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Amicus Therapeutics Announces Positive Results From Two Phase 1 Clinical Studies of AT2220 for Pompe Disease

CRANBURY, N.J., October 23, 2007 -- Amicus Therapeutics, a biopharmaceutical company developing small molecule, orally-administered pharmacological chaperones for the treatment of a range of human genetic diseases, announced today positive results from two recently completed Phase 1 clinical studies of AT2220 (1-deoxynojirimycin HCl) for Pompe disease. The Phase 1 results show that AT2220 was well-tolerated in healthy volunteers with good oral bioavailability and pharmacokinetic parameters. These results will be presented at the American Society of Human Genetics (ASHG) Annual Meeting on October 23-27 in San Diego, CA.

AT2220 is designed to selectively bind to and stabilize acid α -glucosidase (GAA), the enzyme deficient in Pompe disease. This deficiency leads to lysosomal accumulation of glycogen inside muscle cells, which is believed to cause the various symptoms of Pompe disease. AT2220 facilitates proper trafficking of the enzyme to the lysosomes, the compartments in the cell where it is needed to break down glycogen. AT2220 has been shown to increase GAA levels in cell lines derived from Pompe patients, in transfected cells expressing mutant forms of GAA, and in healthy mice and monkeys.

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 AT2220. In a single ascending dose study, 32 individuals received oral doses of 50, 150, 300, or 600 mg AT2220 or placebo. In a multiple ascending dose study, 24 individuals received oral doses of 50, 150, or 450 mg/day AT2220 or placebo for 7 days. In both studies, AT2220 was generally safe and well-tolerated at all doses and was orally bioavailable with a plasma half-life of 4 to 5 hours. 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 study, all possibly-related adverse events were mild in severity and resolved spontaneously.

The Company expects to initiate a Phase 2 clinical trial of AT2220 for Pompe disease in early 2008. "We are pleased to be able to advance this program forward in development and explore the potential for AT2220 as a new therapeutic option for people living with Pompe disease," said John F. Crowley, President and CEO of Amicus Therapeutics.

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. 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 two product candidates in Phase II clinical trials, Amigal™ for the treatment of Fabry disease and Plicera™ for the treatment of Gaucher disease. The Company has completed Phase 1 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 respective Phase II clinical trials for Amigal™ and Plicera™, and the Phase I clinical trial for AT2220 may not proceed in the timeframes or in the manner Amicus expects or at all. Further, the results of earlier clinical trials may not be predictive

of future results; Amicus and its licensors may not be able to obtain, maintain and successfully enforce adequate patent and other intellectual property protection of its product candidates; 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.

SOURCE Amicus Therapeutics