Amicus Therapeutics Announces FDA Agreement to Commence Phase 2 Study of AT2220 Co-administered with Enzyme Replacement Therapy for Pompe Disease

CRANBURY, N.J., March 8, 2011 /PRNewswire/ -- Amicus Therapeutics (Nasdaq: FOLD) today announced that the U.S. Food and Drug Administration (FDA) has removed the clinical hold for the AT2220 (1-deoxynojirimycin HCl) Investigational New Drug Application (IND). AT2220 is a pharmacological chaperone in development as a treatment for Pompe disease. Based on data provided by Amicus, the FDA agreed with the Company's proposal to resume clinical development of AT2220, starting with a new Phase 2 study designed to evaluate the use of AT2220 when co-administered with enzyme replacement therapy (ERT) in subjects with Pompe disease. The Company expects to commence this study in the first half of 2011 and to report preliminary results in the second half of 2011.

Co-administration of Pharmacological Chaperones with ERT

Amicus previously reported promising preclinical data demonstrating that the co-administration of a pharmacological chaperone with ERT has the potential to address key limitations of ERT. The addition of a pharmacological chaperone has been shown to prevent the loss of activity of ERT in the circulation, increase tissue uptake, and increase substrate reduction in multiple disease-relevant tissues. Preclinical proof of concept has been established for Fabry disease and Pompe disease.

About AT2220

Data from 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. Based on these data and encouraging safety data from preclinical studies, Amicus initiated a Phase 2 clinical trial of AT2220 as a monotherapy treatment in adults with Pompe disease. The protocol involved initial treatment with a high dose of AT2220. Two patients enrolled in the trial experienced adverse events categorized as serious and probably related to treatment with AT2220, and as a result the IND was placed on clinical hold.

Amicus completed a thorough investigation of the events, including the completion of additional preclinical and Phase 1 studies. As a result the Company decided to continue development of AT2220 co-administered with ERT but not as a monotherapy.

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 muscle 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 focused on developing treatments for rare diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. Amicus' lead program, Amigal (migalastat HCl) is in Phase 3 development for the treatment of Fabry disease and is partnered with GSK Rare Diseases.

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 products and the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products. 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 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 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 regulatory authorities may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies 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. In addition, all forward looking statements are subject to other risks detailed in our Annual Report on Form 10-K for the year ended December 31, 2010. 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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