December 8, 2016

Amicus Therapeutics Announces Positive Preliminary Data from Phase 1/2 Study of Novel Treatment Paradigm for Pompe Disease

ATB200/AT2221 Safety Data Show No Infusion-Associated Reactions Following 100+ Infusions

Clinical Pharmacokinetic (PK) Profile as Predicted Based on Previously Reported Preclinical Data

Biomarkers of Muscle Damage are Generally Stable or Trending Towards Improvement

Conference Call at 8:30am ET

CRANBURY, N.J., Dec. 08, 2016 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, today announced positive preliminary data from a global Phase 1/2 study (ATB200-02) to investigate 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.

ATB200-02 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 with all patients eligible for 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 nine patients through interim data analysis (maximum 24 weeks)
  - PK and PD (muscle biomarker) data through Week 14 for four patients in Cohort 1

ATB200-02 Study - Preliminary Data Highlights in Initial ERT-Switch Patients

- **ATB200/AT2221 safety measures (n=9) showed:**
  - No serious adverse events (SAEs)
  - AEs were generally mild and transient
- **To date, ATB200/AT2221 has shown no infusion-associated reactions following 100+ infusions**
- **Clinical PK profile through Week 14 was as predicted based on previously reported preclinical data (n=4).**
  - ATB200 plasma clearance suggests optimized carbohydrate structure provides efficient uptake into tissues
  - ATB200 alone showed greater than dose-proportional increases in exposure, which was further enhanced with the addition of the chaperone AT2221
- **Biomarkers of muscle damage were trending toward improvement or stable through Week 14 (n=4)**
  - Biomarkers of muscle damage (creatine kinase (CK) enzyme, alanine aminotransferase (ALT), and aspartate aminotransferase (AST)) in the initial four ERT-switch patients showed early trends toward improvement in two patients and were stable in the other two patients.

"We set out on this journey in Pompe disease at Amicus with the intent to develop a new treatment paradigm," said John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. "These preliminary results, while still early, give us greater confidence that we are developing a drug regimen that may be highly differentiated from any other approach. Even at this interim analysis, it is very encouraging to see no infusion-associated reactions in these initial patients, as well as the desired PK profile and improvements in key muscle damage biomarkers in two of four patients. We have much more work to do to generate additional important data in the months ahead but this is an excellent foundation for this program and for persons living with this devastating disorder."

Barry Byrne, M.D., Ph.D., Professor, Pediatrics and Molecular Genetics & Microbiology and Director, University of Florida Powell Center, stated, "As a lead investigator in the ATB200-02 study, I have had a positive initial experience with the novel Amicus treatment regimen for Pompe. Elevated levels of CK, ALT and AST indicate damage to muscle tissue in patients with Pompe disease. A reduction in markers of muscle damage, such as CK, ALT, and AST in patients with Pompe disease is very encouraging, especially with favorable safety and tolerability. If these positive trends continue, ATB200/AT2221 may help to address the significant unmet need among Pompe patients. I look forward to seeing additional data from this clinical study."

Conference Call and Webcast

Amicus Therapeutics will host a conference call and webcast today, December 8, 2016 at 8:30 a.m. ET. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international). The slide presentation to accompany this conference call and webcast will be available at http://ir.amicusrx.com/events.cfm.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/events.cfm, and will be archived for 30 days. 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 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 34348280.

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.

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 our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. In particular, this press release relates to the preliminary data from a global Phase 1/2 study (ATB200-02) to investigate ATB200/AT2221. The inclusion of forward-looking statements arising from this preliminary data and study should not be regarded as a representation by us that any of our plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong and can be affected by inaccurate assumptions we 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 in particular 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 our business, including, without limitation: the potential that results of clinical or preclinical 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, including the FDA, EMA, and PMDA, may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing Galafold in Europe or our other product candidates if and when approved; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. The preliminary data and Phase 1/2 study discussed herein is inherently preliminary and early in the study, derived from a limited patient set, and later trial results with this patient set or others may not be consistent with these preliminary results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. 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, 2015 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2016. 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 we undertake no obligation to revise or update this news release to reflect events or circumstances after the date hereof.

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