

## AMICUS THERAPEUTICS' AT2101 GRANTED ORPHAN DRUG DESIGNATION FOR THE TREATMENT OF GAUCHER DISEASE

Cranbury, NJ, February 15, 2006 - Amicus Therapeutics, a biopharmaceutical company developing small molecule, orally-active pharmacological chaperones for the treatment of human genetic diseases, today announced that AT2101 has received orphan drug designation from the U.S. Food and Drug Administration. AT2101 is an experimental, oral therapy for the treatment of Gaucher disease, a lysosomal storage disorder resulting from an enzyme deficiency that can cause damage to the liver, spleen, bone marrow and in some cases, the central nervous system. Gaucher disease affects approximately 10,000 people in the developed world. Orphan drug designation provides extended marketing rights and other incentives to support and encourage development of drugs that affect fewer than 200,000 people annually in the United States.

AT2101 acts as a pharmacological chaperone that binds to glucocerebrosidase (GCase), the enzyme involved in Gaucher disease. In most Gaucher patients, the GCase deficiency is caused by a missense mutation, which results in the misfolding and degradation of the protein. As a pharmacological chaperone, AT2101 binds specifically to the misfolded protein, which in turn restores proper structure, trafficking and biological activity.

AT2101 is expected to enter clinical studies in the first half of this year.

Amicus' lead compound Amigal™ (migalastat hydrochloride) is a pharmacological chaperone in Phase II clinical trials for Fabry disease, and the company has a growing pipeline of other products for a range of genetic diseases.

## **About Amicus Therapeutics**

Amicus Therapeutics is a biopharmaceutical company based in Cranbury, New Jersey, developing small molecule, orally-active pharmacological chaperones for the treatment of human genetic diseases. Many of these diseases are the result of missense and other genetic errors that cause the misfolding and degradation or accumulation of a particular protein. Amicus' products act as pharmacological chaperones that selectively bind and "rescue" the misfolded target protein to restore its proper conformation and natural function, which in turn restores the function of the affected cells. Amicus' lead compound Amigal™ is in Phase II clinical trials for Fabry disease. The company has an active drug development program for Gaucher disease and is developing programs for a range of genetic diseases.