Amicus Therapeutics Announces Positive 18-Month Data in Pompe Disease Phase 1/2 Study at 23rd International Annual Congress of the World Muscle Society

October 5, 2018

Consistent and Durable Responses Across Key Measures of Safety, Functional Outcomes and Biomarkers Continue Out to Month 18 for both ERT-Naïve and ERT-Switch Patients

Very Low Rate (<1%) of Infusion Associated Reactions Maintained After ~900 Infusions

CRANBURY, N.J. and Mendoza, ARGENTINA, Oct. 05, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD) today announced additional positive results from a global Phase 1/2 clinical study (ATB200-02) to investigate AT-GAA 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 AT-GAA for up to 18 months showed improvements in six-minute walk test (6MWT) distance and other measures of motor function and muscle strength, 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 23rd International Annual Congress of the World Muscle Society in an oral platform presentation today, Friday October 5, 2018 at 12:20am ART (11:20am EDT). The presentation will be given by Professor Benedikt Schoser, senior consultant at the Friedrich-Baur-Institute, Dept. of Neurology at the Ludwig-Maximilians-University of Munich, Germany and Principal Investigator in the ATB200-02 study.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics stated, “The clinical data for our investigational therapy for Pompe disease AT-GAA are very compelling and consistent across patients and in multiple endpoints now for up to 18 months on treatment. These latest data continue to show meaningful and very durable improvements in functional outcomes in nearly all patients, in addition to persistent and durable reductions in key biomarkers of muscle damage and disease substrate. These new results further support our strategy to significantly enhance the body of clinical data for AT-GAA through our ongoing clinical and natural history studies, as well as our upcoming pivotal study, as we seek to deliver this potential new therapy to as many people living with Pompe disease as soon as possible.”

Professor Schoser stated, “The 18-month results from this Phase 1/2 clinical study of AT-GAA continue to demonstrate a robust effect in adult people living with Pompe disease, including those who had switched from ERT and those who had not previously been treated. I am glad to see the meaningful improvements in muscle strength in most of the patients. Since entering the study, the ambulatory ERT-experienced and ERT-naïve cohorts have walked farther in the 6-minute walking test, and the non-ambulatory ERT-experienced cohort have demonstrated sustained positive changes in their arm and shoulder movements. Overall, the safety and functional data suggest that AT-GAA has the potential to improve treatment for people living with Pompe disease.”

ATB200-02 Study Data Highlights in ERT-Switch and ERT-Naive Patients Out to Month 18

The slide deck with the latest clinical results from the ATB200-02 clinical study presented at World Muscle Society is available at www.amicusrx.com. These results, including additional detailed results at month 18, will be highlighted by Mark Roberts, MD, Department of Neurology, Salford Royal NHS Foundation Trust and Principal Investigator in the ATB200-02 study, at the upcoming Amicus Analyst Day on October 11, 2018.

Safety, Tolerability & Pharmacokinetics (PK) (n=20)
Safety and tolerability data in all 20 patients reflect a maximum of 28+ months of treatment. To date, adverse events have been generally mild and transient. Importantly, AT-GAA has resulted in a low rate of infusion-associated reactions (IARs) following 890+ infusions (7 events of IARs in 5 patients; <1% of all 890+ infusions with an IAR). The clinical pharmacokinetic profile has been consistent with previously reported preclinical data.

Functional Outcomes (n=19)
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 17 of 19 patients at month 12. Muscle function improved in 17 out of 18 patients with available data at month 18.

- **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 18. Improvements were generally consistent across both cohorts.
    - All 5 ERT-naive patients showed increases in 6MWT distance at all time points out to month 18. The ERT-naïve patients showed mean increases of 42 meters at month 6 (n=5), 63 meters at month 12 (n=5), and 49 meters at month 18 (n=5).
    - 6MWT increased in 7/10, 9/10, and 9/9 ERT-switch patients at Months 6, 12, and 18, respectively. The ERT-switch patients showed mean increases of 24 meters at month 6 (n=10), 42 meters at month 12 (n=10), and 52 meters at month 18 (n=9).
    - Other motor function tests generally showed mean improvements consistent with 6MWT distance out to month 18 in both ambulatory cohorts.

- **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 18, as measured by quantitative muscle testing

(QMT) and manual muscle testing (MMT). Further details are provided in the slide deck.

- **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), +4.4% at month 12 (n=5), and +5.0% at month 18 (n=5). In ERT-switch patients mean absolute change in FVC was -1.3% at month 6 (n=9), -3.3% at month 12 (n=9), and -3.7% at month 18 (n=8). 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 AT-GAA 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 18 and continue to suggest a positive effect on muscle tissue. Further details are provided in the slide deck.

AT-GAA Development and Regulatory Strategy
As previously announced, Amicus is building a robust data set for AT-GAA across several studies including the ongoing ATB200-02 clinical study, a retrospective natural history study, and the upcoming pivotal study.

Anticipated milestones:

- Initiation of pivotal study to support full approval in U.S. and EU, as well as other geographies (2H18)
- Complete a retrospective natural history study in approximately 100 ERT-treated Pompe patients (2H18)
- Additional ATB200-02 study data from up to 10 additional ERT-switch patients in a new Cohort 4 (2019)
- Initiation of studies in additional patient populations, including pediatric patients (2019)

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 originally enrolled a total of 20 patients across three patient cohorts: ambulatory ERT-experienced (Cohort 1, n=11), non-ambulatory ERT-experienced (Cohort 2, n=4) and ERT-naïve (Cohort 3, n=5). A fourth cohort of ambulatory ERT-switch patients is also currently enrolling to double the number of patient data in this ambulatory ERT-switch population. 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, 3, and 4 have all received 20 mg/kg ATB200 plus high dose AT2221.

About AT-GAA
AT-GAA is an investigational therapy 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, AT-GAA was associated with increased tissue enzyme levels, reduced glycogen levels in muscle, and improvements in muscle strength. Amicus Therapeutics is currently conducting a global Phase 1/2 study (ATB200-02) to evaluate the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics of AT-GAA.

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. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a robust pipeline of cutting-edge, first- or best-in-class medicines for rare metabolic diseases. For more information please visit the company's website at www.amicusrx.com.

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 AT-GAA for the treatment of Pompe and the potential implications on these data for the future advancement and development of AT-GAA. 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 AT-GAA, 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 AT-GAA. 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, 2017 and Quarterly Report on 10-Q for the Quarter ended June 30, 2018. 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.

1Shoser, *et. al*. **23rd Annual Congress of the World Muscle Society. First-in-human Study of ATB200/AT2221 in Patients With Pompe Disease: 18M Safety and Efficacy Data From the ATB200-02 Trial**

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