Amicus Therapeutics Announces Additional Positive Data in Pompe Disease Phase 1/2 Study at 14th Annual WORLDSymposium™

February 7, 2018

Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers Out to Month 12

Very Low Rate (<1%) of Infusion Associated Reactions Maintained After 550+ Infusions

CRANBURY, N.J. and SAN DIEGO, Feb. 07, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD) today announced additional positive results from a global Phase 1/2 clinical study (ATB200-02) to investigate ATB200/AT2221 in patients with Pompe disease, an inherited lysosomal storage disorder caused by an enzyme deficiency that leads to accumulation of glycogen (disease substrate) in cells. Patients treated with ATB200/AT2221 for up to 12 months showed improvements in six-minute walk test (6MWT) distance and other measures of motor function, stability or increases in forced vital capacity (FVC), and durable reductions in biomarkers of muscle damage and disease substrate. These clinical results are being featured at the 14th Annual World*Symposium*™in a late-breaker poster[1] today, and a corresponding oral platform presentation on Thursday, February 8, 2018 at 1:15pm PT.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, “We continue to be impressed by the clinical data for our novel Pompe treatment paradigm ATB200/AT2221. These latest data, in more patients and over longer periods, have continued to show substantial improvements in functional outcomes in nearly all patients, which are aligned with the persistent and durable reductions in key biomarkers of muscle damage and disease substrate. On the heels of these data for ATB200/AT2221 we look forward to continuing our discussions with global regulators to define the best and fastest pathway to deliver this critically important medicine to people living with Pompe disease.”

Tahseen Mozaffar, MD, Director, Neuromuscular Program, Neurology School of Medicine at UC Irvine and Principal Investigator in the ATB200-02 study stated, “The results from this Phase 1/2 clinical study of ATB200/AT2221 show very meaningful improvements across functional measures in both ERT-switch and ERT-naïve patients for up to 12 months, with remarkable consistency across the vast majority of patients, as well as across endpoints. This treatment regimen has also been well tolerated by the patients in this study. Overall, the safety and functional data, in addition to the biomarkers of muscle damage and disease substrate, suggest the potential for ATB200/AT2221 to become an important treatment paradigm for people living with Pompe disease.”

Amicus continues to engage in a series of collaborative discussions with regulators in the U.S. and EU, and expects to provide an update in the first half of 2018.

ATB200-02 Study Data Highlights in ERT-Switch and ERT-Naive Patients Out to Month 12

A copy of the *WORLDSymposium* ™poster and a slide deck summarizing the latest clinical results from the ATB200-02 clinical study is available at www.amicusrx.com.

Safety, Tolerability & Pharmacokinetics (PK) (n=20)
Safety and tolerability data in all 20 patients reflect a maximum of 20+ months of treatment. To date, adverse events have been generally mild and transient. Importantly, ATB200/AT2221 has resulted in a low rate of infusion-associated reactions (IARs) following 550+ infusions (three events of IARs in two patients; <1% of all 550+ infusions with an IAR). The clinical pharmacokinetic profile has been consistent with previously reported preclinical data.

Functional Outcomes (n=20)
Data on functional outcomes are available for 19 of the 20 patients enrolled (one patient dropped out of the extension study due to travel burden and family considerations). Muscle function improved in 16 of 19 patients at month 9. Muscle function improved in 10 out of 10 patients with available data at month 12.

- **Motor function (n=15):** Six-minute walk test (6MWT) distance, a primary measure of motor function in Pompe disease patients, improved in both ERT-naive and ERT-switch patients with continued benefit observed out to month 12. Improvements were generally consistent across patients and cohorts. Additional detail on patient-level 6MWT distance data is available in the poster.
    - All 5 ERT-naive patients showed increases in 6MWT distance at all time points out to month 12. The ERT-naïve patients showed mean increases of 42 meters at month 6 (n=5), 64 meters at month 9 (n=5), and 87 meters at month 12 (n=2).
    - Of the 10 ERT-switch patients, 8 patients showed increases in 6MWT distance and two patients showed decreases at month 9. All eight of the ERT-switch patients with available data at month 12 showed increases in 6MWT distance. The ERT-switch patients showed mean increases of 24 meters at month 6 (n=10), 25 meters at month 9 (n=10), and 57 meters at month 12 (n=8).
    - Other motor function tests, as detailed in the poster, generally showed mean improvements consistent with 6MWT distance.

- **Muscle Strength (n=4):** three of the four non-ambulatory ERT-switch patients showed improvements in upper extremity strength (which includes elbow and shoulder) from baseline to month 9, as measured by quantitative muscle testing (QMT) and manual muscle testing (MMT).

- **Pulmonary Function (n=14):** Pulmonary function improved in ERT-naïve patients and was generally stable in ERT-switch patients. In ERT-naïve patients, mean absolute change in forced vital capacity (FVC), one of the main measures of pulmonary function in Pompe disease, was +4.2% at month 6 (n=5), +6.2% at month 9 (n=5), and +6.0% at month 12 (n=2). In ERT-switch patients mean absolute change in FVC was -1.3% at month 6 (n=9), -1.7% at month 9 (n=9), and -3.1% at month 12 (n=7). Overall, other pulmonary tests of maximal inspiratory pressure (MIP), a measure of inhalation, and maximal expiratory pressure (MEP), a measure of exhalation, were stable or increased in both ERT-naïve and ERT-switch patients.

Pharmacodynamic (PD) Data on Muscle Damage and Disease Substrate Biomarkers (n=20)
Treatment with ATB200/AT2221 resulted in persistent and durable reductions in key biomarkers of muscle damage (creatine kinase, or CK) and disease substrate (urine hexose tetrasaccharide, or Hex4) across all patient cohorts out to month 12 and continue to suggest a positive effect on muscle tissue. Further details are provided in the poster.

About ATB200-02 Clinical Study
The primary objectives of the open-label ATB200-02 clinical study are to evaluate the safety, tolerability, pharmacokinetics (PK), and pharmacodynamics (PD) of ATB200/AT2221 over an 18-week primary treatment period followed by a long-term extension. Sixteen clinical sites in five countries participated in the ATB200-02 clinical study. The study enrolled a total of 20 patients across three patient cohorts: ambulatory ERT-switch (Cohort 1, n=11), non-ambulatory ERT-switch (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). Patients in Cohort 1 received escalating doses of ATB200 (5, 10, 20 mg/kg), followed by 3 doses of 20 mg/kg ATB200 plus low dose AT2221, followed by ongoing doses of 20 mg/kg ATB200 plus high dose AT2221. Patients in Cohorts 2 and 3 have all received 20 mg/kg ATB200 plus high dose AT2221.

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 the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA leads to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. Pompe disease can be debilitating, and is characterized by severe muscle weakness that worsens over time. Pompe disease ranges from a rapidly fatal infantile form with significant impacts to heart function to a more slowly progressive, late-onset form primarily affecting skeletal muscle. It is estimated that Pompe disease affects approximately 5,000 to 10,000 people worldwide.

About Amicus Therapeutics
Amicus Therapeutics (Nasdaq:FOLD) is a global, patient-centric biotechnology company focused on discovering, developing and delivering novel high-quality medicines for people living with rare metabolic diseases. The lead program in the Amicus portfolio is migalastat, an oral precision medicine for people living with Fabry disease who have amenable genetic mutations. Migalastat is currently approved under the trade name Galafold™ in the European Union, with additional approvals granted and pending in several geographies. The future value driver of the Amicus pipeline is ATB200/AT2221, a novel, late-stage, potential best-in-class treatment paradigm for Pompe disease. The Company is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases.

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, 2016 and Quarterly Report on 10-Q for the Quarter ended September 30, 2017. 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.

[1]Mozaffar, *et. al.*, **14th Annual World*Symposium*™**; Updated results from ATB200-02: a first-in-human, open-label, phase 1/2 study of ATB200 co-administered with AT2221 in adults with Pompe disease

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