FOR IMMEDIATE RELEASE

PTC THERAPEUTICS RECEIVES ORPHAN DRUG DESIGNATION FOR PTC124 FOR THE TREATMENT OF DUCHENNE MUSCULAR DYSTROPHY

SOUTH PLAINFIELD, NJ – January 27, 2005 - PTC Therapeutics, Inc. (PTC), a biopharmaceutical company focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms, today announced that the United States Food and Drug Administration (FDA) has granted orphan drug designation to PTC124 for the treatment of Duchenne muscular dystrophy due to a nonsense mutation in the dystrophin gene. Of the approximately 10,000 children with Duchenne muscular dystrophy in the United States, it is estimated that 15% are afflicted with the disease due to a nonsense mutation. PTC124 is a novel small-molecule drug currently being evaluated in Phase 1 clinical trials involving healthy volunteers.

Orphan drug designation is granted by the FDA Office of Orphan Products Development to promote the development of products that may offer therapeutic benefits for diseases with a prevalence of fewer than 200,000 individuals per year in the United States. Orphan drug designation provides opportunities for grant funding towards clinical trial costs, tax advantages, FDA user-fee benefits, and seven years of market exclusivity in the United States following drug approval by the FDA.

“Current treatments for Duchenne muscular dystrophy consist primarily of steroids and physical supportive interventions such as braces, wheelchairs, and ventilators. These measures may temporarily slow the progression of the disease or provide palliative benefit,” said Langdon Miller, MD, Chief Medical Officer of PTC Therapeutics. “By contrast, PTC124 has the potential to address the underlying cause of the disease for patients with a nonsense mutation.”

Preliminary results from Phase 1 studies have confirmed that PTC124 is orally bioavailable and is generally well tolerated. Final results from the Phase 1 trials are expected in early 2005. Pending FDA concurrence, PTC expects to advance PTC124 into Phase 2 studies in patients with nonsense-mutation-mediated Duchenne muscular dystrophy in the first half of 2005. A Phase 2 program in cystic fibrosis is also planned for the first half of 2005.

“We are very proud of receiving orphan drug designation for PTC124 in the treatment of Duchenne muscular dystrophy due to a nonsense mutation. In December of 2004 we were granted orphan drug designation for the treatment of cystic fibrosis due to a nonsense mutation in the cystic fibrosis
transmembrane regulator (CFTR). The new designation is yet another achievement in the development of PTC124, a drug with the potential to treat multiple genetic disorders by overcoming a specific type of mutation. We are committed to the development of PTC124 and grateful to have the recognition of the Office of Orphan Products Development,” said Stuart Peltz, Ph.D., President and CEO of PTC Therapeutics.

About PTC Therapeutics, Inc.
PTC is a biopharmaceutical company focused on the discovery, development, and commercialization of small-molecule drugs targeting post-transcriptional control mechanisms. Post-transcriptional control processes are the sequence of events in the cell that ultimately regulate the rate and timing of all protein production. PTC’s compounds alter these processes by selectively modulating how RNA is used to produce proteins. 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.

About PTC124
PTC124 represents a first-in-class, orally delivered investigational new drug for the treatment of genetic disorders due to nonsense mutations. Nonsense mutations are single-point alterations in the genetic code that prematurely halt the translation process, producing a shortened, non-functional protein. PTC124 allows the cellular machinery to bypass the nonsense mutation and continue the translation process, restoring the production of full-length, functional proteins. PTC124 has demonstrated the ability to restore full-length functional protein in genetic disease models harboring nonsense mutations. PTC124 represents a unique opportunity to use a single small-molecule drug to address chronic and life-threatening diseases of high unmet medical need. It is estimated that 10% of the cases of cystic fibrosis (CF) and 15% of the cases of Duchenne muscular dystrophy (DMD) are due to nonsense mutations. PTC has catalogued over 1,800 distinct genetic disorders where nonsense mutations are the cause of the disease in an appreciable percentage of patients. In addition to CF and DMD, other potential indications under consideration for PTC124 include hemophilia, neurofibromatosis, retinitis pigmentosa, bullous skin diseases, and lysosomal storage disorders.
About Duchenne Muscular Dystrophy

Duchenne muscular dystrophy (DMD) is a progressive muscle disorder that causes the loss of both muscle function and independence. DMD is perhaps the most prevalent of the muscular dystrophies and is the most common lethal genetic disorder diagnosed during childhood today. Each year, approximately 20,000 children worldwide are born with DMD (one of every 3,500 male children). More information regarding DMD is available through the Muscular Dystrophy Association (http://www.mdausa.org) and the Parent Project Muscular Dystrophy (http://www.parentprojectmd.org).

FOR MORE INFORMATION:

Kerri Donnelly                     Robert Stanislaro
PTC Therapeutics, Inc.             Noonan/Russo
(908) 222-7000, x112                (212) 845-4268
kdonnelly@ptcbio.com               robert.stanislaro@eurorscg.com