



## **AMICUS THERAPEUTICS PRESENTS DATA FROM PRECLINICAL AND PHASE 1 STUDIES OF AMIGAL**

### **Studies in Mice Show Reduction of Enzyme Substrate**

Cranbury, NJ, March 19, 2007 – Amicus Therapeutics, a biopharmaceutical company developing small-molecule, orally administered pharmacological chaperones for the treatment of human genetic diseases, announced today that the Company will present results from studies of Amigal™ (migalastat hydrochloride, AT1001), Amicus' compound in development for the treatment of Fabry disease, at the American College of Medical Genetics (ACMG) Annual Meeting from March 21-25 in Nashville, TN. This presentation will include the first data from the Company demonstrating the reduction of globotriaosylceramide (GL-3), the lipid substrate that accumulates in Fabry disease, after oral administration of a pharmacological chaperone. These data were not available at the time of abstract submission but will be included in the presentation at the meeting.

Amigal is designed to selectively bind to and stabilize  $\alpha$ -galactosidase A ( $\alpha$ -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.

At the ACMG meeting, Amicus scientists will present data from several studies that examined the in vitro and in vivo effects of Amigal in cell lines, mice and healthy volunteers. Among the key findings:

- In vitro exposure to Amigal increased the level of  $\alpha$ -GAL in cells derived from healthy volunteers and from Fabry patients.
- Oral administration of Amigal resulted in a dose-dependent increase in  $\alpha$ -GAL levels in various tissues of normal mice and Fabry mice genetically modified to produce  $\alpha$ -GAL with a human missense mutation.
- Oral administration of Amigal to healthy volunteers in a Phase 1 clinical study resulted in a dose-dependent increase of  $\alpha$ -GAL levels in white blood cells.
- Oral administration of Amigal significantly decreased the level of GL-3 in the skin and heart of Fabry mice and showed a trend towards reduction in the kidney.

#### **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.