Amicus Therapeutics Begins Phase 2 Clinical Trial of AT2220 in Pompe Disease

CRANBURY, N.J., June 3, 2008 /PRNewswire-FirstCall via COMTEX News Network/ -- Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company developing small molecule, orally-administered pharmacological chaperones for the treatment of human genetic diseases, today announced that it has initiated a Phase 2 clinical trial of AT2220 (1-deoxynojirimycin HCl), for the treatment of Pompe disease. Amicus will conduct the study in adult Pompe patients in clinical centers throughout North America and Europe. AT2220 is the third compound based on Amicus’ pharmacological chaperone technology platform to enter Phase 2 clinical development.

“We look forward to evaluating AT2220 as a potential new oral therapeutic option for patients living with Pompe disease,” said Barry J. Byrne, M.D., Ph.D., professor of pediatrics, molecular genetics and microbiology at the University of Florida in Gainesville and an investigator in the Phase 2 trial.

AT2220 is designed to selectively bind to, stabilize and elevate the cellular activity of acid alpha-glucosidase (GAA), the enzyme deficient in Pompe disease. This deficiency leads to lysosomal accumulation of glycogen inside cells, which is believed to cause the various symptoms of Pompe disease.

Amicus is initiating the multi-national, open-label Phase 2 clinical trial designed to enroll 18 adult patients diagnosed with Pompe disease. The primary objective of the study is to evaluate the safety and tolerability of different dosing regimens of AT2220 over a 12-week period. The study will also explore certain pharmacodynamic and pharmacokinetic measures including the effect of treatment with AT2220 on GAA activity and on glycogen levels in various cells and tissues. Additional objectives include preliminary assessments of pulmonary and skeletal muscle function. Participants who complete the study may be eligible to participate in a voluntary extension study that will further evaluate the effect of AT2220 on these functional parameters.

Additional information about the Phase 2 study will be posted at ClinicalTrials.gov.

Initiation of the Phase 2 study of AT2220 follows completion of an ex vivo response study as well as multiple Phase 1 studies of AT2220 in healthy volunteers. The ex vivo response study was designed to test the effect of AT2220 on various Pompe mutations. Blood and skin samples were collected from 30 Pompe patients (26 adults, three juveniles and one infant) who had a variety of GAA mutations. Cells derived from each patient were then treated with AT2220. Of the 26 patients with available data, 24 had cells that showed a dose responsive increase in GAA activity including 22 patients who had at least one copy of the common splice site mutation IVS1-13T>G. Data from the Phase 1 studies in 72 healthy volunteers demonstrated that AT2220 was generally safe and well tolerated at all doses evaluated with no drug-related serious adverse events.

Amicus is developing AT2220 as part of a strategic collaboration with Shire Human Genetic Therapies (HGT), a business unit of Shire plc, to develop and commercialize Amicus’ three lead pharmacological chaperone compounds for lysosomal storage disorders. Under the agreement, Shire received commercial rights outside of the United States. Amicus retains all U.S. rights.

About Pompe Disease

Pompe disease affects an estimated 5,000-10,000 individuals world-wide 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.

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 potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; our dependence on third parties in the conduct of our clinical studies; further, the results of earlier clinical trials may not be predictive of future results; and other risks detailed in our annual Report on Form 10-K for the year ended December 31, 2007, and our other public filings 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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