Amicus Therapeutics Announces Positive Long-Term Data from Phase 1/2 Study of AT-GAA in Pompe Disease at the 2022 MDA Clinical & Scientific Conference

March 16, 2022

Meaningful and Durable Responses in Key Endpoints of Six-Minute Walk and Forced Vital Capacity for ERT-Naïve and ERT-Experienced Patients Out to 3 Years

Consistent Reduction in Biomarkers Continue to Suggest a Positive Effect on Muscle Tissue

Safety Profile Aligns with Previously Reported Data

Data Further Support Belief That AT-GAA May Rapidly Become the New Standard of Care Treatment Regimen for People Living with Pompe Disease

PHILADELPHIA and NASHVILLE, Tenn., March 16, 2022 (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 adult patients with Pompe disease, an inherited lysosomal storage disorder caused by an enzyme deficiency that leads to accumulation of glycogen (disease substrate) in cells.

Study participants treated with AT-GAA for up to 36 months showed persistent and durable effects on six-minute walk test (6MWT) distance and other measures of motor function and muscle strength, stability or increases in forced vital capacity (FVC), and reductions in biomarkers of muscle damage and disease substrate.

These clinical results are being featured at the 2022 MDA Clinical & Scientific Conference in an oral platform presentation on Wednesday, March 16, 2022, at 12:10pm CT (1:10pm ET). The presentation will be given by Barry Byrne MD, PhD, University of Florida Health and Principal Investigator in the ATB200-02 study.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, stated, “These latest long-term data from our Phase 1/2 study of AT-GAA continue to represent very meaningful and durable improvements in functional outcomes, as well as persistent reductions in key biomarkers of muscle damage and disease substrate out to three years. The results have been shared with the global regulatory authorities in the US and EU as part of their ongoing reviews. Compared with what is known about the natural history of both untreated and ERT-experienced Pompe patients, these results give great hope that AT-GAA indeed has the potential to become the new global standard of care for people living with Pompe disease.”

Dr. Byrne stated, “There remains a need for new treatment options to address multiple aspects of Pompe disease across a broad spectrum of patients, including previously untreated and ERT-experienced patients, as well as those who are non-ambulatory. The results from this Phase 1/2 clinical study of AT-GAA continue to demonstrate a significant effect out to 36 months in adults with Pompe disease, including improvements in six-minute walk distance among ambulatory ERT-experienced and ERT-naïve participants and sustained positive changes in arm and shoulder strength among the non-ambulatory ERT-experienced patients.”

AT-GAA is currently under global regulatory reviews. In the U.S., the Food and Drug Administration (FDA) accepted for review the Biologics License Application (BLA) for cipaglucosidase alfa and the New Drug Application (NDA) for miglustat, the two components of AT-GAA. The FDA has set a Prescription Drug User Fee Act (PDUFA) action date of May 29, 2022 for the NDA and July 29, 2022 for the BLA. In the EU, the Marketing Authorization Applications (MAA) were submitted and validated in the fourth quarter of 2021 by the European Medicines Agency (EMA).

Long-term data from the Phase 3 PROPEL open-label extension study is expected to be presented later this year.

ATB200-02 Study Data Highlights in ERT-Experienced and ERT-Naïve Patients Out to Month 36
Cohorts 1 – 4 (n=28)
Long-term follow-up data up to 36 months were presented for patients enrolled across all four study cohorts, including ERT-experienced ambulatory patients (n=17), ERT-experienced non-ambulatory patients (n=6), and ERT-naïve patients (n=6).

- **Functional Outcomes** (n=23):
  - Motor function: Ambulatory cohorts showed durable mean improvements from baseline that were sustained for up to 36 months of follow-up.
  - Amongst ERT-naïve patients, 6MWT distance increased in 6/6, 5/5, and 4/5 patients at months 12, 24, and 36, respectively. The ERT-naïve patients showed mean increases of 57 meters at month 12 (n=6), 61 meters at month 24 (n=5), and 44 meters at month 36 (n=5).
  - 6MWT distance increased in 13/16, 8/10, and 6/8 ERT-experienced patients at months 12, 24, and 36, respectively. The ERT-experienced patients showed mean increases of 34 meters at month 12 (n=16), 21 meters at month 24 (n=10), and 48 meters at month 36 (n=8).
- **Muscle Strength**: Ambulatory and non-ambulatory patients, including ERT-experienced and ERT-naïve, showed improvements in strength testing as assessed by manual muscle testing (MMT) and improvements were maintained out to 36 months.
- **Pulmonary Function**: Pulmonary function improved in ERT-naïve patients and was generally stable in ERT-experienced

patients.

- In ERT-naïve patients, mean change in percent predicted forced vital capacity (FVC), one of the main measures of pulmonary function in Pompe disease, was +4.5% at month 12 (n=6), +6.8% at month 24 (n=5), and +6.2% at month 36 (n=5).
- In ERT-experienced patients, mean change in % predicted FVC was -1.3% at month 12 (n=16), -0.9% at month 24 (n=10), and -0.4% at month 36 (n=8).

Safety, Tolerability, and Pharmacokinetics/Pharmacodynamics (PK/PD) in Cohorts 1-4 (n=28)

- To date, adverse events have been generally mild and transient.
- The clinical pharmacokinetic profile has been consistent with previously reported data.
- 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 36 continue to suggest a positive effect on muscle tissue.

About ATB200-02 Clinical Study

The primary objectives of the open-label ATB200-02 clinical study is 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); in addition, two more patients were enrolled in Cohort 2. A fourth cohort of six ambulatory ERT-experienced patients was also enrolled, adding to the patient data in the ambulatory ERT-experienced population. Participants 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 all receive 20 mg/kg ATB200 plus high dose AT2221.

About AT-GAA

AT-GAA is an investigational two-component therapy that consists of cipaglucosidase alfa (ATB200), a recombinant human acid alpha-glucosidase (rhGAA) enzyme with optimized carbohydrate structures, particularly bis-phosphorylated mannose-6 phosphate (bis-M6P) glycans, to enhance uptake into cells, administered in conjunction with miglustat (AT2221), a stabilizer of cipaglucosidase alfa. In preclinical studies, AT-GAA was associated with increased levels of the mature lysosomal form of GAA and reduced glycogen levels in muscle, alleviation of the autophagic defect and improvements in muscle strength.

About Pompe Disease

Pompe disease is an inherited lysosomal disorder caused by deficiency of the enzyme acid alpha-glucosidase (GAA). Reduced or absent levels of GAA levels lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. The 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-dedicated 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, and follow us on Twitter and LinkedIn.

Forward Looking Statement

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to 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, including regulatory submissions for AT-GAA and the status of those submissions. There can be no assurance that the FDA or EMA will grant approval for 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 Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully manufacture and 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, 2021. 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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