



Amicus Therapeutics Announces Plan to Initiate Phase 1 Study of AT2220 for Pompe Disease

CRANBURY, N.J., Sept 30, 2009 /PRNewswire-FirstCall via COMTEX News Network/ -- Amicus Therapeutics (Nasdaq: FOLD) today announced it plans to initiate a Phase 1 study of AT2220 (1-deoxynojirimycin HCl), its investigational drug in development for the treatment of Pompe Disease. The primary objective of this study is to evaluate the pharmacokinetics of AT2220 in muscle tissue in healthy adult subjects. The U.S. Food and Drug Administration (FDA) has agreed to Amicus' proposal for the Phase 1 study and subsequently converted the clinical hold of AT2220 to a partial hold to allow the conduct of this study.

In June 2008, the Company announced the commencement of a Phase 2 clinical trial of AT2220 in adults with Pompe disease based on data from both preclinical and Phase 1 studies. In February 2009, the Company announced it had suspended enrollment after two patients enrolled in the trial experienced serious adverse events that were probably related to treatment with AT2220. The AT2220 Investigational New Drug application (IND) was subsequently placed on clinical hold by the FDA.

The Company has completed a thorough evaluation of all data from these two subjects and has completed additional preclinical studies of AT2220. Based on these data, the Company proposed the planned Phase 1 study to FDA in order to further evaluate the pharmacokinetics of AT2220 in muscle, the key target tissue in Pompe disease. This study will be an open label, single dose study and will commence in the fourth quarter of this year. The Company expects data from this trial to be available in the first quarter of 2010. Based on the results of this study, the Company will determine the appropriate next steps for the program working in close collaboration with the FDA.

One of the recently completed preclinical studies evaluated the effects of various doses and regimens of AT2220 on glycogen reduction in an appropriate transgenic mouse model of Pompe disease. Preliminary results of this study demonstrate consistent glycogen reduction across a range of different tissues, including heart, diaphragm and multiple skeletal muscles. Glycogen is the substrate that accumulates in the cells of patients with Pompe disease, which is believed to result in the clinical symptoms of the disease. The Company expects to present the results of this and other preclinical studies at appropriate scientific conferences in 2010.

Additionally, Amicus continues to be encouraged by the results of preclinical studies designed to evaluate the use of AT2220 in combination with enzyme replacement therapy (ERT). The Company expects to report additional data from these studies at scientific conferences in 2010.

About Pompe Disease

Pompe disease affects an estimated 5,000 to 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 muscle weakness and respiratory insufficiency. A high majority of people with Pompe disease have the late onset form. 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 a strategic collaboration with Shire Human Genetic Therapies, Inc., a wholly-owned subsidiary 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.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of Amicus' candidate drug product, AT2220 (1-deoxynojirimycin HCl), the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, and the timing of updates on the interactions with the FDA on the AT2220 program. 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 goals, progress, timing and outcomes of ongoing discussions with regulatory authorities and the potential goals, progress, timing and results of preclinical studies and 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 inability to reach final agreement with regulatory agencies on the lifting of the clinical hold on the AT2220 program; the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. Additionally, all forward looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2008, 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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