



## **Amicus Therapeutics Presents Positive Results From Phase 1 Clinical Studies of Plicera (TM) for Gaucher Disease**

### **Phase 2 Clinical Trials Initiated**

CRANBURY, N.J., March 21, 2007 /PRNewswire via COMTEX News Network/ -- Amicus Therapeutics, a biopharmaceutical company developing small molecule, orally-administered pharmacological chaperones for the treatment of a range of human genetic diseases, announced today that it will present positive results from its recently completed Phase 1 clinical studies of Plicera(TM) (isofagomine tartrate, AT2101) for Gaucher disease at the American College of Medical Genetics (ACMG) Annual Meeting on March 21-25 in Nashville, TN. The Phase 1 results show that Plicera was well-tolerated and that oral administration resulted in a significant elevation of target enzyme levels in healthy volunteers. Based on these results, Amicus announced today the initiation of two Phase 2 clinical trials of Plicera for Gaucher disease.

Plicera is designed to selectively bind to and stabilize GCase, the enzyme deficient in Gaucher disease. This deficiency leads to lysosomal accumulation of glucocerebroside inside certain cells, which is believed to cause the various symptoms of Gaucher disease. Plicera facilitates proper trafficking of the enzyme to the lysosomes, the compartments in the cell where it is needed to break down glucocerebroside.

Phase 1 Plicera data being presented at ACMG

Two double-blind, placebo-controlled, dose escalation Phase 1 studies in healthy volunteers were completed. These studies were designed to evaluate the safety, tolerability and pharmacokinetics of Plicera. In the first study, 36 subjects received a single dose of one of five dose levels of Plicera. The second study was a multiple-dose study in which 18 subjects received one of three dose levels of Plicera once daily for 7 consecutive days. In both studies, Plicera was safe and well tolerated at all doses. There were no serious adverse events and no subjects withdrew or discontinued due to an adverse event. In the multiple-dose study, a dose-dependent increase in GCase levels was observed in white blood cells from healthy volunteers receiving Plicera.

Phase 2 Clinical Trials of Plicera in Gaucher Disease

Based on the Phase 1 results, Amicus has initiated two Phase 2 clinical trials of Plicera for Gaucher disease. One is a 4-week study designed to evaluate the safety and pharmacodynamic effects of Plicera in Type I Gaucher patients who will discontinue enzyme replacement therapy for the duration of the study. The second is a 6-month study designed to evaluate the safety of Plicera and its effect on parameters that are commonly abnormal in Gaucher disease. This study will be conducted in Type I Gaucher patients who have never received enzyme replacement therapy. More information regarding these studies can be found at [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and [www.amicustherapeutics.com](http://www.amicustherapeutics.com).

About Gaucher Disease

Gaucher disease, the most commonly diagnosed lysosomal storage disorder, is caused by inherited genetic mutations in the GBA gene, which result in deficient activity of the enzyme acid beta-glucosidase, also known as glucocerebrosidase (GCase). Deficient GCase activity leads to lysosomal accumulation of glucocerebroside inside certain cells, which is believed to cause the various symptoms of Gaucher disease, including an enlarged liver and spleen, abnormally low levels of red blood cells and platelets and skeletal complications. In some cases there is significant impairment of the central nervous system. Gaucher disease affects an estimated 8,000 to 10,000 people worldwide. The U.S. Food and Drug Administration's Office of Orphan Products Development has granted orphan drug designation for the active ingredient in Plicera in the United States.

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(TM) for Fabry disease, and Plicera(TM) for Gaucher disease. The company is currently conducting Phase 1 trials with AT2220 for the treatment of Pompe disease.

SOURCE Amicus Therapeutics

Matthew Patterson of Amicus Therapeutics, +1-609-662-2000; or Dan Budwick of BMC Communications Group, +1-212-477-9007, Ext. 14

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