PTC Therapeutics Completes Enrollment of Phase 3 Trial of Ataluren in Patients with Cystic Fibrosis

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SOUTH PLAINFIELD, N.J., Dec. 21, 2010 /PRNewswire/ -- PTC Therapeutics, Inc. announced today that it has completed enrollment of a Phase 3 clinical trial of ataluren, an investigational new drug, in patients with nonsense mutation cystic fibrosis (nmCF).

(Logo: http://photos.prnewswire.com/prnh/20010919/PTCLOGO [1])

The 48-week study is designed to determine whether ataluren can improve lung function in patients with nmCF. The trial has enrolled 238 patients at 36 sites in North America, Europe and Israel. Patients who complete the treatment phase of the Phase 3 trial are eligible to participate in a 48-week, open-label extension study, which has begun enrolling patients.

"The enrollment of this trial represents an important step forward in our efforts to develop disease-modifying treatments that advance the standard of care in CF and improve quality of life for CF patients," stated Michael Konstan, MD, Chairman, Department of Pediatrics at Rainbow Babies and Children's Hospital in Cleveland, Ohio. Dr. Eitan Kerem, Head, Department of Pediatrics and CF Center, Hadasash University Hospital, Jerusalem, Israel added, "Despite significant advances in the 21 years since the identification of the disease-causing gene, cystic fibrosis remains a debilitating and life-threatening disorder and available therapies focus only on alleviating symptoms. Ataluren couples a patient's genetic diagnosis with a mutation-specific therapeutic approach designed to address the underlying cause of the disease."

Ataluren is a protein restoration therapy designed to enable the formation of full-length, functional cystic fibrosis transmembrane regulator (CFTR) protein in patients with cystic fibrosis due to a nonsense mutation. CFTR is a critical protein lacking in CF patients. Nonsense mutations are categorized as Class I mutations that result in little or no production of the CFTR protein. CF patients with Class I mutations typi
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