



Amicus Therapeutics Presents Data from Clinical Ex Vivo Response Study and Phase 1 Studies of AT2220

Data Suggest Majority of Pompe Patients May Be Amenable to Chaperone Therapy

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 an ex vivo response study and three Phase 1 studies of AT2220 (1-deoxynojirimycin HCl), Amicus' compound in development for the treatment of Pompe disease, at the American College of Medical Genetics (ACMG) Annual Meeting from March 12-16 in Phoenix, AZ. The results of the ex vivo response study along with the previously reported results of the Phase 1 studies support moving into Phase 2 trials in the first half of 2008.

Ex Vivo Response Study data

Interim data will be presented from an ex vivo response study designed to test the effect of AT2220 on various Pompe mutations. Blood and skin samples were collected from 30 Pompe patients (26 adults, 3 juveniles and 1 infant) with a variety of different mutations in acid alpha-glucosidase (GAA), the target enzyme in Pompe disease. Cells derived from these samples were then tested to determine whether treatment with AT2220 caused an increase in the level of GAA. Of the 26 patients with available data, 24 had cells that showed a dose responsive increase in GAA levels, including 22 patients who had at least 1 copy of the common splice site mutation IVS1-13T>G. It has been reported that more than 80% of Caucasian adult Pompe patients have at least 1 copy of this common splicing mutation.

Phase 1 AT2220 data

A total of 72 healthy volunteers were treated in three double-blind, placebo-controlled, dose escalation Phase 1 studies designed to evaluate the safety, tolerability and pharmacokinetics of AT2220. Across all three studies, AT2220 was shown to be generally safe and well tolerated at all doses. There were no drug-related serious adverse events and no adverse events were considered to be definitely or probably related to study treatment. In the multiple ascending dose studies all possibly-related adverse events were mild in severity and resolved spontaneously.

"We're very encouraged with the results of the ex vivo response study that suggest a majority of Pompe patients may be treatable with our pharmacological chaperone AT2220," said John F. Crowley, President and CEO of Amicus Therapeutics. "Based on these data and the safety data from the Phase 1 studies, we look forward to commencing a Phase 2 clinical trial in Pompe patients the first half of this year."

As of November 2007, AT2220 is being developed in partnership with Shire Human Genetic Therapies (HGT), a business unit of Shire plc, which is focused on genetic diseases.

About Pompe Disease

Pompe disease affects an estimated 5,000-10,000 patients worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression. The early onset form of the disease is the most severe, progresses most rapidly, and is characterized by musculoskeletal, pulmonary, gastrointestinal, and cardiac symptoms that usually lead to death from cardio-respiratory failure between 1 and 2 years of age. The late onset form of the disease begins between childhood and adulthood and has a slower rate of progression that is characterized by musculoskeletal and pulmonary symptoms that usually lead to progressive weakness and respiratory insufficiency. A high majority of patients have the late onset form of the disease. The U.S. Food and Drug Administration's Office of Orphan Products Development has granted orphan drug designation for the active ingredient in AT2220 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 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: 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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