Amicus Therapeutics Announces Positive Results from All Four Cohorts in Phase 2 Chaperone-Enzyme Replacement Therapy (ERT) Co-Administration Study for Pompe Disease

Strong Proof-of-Concept Data for Chaperone's Ability to Stabilize and Enhance Activity and Uptake of Currently Marketed ERT Products for Pompe Disease

Results to be Presented at LDN WORLD Symposium in February 2013

Initiation of Repeat-Dose Pompe Study Anticipated in 3Q13

CRANBURY, NJ, January 4, 2013 – Amicus Therapeutics (Nasdaq: FOLD) today announced positive preliminary results from all 4 dose cohorts in a Phase 2 study (Study 010) to evaluate the safety and pharmacokinetic (PK) effects of the pharmacological chaperone AT2220 (duvoglustat HCl) co-administered with enzyme replacement therapy (ERT) for Pompe disease (Myozyme® and Lumizyme®). Myozyme and Lumizyme (alglucosidase alfa, or recombinant human GAA enzyme, rhGAA) are the first and only approved treatments for Pompe disease. Based on the Study 010 results, Amicus expects to initiate a repeat-dose clinical study in the third quarter of 2013.

For people with Pompe disease, deficient GAA enzyme leads to the accumulation of glycogen in tissues affected by disease (primarily muscle). Preclinical data[1] demonstrated that AT2220 in combination with ERT enhances rhGAA enzyme activity, reduces glycogen accumulation, and potentially mitigates ERT-related immunogenicity in a mouse model of Pompe disease. In Study 010, co-administration of AT2220 to Pompe patients increased rhGAA enzyme activity and enhanced rhGAA enzyme uptake into muscle tissue compared to ERT alone.

John F. Crowley, Chairman and Chief Executive Officer stated, "Study 010 has established human proof-of-concept that AT2220-ERT co-administration increases GAA enzyme activity in muscle. We look forward to initiating our repeat-dose clinical study to investigate the effect of AT2220-ERT co-administration on ERT stability and activity, ERT-related immunogenicity, and other clinical measures. We believe that co-administration may deliver significant benefits compared to ERT alone and become an important therapy for people with Pompe disease."

Additional details surrounding the development strategy for AT2220 in combination with ERT for Pompe disease will be provided during a live presentation and webcast at the 31st Annual JPMorgan Healthcare Conference on January 9, 2013 at 3:00 p.m. PT.

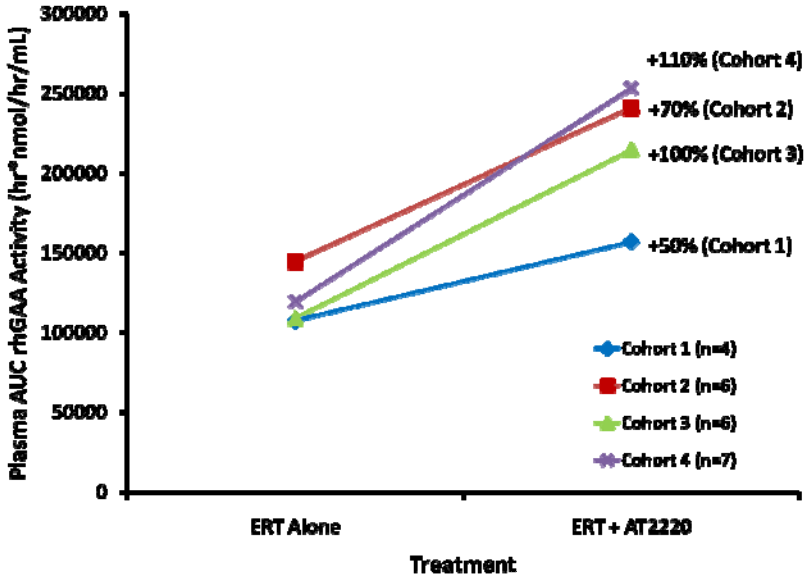
Study 010 Results – AT2220 Co-Administered with ERT (n=23)

Study 010 investigated single ascending oral doses of AT2220 co-administered with Myozyme or Lumizyme in patients with Pompe disease. The doses of AT2220 were 50 mg (Cohort 1), 100 mg (Cohort 2), 250 mg (Cohort 3), and 600 mg (Cohort 4). Each patient received one infusion of ERT alone, and then a single oral dose of AT2220 one hour before the next ERT infusion. Highlights from all four dose cohorts of AT2220 were as follows:

Safety: Single doses of AT2220 co-administered with ERT were well-tolerated, with no drug-related adverse events reported. In addition, AT2220 was cleared from muscle to near-undetectable levels by Day 7 in all four cohorts.

Recombinant Human GAA (rhGAA) Enzyme Activity in Plasma: 24-hour plasma PK was measured during and after each infusion. Plasma rhGAA activity increased in 23 out of 23 patients (100%) following co-administration and the increases were dose-related. These data suggest that co-administration increases the amount of stabilized, properly folded, and active rhGAA enzyme available for uptake into tissue.

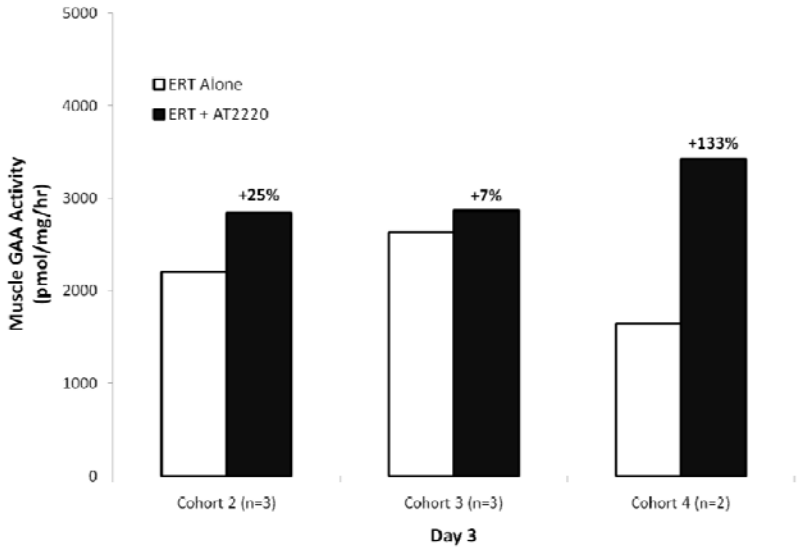
rhGAA Enzyme Activity in Plasma Area Under Curve (AUC)	
ERT + AT2220 vs. ERT Alone	
Cohort	**% Increase vs. ERT Alone**
1 (n=4)	50%
2 (n=6)	70%
3 (n=6)	100%
4 (n=7)	110%



Enzyme Activity in Muscle: Muscle biopsies were taken to measure GAA enzyme uptake into muscle tissue, with and without AT2220. In Cohort 1, all 4 patients had muscle biopsies on Day 7. In Cohorts 2-4, muscle biopsies were taken on Day 3 for half the patients, and on Day 7 for the other half of patients.

In Cohort 1, no consistent change in GAA enzyme activity was observed at day 7. In Cohorts 2, 3, and 4 the results show that more enzyme is taken up into muscle tissue following AT2220 co-administration compared to ERT alone. The effect was most pronounced at the highest (600 mg) dose of AT2220.

GAA Enzyme Activity in Muscle at Day 3	
ERT + AT2220 vs. ERT Alone	
Cohort	**% Increase vs. ERT Alone**
2 (n=3)	25%
3 (n=3)	7%
4 (n=2)	133%



At Day 3 the GAA enzyme activity in muscle following co-administration compared to ERT alone in patients with evaluable biopsies increased by the following: 25% in Cohort 2 (n=3), 7% in Cohort 3 (n=3), and 133% in Cohort 4 (n=2). At Day 7 the GAA enzyme activity in muscle was lower relative to Day 3, as expected based on the cellular half-life of the enzyme. However, following co-administration compared to ERT alone in patients with evaluable biopsies the following increases were sustained: 20% in Cohort 2 (n=3), 40% in Cohort 3 (n=2), and 20% in Cohort 4 (n=3).

Effect of AT2220 on ERT-Related Immunogenicity Measured *ex vivo*

By stabilizing the folded and active form of the rhGAA enzyme, AT2220 may mitigate ERT-induced immunogenicity since unfolded and aggregated proteins are generally more antigenic than properly folded proteins. Recent published studies show that approximately 40% of the administered ERT can be captured by circulating antibodies and infusion associated reactions occur in approximately 50% of Pompe patients receiving ERT infusions.[2] 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. The studies utilized Antitope Ltd.'s EpiScreen™ assay and are being repeated in samples from the Pompe patients in Study 010. Results from these *ex vivo* studies may help to guide the clinical investigation of the effects of AT2220 on ERT-related immunogenicity.

Study 010 Design

Study 010 is a Phase 2 open-label, multi-center study to evaluate the safety and PK effects of four increasing oral doses of AT2220 (50 mg, 100 mg, 250 mg, or 600 mg) co-administered with ERT (Myozyme®/Lumizyme®) versus ERT alone in males and females with Pompe disease. The study enrolled male and female patients who had been on a stable dose and regimen of ERT for at least three months. All patients were given a regularly scheduled ERT infusion. One hour prior to the initiation of the next ERT infusion, patients received a single oral dose of AT2220. Plasma rhGAA activity and protein levels were evaluated during each infusion. Each patient underwent muscle biopsies three or seven days after each infusion to measure tissue GAA enzyme activity with and without the chaperone, as well as to measure the level of AT2220 in the muscle. More information about Study 010 can be obtained by visiting www.clinicaltrials.gov: NCT1380743 or www.pompestudy.com.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq: FOLD) is a biopharmaceutical company at the forefront of developing therapies 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 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. The Company has completed a Phase 2 study (Study 010) of AT2220 (duvoglustat HCl) co-administered with the ERT alglucosidase alfa (Myozyme/Lumizyme) in individuals with Pompe disease. Published preclinical data[1] suggest that AT2220 in combination with this ERT may improve rhGAA enzyme activity, reduce glycogen accumulation, and potentially mitigate ERT-related immunogenicity in patients with Pompe disease.

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. Myozyme and Lumizyme (alglucosidase alfa, or recombinant human GAA enzyme, rhGAA) are the first and only approved treatments for Pompe disease. The clinical benefit of Myozyme and Lumizyme may be limited by low stability of the recombinant enzyme at neutral pH and body temperature, modest tissue uptake, and immune responses that affect tolerability and efficacy. Immune responses in the form of antibodies to rhGAA occur in a majority of Pompe patients receiving Myozyme/Lumizyme infusions[3] and may limit treatment outcomes with ERT.

1. Khanna R, *et al.*, The Pharmacological Chaperone AT2220 Increases Recombinant Human Acid α-Glucosidase Uptake and Glycogen Reduction in a Mouse Model of Pompe Disease., **PLoS ONE** (2012) 7(7): e40776. doi:10.1371/journal.pone.0040776.
2. Banati M, *et al.*, Enzyme Replacement Rherapy Induces T-cell Responses in Late-Onset Pompe Disease., **Muscle Nerve**. 2011 Nov;44(5):720-6.
3. Lacana E, *et al.*, The Role of Immune Tolerance Induction in Restoration of the Efficacy of ERT in Pompe Disease., **Am J Med Genet C Semin Med Genet.** 2012 160C:30-39

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 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 year ended September 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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