Amicus Therapeutics Announces Positive Long-Term Data from Phase 3 Open-label Extension Study of AT-GAA in Late-Onset Pompe Disease at the 19th Annual WORLDSymposium™ 2023

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

Consistent Reduction in Biomarkers Continue to Suggest a Positive Effect on Muscle Tissue; Including Participants who Switched from alglucosidase alfa to AT-GAA in the Open-label Extension

Safety Profile Aligns with Previously Reported Data

PHILADELPHIA, Feb. 22, 2023 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a patient-dedicated global biotechnology company focused on developing and commercializing novel medicines for rare diseases, today announced positive results from the global Phase 3 open-label extension (OLE) study (ATB200-07) to investigate the long-term efficacy and safety of AT-GAA in adult patients with late-onset Pompe disease, an inherited lysosomal disorder caused by an enzyme deficiency that leads to the accumulation of glycogen in cells. Study participants treated with AT-GAA for up to 104 weeks showed persistent and durable effects on six-minute walk distance (6MWD), stability in forced vital capacity (FVC), and continued reductions in biomarkers of muscle damage and disease substrate.

These clinical results are being featured at the 19th Annual WORLD*Symposium* ™ 2023 in a poster presentation and an oral platform presentation scheduled for Sunday, February 26, 2023, at 9:00 a.m. ET. The presentation will be given by Benedikt Schoser, MD, Department of Neurology, Ludwig-Maximilians-University of Munich and Principal Investigator in the PROPEL study.

Bradley Campbell, President and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “These open label extension data from our Phase 3 PROPEL study of AT-GAA continue to represent meaningful and durable improvements in functional outcomes, as well as persistent reductions in key biomarkers of muscle damage and disease substrate out to two years. These results give great hope that AT-GAA has the potential to become the new global standard of care for people living with Pompe disease.”

Dr. Schoser stated, “There remains a need for new treatment options to address multiple facets of the Pompe disease spectrum, including previously untreated and ERT-experienced patients living with Pompe disease. Consistent with the Phase 1/2 study and the Phase 3 PROPEL study results, these first long-term results suggest that AT-GAA treatment for up to two years was associated with a durable effect. This new data set could support a long-term treatment benefit in people with late-onset Pompe disease.”

ATB200-07 Study Data Highlights in ERT-experienced and ERT-naïve Patients Out to Week 104 (n=118) Long-term follow-up data up to 104 weeks were presented for ERT-experienced patients (n=90) and ERT-naïve patients (n=28).

- **Motor function (% predicted 6MWD):**
  - ERT-experienced and -naïve patients treated with AT-GAA in the PROPEL study showed durable mean improvements in percent predicted six-minute walk distance (6MWD) from baseline that were sustained through 104 weeks of follow-up.
  - ERT-experienced and -naïve patients who received alglucosidase alfa/placebo in PROPEL and switched to AT-GAA in the OLE showed stability in percent predicted 6MWD throughout the OLE.
- **Pulmonary Function (sitting % predicted FVC):**
  - Pulmonary function remained stable in participants throughout the OLE.
  - ERT-experienced patients treated with AT-GAA throughout remained stable, while patients who received alglucosidase alfa/placebo in PROPEL experienced a decline in sitting % predicted forced vital capacity (FVC) that stabilized after switching to AT-GAA in the OLE.
  - ERT-naïve patients in both treatment groups experienced some decline in FVC during the controlled portion of PROPEL that stabilized in the OLE to week 104.
- **Biomarker (Serum CK and Urine Hex4):**
  - Durable reductions in key biomarkers of muscle damage (creatine kinase or CK) and disease substrate (urine hexose tetrasaccharide or Hex4) across participants treated with AT-GAA for 104 weeks continue to suggest a positive effect on muscle tissue.
  - ERT-experienced and -naïve patients who received alglucosidase alfa/placebo in PROPEL showed a slight increase or stability in serum CK levels to week 52, and a marked decline after switching to AT-GAA in the OLE.
  - ERT-experienced patients who received alglucosidase alfa/placebo in PROPEL experienced an increase in Hex4 and a marked decline after switching to AT-GAA in the OLE.

- ERT-naïve patients experienced a decline in Hex4 levels during PROPEL in both treatment groups that stabilized or declined further during the OLE to week 104.

- **Safety**
  - The safety profile was similar for patients who continued AT-GAA treatment from the start of PROPEL and those who switched from alglucosidase alfa/placebo.
  - Most treatment emergent adverse events were mild to moderate in severity.
  - No new safety signals were identified during the open label extension study.

About AT-GAA

AT-GAA is a two-component therapy that consists of cipaglucosidase alfa, a bis-M6P-enriched rhGAA which facilitates high-affinity uptake through the M6P receptor while retaining its capacity for processing into the most active form of the enzyme, and the oral enzyme stabilizer, miglustat, that's designed to minimize loss of enzyme activity in the blood. In clinical studies, AT-GAA was associated with demonstrated improvements in both musculoskeletal and respiratory measures.

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 lead to accumulation of glycogen in cells, which is believed to result in the clinical manifestations of Pompe disease. 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 and progressive respiratory involvement. Late-onset Pompe disease can be severe and debilitating, including progressive muscle weakness throughout the body, particularly the skeletal muscles and muscles controlling breathing, that worsens over time.

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 diseases. With extraordinary patient focus, Amicus Therapeutics is committed to advancing and expanding a pipeline of cutting-edge, first- or best-in-class medicines for rare diseases. For more information please visit the company's website at www.amicusrx.com, and follow 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 3 open-label extension 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.

CONTACT:

Investors:
Amicus Therapeutics
Andrew Faughnan
Vice President, Investor Relations
afaughnan@amicusrx.com
(609) 662-3809

Media:
Amicus Therapeutics
Diana Moore
Head of Global Corporate Communications
dmoore@amicusrx.com
(609) 662-5079

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