AMICUS THERAPEUTICS RECEIVES U.S. ORPHAN DRUG DESIGNATION FOR AT1001 IN 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 the U.S. Food & Drug Administration (FDA) has granted Amicus orphan-drug status for the company's first clinical candidate, AT1001, for the treatment of Fabry disease.

"Orphan-drug designation will play an important role in the commercialization strategy for AT1001 as we move the product forward, and we continue to be very pleased with the progress of AT1001 through the preclinical development phase," said Norman Hardman, Ph.D., Chief Executive Officer of Amicus Therapeutics, Inc. "AT1001 is a small molecule drug that could potentially provide a novel oral therapy for the treatment of Fabry disease, and one that has possible advantages for long-term treatment when compared with currently available therapies, providing significant benefits to patients suffering from this 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.

Orphan Drug designation allows special incentives for sponsors planning to test a product for use in a rare disease or condition. These incentives include eligibility for research grants, certain tax benefits, protocol assistance and possible exemptions or reductions of certain regulatory fees during development or at the time of application for marketing approval. Once approved, the product may qualify for seven years of marketing exclusivity in the U.S.

About Amicus Therapeutics

Amicus was established in April 2002 with seed financing provided by CHL Medical Partners II, a venture capital fund managed by Collinson, Howe & Lennox, and 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/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 late-stage preclinical development. Amicus currently has 10 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.