



AMICUS THERAPEUTICS INITIATES PHASE I TRIAL WITH LEAD COMPOUND AT1001 FOR THE TREATMENT OF FABRY DISEASE

New Brunswick, NJ - Amicus Therapeutics, Inc., an emerging drug development company focused on the development of a novel therapeutic approach to the treatment of human genetic disorders, with an initial focus on lysosomal storage diseases, today announced that it has initiated a Phase I clinical trial with its first clinical candidate, AT1001, for the treatment of Fabry disease.

"Fabry disease is a lysosomal storage disorder caused by a deficiency of alpha-galactosidase A, an enzyme involved in the biodegradation of glycolipids. This is a chronic genetic condition that is responsible for a host of serious ailments, including severe pain, heart disease, and kidney failure," said David Palling, Ph.D., Vice President of Pre-Clinical Development of Amicus Therapeutics, Inc. "AT1001 is designed to be an orally active, small molecule drug that aims to enhance alpha-galactosidase A activity in patients suffering from Fabry disease, and could provide a novel treatment that is more convenient to use, and is potentially better suited for long-term administration, compared with current therapies."

Amicus received orphan drug designation from the U.S. Food & Drug Administration for AT1001 in March, 2004.

The Phase I trial will enroll healthy volunteers in a dose-escalating study designed to evaluate the safety and pharmacokinetics of AT1001. In pre-clinical studies, AT1001 was shown to have activity consistent with the pharmacological chaperone mechanism of action, excellent oral bioavailability and favorable toxicological and pharmacokinetic profiles.

"This has been a very eventful and productive year for Amicus. The start of this clinical trial is another significant milestone and marks our transition into a product development company," said Norman Hardman, Ph.D., Chief Executive Officer of Amicus Therapeutics, Inc. "The completion of our \$31 million Series B fundraising in May has provided sufficient financial resources to advance this first product a considerable way through clinical development and to progress our earlier stage pipeline as well. We are very much looking forward to seeing the results of the initial clinical studies with AT1001, as well as advancing the pre-clinical development of additional product candidates that address other serious lysosomal storage disorders, such as Gaucher disease, and other genetic diseases."

About Fabry Disease

Fabry disease is a lysosomal storage disease caused by a deficiency of alpha-galactosidase A. Patients with classic Fabry disease, of which there are approximately 5,000 people worldwide, have early-onset symptoms, including neuropathic pain, heart disease and kidney disease. Late-onset Fabry disease is characterized by heart and renal involvement in patients who first present to the clinic later in life, typically in early middle-age. Fabrazyme (marketed by Genzyme Corporation) and Replagal (marketed by Transkaryotic Therapies, Inc.) are enzyme replacement therapies that aim to replace the alpha-galactosidase A enzyme that is diminished or absent in Fabry patients. In contrast, AT1001 is designed to provide a small molecule, oral therapy to enhance the patient's own alpha-galactosidase A activity.

About Amicus Therapeutics

Founded in April 2002, Amicus is focused on the development of orally-active, small molecule drugs capable of restoring normal function to mutant proteins. Amicus was founded to capitalize on the discovery that many diseases of genetic origin are caused by missense mutations and other rescuable mutations that result in the misfolding of a protein or enzyme. These misfolded mutant proteins become targeted for degradation before reaching their normal site of action, leading to the disease phenotype. Pharmacological Chaperones are designed to help the mutant protein fold correctly into its normal 3-dimensional conformation, restoring the normal processing and transport of the protein and rescuing its intrinsic biological activity and function.

Amicus technology is based on research conducted by Jian-Qiang Fan, Ph.D., Assistant Professor, Department of Human Genetics at Mount Sinai School of Medicine, and a founder of Amicus. Amicus' pharmacological chaperone approach has the potential to be applied to a wide range of genetic disorders. The Company's initial focus is on lysosomal storage disorders and its first compound, AT1001 for Fabry disease, is in Phase I clinical trials. Amicus currently has 11 employees and is headquartered at the New Jersey Technology Center in North Brunswick, New Jersey. Additional information about the Company can be found at www.amicustherapeutics.com