September 2, 2004** – PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development, and commercialization of small molecule drugs targeting post-transcriptional control mechanisms, announced today that it has been awarded a grant of \$1 million from the Parent Project Muscular Dystrophy (PPMD) to discover novel agents to treat Duchenne muscular dystrophy (DMD). PTC and PPMD have jointly selected target genes of potential therapeutic relevance to DMD. PTC will apply its proprietary GEMS (Gene Expression Modulation by Small-molecules) technology to identify small molecule compounds that can be developed into novel drugs for the treatment of DMD.

“This is the first award PPMD has granted to a biopharmaceutical company and we are proud to have PTC on our team to fight this debilitating disorder,” said Pat Furlong, Executive Director, PPMD. “We look forward to working with PTC as they advance their research and hope that this project will lead to potential new treatments for DMD.”

DMD is a disease characterized by muscle weakness and progressive loss of muscle function that results in serious disability and shortened life expectancy. DMD is perhaps the most prevalent of the muscular dystrophies and is the most common genetic disorder diagnosed during childhood today. It is estimated that approximately 20,000 boys worldwide are born with DMD annually.

“We are extremely pleased to have the strong support of PPMD. This collaboration allows us to investigate potential therapies for DMD beyond the scope of our clinical compound, PTC124, which targets nonsense mutations that cause genetic disorders such as DMD,” said Stuart Peltz, Ph.D., President and CEO of PTC Therapeutics. “PTC is committed to finding new treatments for diseases like DMD in which there is a high unmet medical need.”

## **ABOUT PTC THERAPEUTICS, INC.**

PTC Therapeutics, Inc. is a biopharmaceutical company focused on the discovery, development, and commercialization of small molecule drugs targeting post-transcriptional control mechanisms. PTC's compounds regulate gene expression by selectively modulating how RNA is used to produce proteins. Post-transcriptional control processes are the sequence of events in the cell that ultimately regulate how much, and when, each particular protein is produced. By applying this approach, PTC has advanced its drug discovery programs rapidly from targets to preclinical and clinical drug candidates, building a robust pipeline across genetic disorders, oncology, and infectious diseases. For more information please visit [www.ptcbio.com](http://www.ptcbio.com).

## **ABOUT GEMS**

GEMS is PTC's proprietary technology that exploits the regulatory mechanisms found in the untranslated regions of messenger RNA for the rapid identification of small molecule drugs that can treat diseases by selectively increasing or decreasing the expression of key proteins. GEMS is broadly applicable to targets across multiple therapeutic areas including newly identified or previously intractable targets. The ability to identify orally bioavailable molecules that modulate the expression of specific proteins in cells also allows PTC to pursue targets currently only addressed by protein drugs. PTC's preclinical programs in infectious diseases and oncology have validated the applicability of GEMS to identify orally bioavailable compounds that selectively modulate protein expression.

## **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. Duchenne muscular dystrophy is the most common lethal genetic disorder diagnosed during early childhood, affecting approximately 1 out of every 3,500 boys and 20,000 babies born each year. 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, education, advocacy and compassion. PPMD is the largest grassroots organization in the U.S. entirely focused on Duchenne muscular dystrophy. It is headquartered in Middletown, Ohio with offices in Fort Lee, New Jersey. For more information please visit [www.parentprojectmd.org](http://www.parentprojectmd.org).

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