



## **Amicus Therapeutics Presents Positive Data from Phase 2 Clinical Trial of Plicera(TM) for Gaucher Disease**

### **Data Support Commencement of Longer-term Phase 2 Switching Study**

CRANBURY, N.J., March 13, 2008 /PRNewswire via COMTEX News Network/ -- 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 positive results from a Phase 2 clinical study of Plicera(TM) (isofagomine tartrate) for Gaucher disease at the American College of Medical Genetics (ACMG) Annual Meeting from March 12-16 in Phoenix, AZ. Results from the fully enrolled Phase 2 trial support the previously reported interim findings that Plicera was generally safe and well tolerated at all doses and increased target enzyme activity levels in a majority of patients.

Phase 2 Plicera data presented at ACMG

The primary objective of this study was to evaluate safety and tolerability of different doses and dosing regimens of Plicera. The secondary objective was to evaluate certain pharmacodynamic measures of treatment, including effects on GCCase (the enzyme deficient in individuals with Gaucher disease) levels in white blood cells.

Thirty patients with Gaucher disease (8 men and 22 women between the ages of 18 and 63) were enrolled, and there were 12 unique alleles represented including the most common N370S and L444P mutations. Patients were on enzyme replacement therapy (ERT) with imiglucerase for an average of 9 years prior to entering the trial, and they temporarily discontinued ERT to receive Plicera for the 4 week duration of the study.

The key findings from the trial were as follows:

- Plicera was generally well-tolerated at all doses evaluated, and no serious adverse events were reported.
- GCCase activity as measured in white blood cells was increased in 20 of the 26 patients with evaluable GCCase data, and 5 of the 6 patients without a clear increase were either in the lowest dose cohort or the cohort dosed least frequently.
- As expected in this short term study, the levels of relevant markers of Gaucher disease including platelet counts, hemoglobin levels, glucocerebroside (substrate) levels, chitotriosidase activity and pulmonary activation-related chemokine (PARC) levels were maintained.

"These data give us great confidence in moving our Gaucher program forward," said John F. Crowley, President and CEO of Amicus Therapeutics. "In addition to a 6-month Phase 2 study in individuals naive to ERT, which is currently underway, we plan to initiate a longer-term study in individuals switching from enzyme replacement therapy to Plicera in the second half of this year."

As of November 2007, Plicera is being developed in partnership with Shire Human Genetic Therapies (HGT), a business unit of Shire plc, which is focused on genetic diseases.

#### About Gaucher Disease

Gaucher disease is a lysosomal storage disorder caused by inherited genetic mutations in the GBA gene, which result in deficient activity of the enzyme acid beta-glucosidase, also known as glucocerebrosidase (GCCase). Deficient GCCase 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 is estimated to affect approximately 10,000 people in the developed world. 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 and the European Commission has designated Plicera 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 has completed Phase 2 clinical trials of Amigal(TM) for the treatment of Fabry disease and is conducting Phase 2 clinical trials of Plicera(TM) for the treatment of Gaucher disease. The Company recently completed Phase I clinical trials of AT2220 for the treatment of Pompe disease.

#### Forward-Looking Statements

Amicus cautions you that statements included in this press release that are not a description of historical facts are "forward-looking statements" within the meaning of Section 21E of the Private Securities Litigation Reform Act of 1995. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "plan," "targets," "likely," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. For example, with respect to statements regarding the potential progress and results of clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the effect of the completion of the Phase 2 clinical trial for Amigal(TM) for the treatment of Fabry disease, the plans for the Phase 3 clinical trial for Amigal(TM), the Phase 2 clinical trials for Plicera(TM) for the treatment of Gaucher disease and the effect of the completion of the Phase 1 clinical trials for AT2220 for the treatment of Pompe disease. Further, the results of earlier clinical trials may not be predictive of future results; and other risks detailed in the public filings of Amicus with the Securities and Exchange Commission. You are cautioned not to place undue reliance on these forward-looking statements, which speak only as of the date hereof. All forward-looking statements are qualified in their entirety by this cautionary statement and Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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