



## **AMICUS THERAPEUTICS COMPLETES PHASE 2 ENROLLMENT OF AMIGAL™ FOR FABRY DISEASE**

Cranbury, NJ, March 16, 2007 – Amicus Therapeutics, a biopharmaceutical company developing small-molecule, orally administered pharmacological chaperones for the treatment of human genetic diseases, announced today that enrollment has been completed for all ongoing Phase 2 clinical trials of Amigal™ (migalastat hydrochloride, AT1001). Amigal is in development for the treatment of Fabry disease.

The four open-label, multi-national Phase 2 trials are examining various dose levels and frequencies of Amigal in men and women with Fabry disease. The primary objective of the studies is to evaluate the safety and tolerability of treatment with Amigal. The secondary objective is to evaluate certain pharmacodynamic measures of treatment, including effects on  $\alpha$ -galactosidase A ( $\alpha$ -GAL) and globotriaosylceramide (GL-3) levels in various cells and tissues of disease. An additional objective is the preliminary assessment of cardiac, renal and central nervous system function. The results of these clinical trials are expected to be available by the end of 2007.

Amigal is designed to selectively bind to and stabilize  $\alpha$ -GAL, the enzyme deficient in Fabry disease. This deficiency leads to lysosomal accumulation of GL-3, which is believed to cause the various symptoms of Fabry disease. Amigal facilitates proper trafficking of the enzyme to the lysosomes, the compartments in the cell where it is needed to break down GL-3.

### **About Fabry Disease**

Fabry disease is a lysosomal storage disorder caused by inherited genetic mutations in the GLA gene, which result in deficient activity of the enzyme  $\alpha$ -galactosidase A ( $\alpha$ -GAL). Deficient  $\alpha$ -GAL activity leads to lysosomal accumulation of globotriaosylceramide (GL-3), which is believed to cause the various symptoms of Fabry disease, including pain, kidney failure and increased risk of heart attack and stroke. Fabry disease is estimated to affect approximately 5,000 to 10,000 people in the developed world, but recent evidence suggests that the disease may be significantly underdiagnosed. The U.S. Food and Drug Administration's Office of Orphan Products Development has granted orphan designation for Amigal in the United States, and the European Commission has designated Amigal as an orphan medicinal product in the European Union.

### **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 that selectively bind to and stabilize proteins in cells, leading to improved protein folding and trafficking, and increased activity. 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 two lead compounds, Amigal™ for Fabry disease, and AT2101 for Gaucher disease. The company is currently conducting Phase 1 trials with AT2220 for the treatment of Pompe disease.