PTC THERAPEUTICS INITIATES PHASE 2 CLINICAL TRIAL OF ATALUREN (PTC124®) IN PATIENTS WITH HEMOPHILIA

- First potential oral therapy for hemophilia -

- Third proof-of-concept indication for ataluren -

SOUTH PLAINFIELD, NJ - October 26, 2009 - PTC Therapeutics, Inc. (PTC) today announced it is expanding the development of ataluren, an investigational new drug, to a third indication with the initiation of a Phase 2a clinical trial in nonsense mutation hemophilia A (nmHA) and hemophilia B (nmHB). Hemophilia is a rare and debilitating genetic disorder that causes loss of blot clotting proteins and can lead to serious, recurrent bleeding episodes. It is estimated that nonsense mutations account for about 10 to 30 percent of all hemophilia cases.

The purpose of this multi-center, open-label trial is to evaluate the activity, safety, and pharmacokinetics of ataluren in approximately 24 adult male patients with nmHA or nmHB. The primary goal of the trial, which consists of two 14-day treatments at two different dose levels, is to determine if ataluren can safely increase Factor VIII (FVIII) and Factor IX (FIX) activity levels.

"We are excited about the initiation of this proof-of-concept trial of ataluren in patients with nonsense mutation hemophilia, a more severe form of the disorder with a great need for alternative treatment options," said Langdon Miller, M.D., Chief Medical Officer of PTC Therapeutics. "The data from this study will add to the growing body of data that we are compiling with our ongoing studies in Duchenne/Becker muscular dystrophy and cystic fibrosis."

Patients with hemophilia A and B lack adequate levels of FVIII or FIX proteins due to mutations in their genetic code. The lack of FVIII and FIX proteins leads to improper blood clotting and excessive bleeding. In nmHA and nmHB, an interruption in the genetic code - called a nonsense mutation- prematurely halts the synthesis of FVIII and FIX, causing the protein to be short and non-functioning. A genetic test is required to determine if a patient's disease is caused by a nonsense mutation. Patients with nmHA or nmHB typically produce little or no functional FVIII or FIX (less than one percent of normal plasma levels) and experience more severe symptoms than patients whose disease is caused by other mutations. Such low levels can result in spontaneous bleeding episodes that cause destruction of the joints, damage to other tissues and increased risk for intracranial bleeding, or bleeding into the brain, which may cause severe neurological symptoms, including stroke. Current therapies require repeated, frequent intravenous infusions to maintain FVIII or FIX levels. Ataluren administered orally is designed to address the underlying cause of nmHA and nmHB by promoting restoration of functional FVIII and FIX enzymatic protein.

"We are very pleased to be part of this groundbreaking trial," stated Kathy High, M.D., principal investigator at The Children's Hospital of Philadelphia. "HA and HB are chronic, disabling and potentially life-threatening genetic disorders, and there is a significant need for new strategies for preventing bleeding complications. For patients with nonsense mutation hemophilia, even a small increase in FVIII or FIX could be very beneficial. As an oral therapy, ataluren has the potential to offer a noninvasive treatment option."

"This proof-of-concept trial in a third indication represents a major step forward in the development of ataluren as a potential treatment for nonsense mutation genetic disorders," stated Stuart W. Peltz, M.D., president and CEO of PTC Therapeutics. "We are excited by the clinical progression of ataluren. We recently initiated a pivotal trial in nonsense mutation cystic fibrosis and expect to complete the pivotal trial in nonsense mutation Duchenne and Becker muscular dystrophy by the end of the year with results expected to be announced in the first half of 2010."

ABOUT HEMOPHILIA
Hemophilia is an inherited genetic disorder that results in abnormal bleeding with longer periods of bleeding than normal. It occurs when one of the proteins needed to form blood clots is absent or reduced. There are two types of hemophilia, hemophilia A (HA) and hemophilia B (HB). HA results from Factor VIII deficiency, and HB results from Factor IX deficiency. According to the National Hemophilia Foundation, HA is the most common form of hemophilia and affects approximately 18,000 males in the US, while HB afflicts about 3,300 males in the US and the worldwide incidence of hemophilia is estimated at more than 400,000 people. In about one-third of hemophilia cases, there is no family history of the disorder and instead, the condition is caused by a spontaneous gene mutation. Approximately 10 to 30 percent of patients with hemophilia have the disease to a nonsense mutation. More information regarding hemophilia is available through the National Hemophilia Foundation (www.hemophilia.org).

ABOUT ATALUREN (PTC124®)
Ataluren, an orally-administered small-molecule, is the first investigational new drug designed to restore the formation of a functioning protein in patients with genetic disorders due to a nonsense mutation. A nonsense mutation is an alteration in the genetic code that prematurely halts the synthesis of an essential protein. Ataluren is currently being investigated for use in patients with nonsense mutation cystic fibrosis (nmCF) and nonsense mutation Duchenne and Becker muscular dystrophy (nmDMD/BMD). Ataluren has been granted orphan drug status for the treatment of nmCF and nmDMD/BMD by the U.S. Food and Drug Administration (FDA) and the European Commission. The FDA has also granted ataluren Subpart E designation for expedited development, evaluation, and marketing. The development of ataluren has been supported by the Cystic Fibrosis Foundation Therapeutics Inc. (the nonprofit affiliate of the Cystic Fibrosis Foundation), the FDA Office of Orphan Products Development, the Muscular Dystrophy Association, Parent Project Muscular Dystrophy, and the National Center for Research Resources.

COLLABORATION WITH GENZYME
PTC Therapeutics has an exclusive collaboration with Genzyme Corporation for the development and commercialization of ataluren. PTC Therapeutics will commercialize ataluren in the United States and Canada, while Genzyme will commercialize the product in other regions of the world.

ABOUT PTC THERAPEUTICS
PTC is a biopharmaceutical company focused on the discovery, development and commercialization of oral 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 genetic disorders, oncology, and infectious diseases. PTC has developed proprietary technologies that it applies in its drug discovery activities and are the basis for collaborations with leading biopharmaceutical companies such as Celgene, Genzyme, Gilead, Pfizer, Roche and Schering-Plough. For more information, visit the company's web site at www.ptcbio.com.

FOR MORE INFORMATION:
INVESTORS AND MEDIA
Jane Baj
PTC Therapeutics, Inc.
(908) 912-9167
jbaj@ptcbio.com

Sheryl Seapy
Pure Communications
(949) 608-0841
sheryl@purecommunicationsinc.com

PATIENTS, PATIENTS' FAMILIES,
INVESTIGATORS AND PATIENT ORGANIZATIONS
Diane Goetz
PTC Therapeutics, Inc.
(908) 912-9256
patientinfo@ptcbio.com