



AMICUS THERAPEUTICS COMMENCES PHASE 1 CLINICAL TRIALS FOR AT2220 FOR POMPE DISEASE

Cranbury, NJ, DECEMBER 14, 2006 – Amicus Therapeutics, a biopharmaceutical company developing small molecule, orally-administered pharmacological chaperones for the treatment of human genetic diseases, today announced that it has commenced Phase 1 clinical trials for AT2220 for the treatment of Pompe disease, following acceptance of an investigational new drug application (IND) by the U.S. Food and Drug Administration (FDA).

Pompe disease, also known as glycogen storage disease type II or acid maltase deficiency, is a relatively rare lysosomal storage disorder caused by an inherited mutation in the lysosomal enzyme α -glucosidase (GAA). GAA is normally made in the endoplasmic reticulum where it is properly folded and subsequently trafficked to the lysosome where it catalyzes the breakdown of glycogen. In many Pompe patients, a genetic mutation alters the structure and stability of GAA which results in reduced levels of enzyme in the lysosome and reduced cellular activity. The deficiency in GAA activity leads to excessive glycogen accumulation in the cells of various tissues, especially in heart and skeletal muscle.

AT2220 is a small molecule designed to act as a pharmacological chaperone that specifically binds, stabilizes, and facilitates the proper folding and trafficking of GAA to the lysosome, where it can perform its normal function. AT2220 has been shown to increase GAA activity in cell lines derived from Pompe patients and in transfected cells expressing misfolded forms of GAA.

"We are very pleased to see continued progress in the fight against Pompe disease," says Dr. Sharon Hesterlee, Vice President of Translational Research at the Muscular Dystrophy Association (MDA). "We look forward to exploring the opportunities to work with Amicus as this new potential treatment option for individuals and families with Pompe disease is evaluated through human clinical trials."

"AT2220 for Pompe disease is the third Amicus product to enter clinical trials," says Donald Hayden, Amicus interim President and CEO. "This accomplishment further demonstrates the company's progress in developing new potential treatments for important diseases using pharmacological chaperone technology."

The company's lead compound, Amigal™ (migalastat hydrochloride), is in Phase 2 clinical trials for Fabry disease and AT2101 is in Phase 1 clinical trials for the treatment of Gaucher disease.

About Pompe Disease Pompe disease affects an estimated 5,000-10,000 patients worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression. The early onset form of the disease is the most severe, progresses most rapidly, and is characterized by musculoskeletal, pulmonary, gastrointestinal, and cardiac symptoms that usually lead to death from cardio-respiratory failure between 1 and 2 years of age. The late onset form of the disease begins between childhood and adulthood and has a slower rate of progression that is characterized by musculoskeletal and pulmonary symptoms that usually lead to progressive weakness and respiratory insufficiency.

About Amicus Therapeutics

Amicus Therapeutics is a biopharmaceutical company developing novel, oral therapeutics known as pharmacological chaperones for the treatment of a range of human genetic diseases. Pharmacological chaperone technology involves the use of small molecules to restore or improve biological activity in cells by selectively binding to misfolded proteins caused by genetic mutations. Amicus is initially targeting lysosomal storage disorders, which are severe, chronic genetic diseases with unmet medical needs. Amicus' is currently conducting Phase 2 clinical trials for its lead compound, Amigal™, for Fabry disease, and is conducting Phase 1 clinical trials of AT2101 for Gaucher disease and AT2220 for Pompe disease.