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Amicus Therapeutics Presents Additional Positive Preliminary Phase 1/2 Data at WORLDSymposium™ 2017

Biomarkers of Muscle Damage (AST, ALT and CK) Demonstrate Improving Trends in Majority of Patients

Biomarker of Key Disease Substrate (Hex4) Demonstrates Improving Trend in All Patients

Safety Data Continue to Show No Infusion-Associated Reactions Following 150+ Infusions

CRANBURY, N.J. and SAN DIEGO, Feb. 15, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, today presented additional positive preliminary data from a global Phase 1/2 study ([ATB200-02](#)) to investigate [ATB200/AT2221](#) in a [poster](#)¹ at the 13th Annual [WORLDSymposium™](#) in San Diego, CA. ATB200/AT2221 is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone.

"We are very pleased to share these additional positive preliminary clinical results here at the *WORLDSymposium* as we continue with the cascade of data from this important study in Pompe Disease patients," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "In addition to excellent continued safety, tolerability and PK profile, we now see evidence that this novel Amicus treatment paradigm is further reducing important biomarkers of disease in both ERT-switch patients as well as in the first ERT-treatment naïve patients. In particular, demonstrating with these data that we can further reduce these biomarkers in a switch population is very encouraging. To our knowledge, there are no published data for other Pompe ERTs where these types of reductions in biomarkers have been shown. Moreover, the fact that these biomarkers have demonstrated a sustained reduction in a majority of patients over the first 18 weeks suggests that the effects of our approach may indeed be persistent and durable. We look forward to data in more patients and over longer periods of time in the months ahead, including important measures of muscle function in these patients. We could not be more pleased with the preliminary data to date."

UPDATED CLINICAL DATA HIGHLIGHTS:

ATB200-02 Clinical Study -- Design and Objectives

- Primary objectives: to evaluate safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221
- Study duration: 18-week primary treatment period followed by a long-term extension
- Patient cohorts: ambulatory ERT-switch patients (Cohort 1), non-ambulatory ERT-switch patients (Cohort 2) and ERT-naïve patients (Cohort 3). Enrolling up to ~20 total patients across all cohorts.
- Preliminary data now available:
 - Safety data for 13 patients through interim data analysis (maximum 36 weeks)
 - PK and PD (muscle biomarker and disease substrate biomarker) data for 10 patients (eight ERT-switch patients and two naïve patients)

ATB200-02 Study -- [Preliminary Data](#) Highlights in Initial ERT-Switch and Naive Patients

- **ATB200/AT2221 safety measures (n=13) showed:**
 - No serious adverse events (SAEs)
 - TEAEs were generally mild and transient
- **To date, ATB200/AT2221 has shown no infusion-associated reactions following 150+ infusions**
- **Clinical PK profile was consistent with previously reported preclinical data (n=10).**
 - ATB200 plasma clearance suggests optimized carbohydrate structure provides efficient uptake into tissues
 - ATB200 alone showed greater than dose-proportional increases in exposure
 - ATB200 exposure was further enhanced with the addition of the chaperone AT2221, consistent with stabilization of ATB200 by AT2221.

• **Reductions observed in biomarkers of muscle damage (creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST)) in ambulatory switch patients (N=8, week 18) and naïve patients (N=2, week 4)**

- In the eight ERT-switch patients:
 - ALT decreased in 5 of 8 patients; 4/4 patients with elevated baseline levels normalized
 - AST decreased in 6 of 8 patients; 3/4 patients with elevated baseline levels normalized
 - CK decreased in 6 of 8 patients; 2/6 patients with elevated baseline levels normalized
 - ALT, AST, CK generally remained stable in patients not demonstrating a decrease
- In the two ERT-naïve patients, all three biomarkers improved

• **Reduction observed in a biomarker of glycogen substrate -- Urine Hexose Tetrasaccharide (Hex4) -- in all eight ERT-switch patients and both naïve patients; overall reduction approximately 30%**

• **Next interim analysis anticipated in the coming months**

About ATB200/AT2221

[ATB200/AT2221](#) is a novel treatment paradigm that consists of ATB200, a unique recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly mannose-6 phosphate (M6P), to enhance uptake, co-administered with AT2221, a pharmacological chaperone. In preclinical studies, ATB200 was associated with increased tissue enzyme levels and reduced glycogen levels in muscle, which was further improved when AT2221 was co-administered with ATB200. Amicus Therapeutics is currently conducting a global Phase 1/2 study ([ATB200-02](#)) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of ATB200/AT2221.

About Pompe Disease

Pompe disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called acid alpha-glucosidase (GAA). Reduced or absent levels of GAA lead to the accumulation of the substrate glycogen in the lysosomes of muscles and other tissues. Progressive accumulation of glycogen is believed to lead to the morbidity and mortality associated with Pompe disease, including muscle weakness and respiratory insufficiency.

About Amicus Therapeutics

[Amicus Therapeutics](#) (Nasdaq:FOLD) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone [migalastat](#) as a monotherapy for Fabry disease, [SD-101](#) for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

¹[Johnson, et. al. WORLDSymposium 2017. First-in-human preliminary pharmacokinetic and safety data on a novel recombinant acid-α-glucosidase, ATB200, co-administered with the pharmacological chaperone, AT2221, in ERT-experienced Pompe patients](#)

Forward-Looking Statements

This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to encouraging preliminary data from a global phase 1/2 study to investigate ATB200/AT2221 for the treatment of Pompe and the potential implications on these data for the future advancement and development of ATB200/AT2221. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "confidence," "encouraged," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. The forward looking statements included in this press release are based on management's current expectations and belief's which are subject to a number of risks, uncertainties and factors, including that the preliminary data based on a small patient sample and reported before completion of the study will not be predictive of future results, that results of additional preliminary data or data from the completed study or any future study will not yield results that are consistent with the preliminary data presented, that the Company will be not able to demonstrate the safety and efficacy of ATB200/AT2221, that later study results will not support further development, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize ATB200/AT2221. In addition, all forward looking statements are subject to the other risks and uncertainties detailed in our Annual Report on Form 10-K for the year ended December 31, 2015 and Quarterly Report on 10-Q for the Quarter ended September 30, 2016. As a consequence, actual results may differ materially from those set forth in this press release. You are cautioned not to place undue reliance on these forward looking statements, which speak only of the date hereof. All forward looking statements are qualified in their entirety by this cautionary statement and we undertake no obligation to revise this press release to reflect events or circumstances after the date hereof.

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