February 14, 2013

Amicus Therapeutics Advances Chaperone-Enzyme Replacement Therapy (ERT) Combination Platform in Pompe Disease

Phase 2 AT2220-ERT Co-Administration and Preclinical AT2220-ERT Co-Formulation Studies Presented at LDN WORLD Symposium

Next-Generation ERT Advancing in Preclinical Studies

Conference Call on February 15, 2013 at 11:30am ET

CRANBURY, N.J., and ORLANDO, Fla., Feb. 14, 2013 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) today announced positive results from clinical and preclinical studies of the pharmacological chaperone AT2220 (duvoglustat HCl) in combination with ERT for Pompe disease at the Lysosomal Disease Network WORLD Symposium (LDN WORLD).

John F. Crowley, Chairman and Chief Executive Officer of Amicus stated, "The advancement of our core platform technology in Pompe and other lysosomal storage disorders is a continued great step forward for Amicus. Across these serious genetic diseases, we are leveraging this chaperone-ERT combination platform to work towards improving currently marketed ERTs and to develop our own proprietary next-generation ERTs that incorporate our small molecule chaperones. These chaperone stabilizers have the potential to enhance ERT activity and tissue uptake while also significantly reducing the immunogenicity of the ERTs. Through these programs we hope to offer new benefits and treatment options for patients with lysosomal storage diseases."

Chaperone-ERT Combinations for Pompe Disease

AT2220 Co-Administered with Marketed ERTs

Positive results from a Phase 2 study (Study 010) established human proof-of-concept that co-administration of AT2220 just prior to infusing ERT (Myozyme/Lumizyme, or rhGAA enzymes) increases GAA enzyme activity in muscle tissue compared to ERT alone. These results appear in a poster[1] and will be featured in an oral platform presentation at LDN WORLD on Friday, February 15 at 9:15 am ET.

Based on these results, Amicus plans to initiate a repeat-dose clinical study in the third quarter of 2013 to evaluate a novel intravenous formulation of AT2220 (AT2220-IV) co-administered with Myozyme/Lumizyme. AT2220-IV when co-administered with ERT is designed to have an improved pharmacokinetic (PK) profile compared to oral AT2220 for all Pompe patients, many of whom are unable to swallow an oral small molecule. The upcoming clinical study will investigate multiple doses of AT2220-IV co-administered with Myozyme/Lumizyme every two weeks in Pompe patients. Objectives of the study are to characterize safety and PK for later evaluation of infants and special populations. Key parameters are expected to include GAA enzyme activity and AT2220 levels in plasma and muscle, as well as rhGAA antibody titers.

Next-Generation ERT (AT2220 Co-Formulated with a Proprietary Amicus ERT)

Preclinical studies of AT2220 co-formulated with rhGAA enzyme (Myozyme/Lumizyme) were presented for the first time in a poster[2] at LDN WORLD. This chaperone-ERT co-formulation resulted in up to 2.5-fold greater enzyme uptake in multiple disease-relevant tissues and led to greater glycogen reduction compared to rhGAA alone in GAA knock-out mice. Collectively these data suggest that AT2220 directly binds to and stabilizes rhGAA, potentially leading to a larger amount of properly folded, active enzyme available for uptake into tissue. AT2220 co-formulated with ERT may also mitigate Pompe ERT-related immunogenicity since properly-folded proteins are less prone to aggregation and less immunogenic.

Following the completion of these preclinical studies, Amicus entered into a contract with Laureate Pharmaceuticals for the manufacture of a proprietary rhGAA enzyme. Amicus is developing AT2220 co-formulated with this proprietary enzyme as a next-generation ERT for Pompe disease. Through this approach Amicus believes it has the potential to improve the properties of the rhGAA enzyme itself while incorporating AT2220 as a small molecule stabilizer to increase exposure and tissue uptake, and reduce immunogenicity relative to currently marketed ERTs. Successful development of a more stable ERT may also enable novel routes of delivery such as subcutaneous administration.

Conference Call and Webcast

John F. Crowley, Chairman and Chief Executive Officer, and members of the Amicus executive team will host a conference call and live audio/visual webcast on Friday, February 15, 2013 at 11:30am ET to discuss data from several programs presented at LDN WORLD. Interested participants and investors may access the conference call at 11:30 a.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international). A live audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicustherapeutics.com/events.cfm, and will be archived for 30 days. The slide presentation for the conference call/webcast will also be available at http://ir.amicustherapeutics.com/events.cfm. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 2:30 p.m. ET on February 15, 2013. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 97505816.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan 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 human genetic diseases. Amicus' late-stage programs for lysosomal storage disorders include migalastat HCl monotherapy in Phase 3 for Fabry disease; migalastat HCl co-administered with enzyme replacement therapy (ERT) in Phase 2 for Fabry disease; and AT2220 co-administered with ERT in Phase 2 for Pompe disease.

About AT2220 for Pompe Disease

AT2220 is an investigational, orally-administered pharmacological chaperone owned exclusively by Amicus. In published preclinical studies, AT2220-ERT co-administration resulted in significant increases in muscle rhGAA levels and decreases in glycogen levels in a mouse model of Pompe disease. Preclinical results to date also suggest that AT2220-ERT co-administration may mitigate ERT-induced immunogenicity by stabilizing the enzyme in its properly folded and active form. Initial *ex vivo* studies using T cells derived from blood from 50 healthy donors demonstrated that the addition of AT2220 may significantly reduce the immunogenicity of Myozyme and Lumizyme.

Pompe disease is a lysosomal storage disease characterized by progressive skeletal muscle weakness and respiratory insufficiency. It is caused by a deficiency in GAA activity, which leads to accumulation of glycogen in tissues affected by the disease (primarily muscle). Pompe disease affects an estimated 5,000 to 10,000 individuals worldwide and is clinically heterogeneous in the age of onset, the extent of organ involvement, and the rate of progression.

[1] Kishnani, *et al.*, A Phase 2a Study to Investigate Drug-Drug Interactions between Escalating Doses of AT2220 (Duvoglustat Hydrochloride) and Acid Alfa-Glucosidase in Subjects with Pompe Disease, **LDN WORLD 2013**

[2] Khanna, *et al.*, Exploring the Use of a Co-formulated Pharmacological Chaperone AT2220 with Recombinant Human Acid Alpha-Glucosidase for Pompe Disease, **LDN WORLD 2013**

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to clinical development of Amicus' candidate drug products and the timing and reporting of results from 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 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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2012. 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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