Amicus Therapeutics Announces Regulatory and Clinical Updates for AT-GAA in Pompe Disease

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CRANBURY, N.J., Sept. 10, 2018 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq: FOLD), a global biotechnology company focused on discovering, developing and delivering novel medicines for rare metabolic diseases, announced today regulatory and clinical advancements in its development program AT-GAA for Pompe disease.

During the third quarter, Amicus held a Type C meeting with the United States Food and Drug Administration (FDA) in order to discuss the regulatory path for AT-GAA. Specifically, Amicus sought input on the design of a pivotal study for full approval for AT-GAA, other supplemental clinical studies in Pompe disease patients, and whether Amicus may pursue an Accelerated Approval pathway in the United States at this time. Amicus has now received final written minutes from the Type C meeting, in which the FDA noted “the importance of expediting new treatments to Pompe patients as fast as possible.” Amicus has incorporated key elements of feedback from the FDA, including the Type C meeting, along with the prior scientific advice received from the European Medicines Agency (EMA) and plans to initiate a pivotal study in 2H18.

The planned pivotal study, which will compare AT-GAA to the current standard of care, is expected to enroll approximately 100 total Pompe patients. Amicus intends to include both ERT-switch patients and ERT treatment-naïve patients in this single pivotal study to support full approval. The primary endpoint will be 6-minute walk with a primary treatment period of up to 12 months. Patients will be eligible to enroll directly into the pivotal study without participating in the observational study (POM-003). Patients currently enrolled in study POM-003 will be eligible for the pivotal study as well. Amicus also intends to initiate studies in additional patient populations, including pediatric Pompe patients, in 2019.

With respect to the U.S. regulatory pathway, the FDA also indicated that the current clinical package is not sufficient to support Accelerated Approval. Amicus Therapeutics intends to continue to generate data to support further discussions on a potential pathway for Accelerated Approval with the FDA in 2019, including:

- Data from up to 10 additional ERT-switch patients in a new Cohort 4 as part of the ongoing Phase 1/2 study (data expected in 2019)
- Presentation of longer-term clinical data out to 18-months for the 19 original Phase 1/2 patients (data expected in 2H 2018)
- Completion of a retrospective natural history study in approximately 100 ERT-treated Pompe patients (data expected in 2H 2018)

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “We look forward to initiating the Pompe pivotal trial this year following collaborative discussions with regulators in the U.S. and the EU. We continue to take steps forward in this vitally important program and will significantly enhance the body of clinical data for AT-GAA through the upcoming pivotal study as well as through ongoing clinical studies over the coming months. We look forward in the near term to sharing the latest clinical results from the ongoing Phase 1/2 study at the World Muscle Society in early October. Our commitment remains the same as it has always been since we initiated the development of our Pompe cell line -- to deliver this potential new therapy to as many people living with Pompe disease as soon as possible.”

About AT-GAA (ATB200/AT2221)
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. The cornerstone of the Amicus portfolio is Galafold™ (migalastat), an oral precision medicine for people living with Fabry disease who have amenable *GLA* variants. The lead biologics program in the Amicus Therapeutics pipeline is AT-GAA, an investigational therapy for Pompe disease. 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 relating to clinical development of our product candidates, the timing and reporting of results from clinical trials and the prospects and timing of the potential regulatory approval of our product candidates. The inclusion of forward-looking statements 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 and timing of clinical trials, 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 regulatory authorities including the FDA, EMA, and PMDA may not accept or may modify clinical trial protocols; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, , may not grant or may delay approval for our product candidates; the potential that we may not be successful in commercializing our 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; the potential that we may not be able to manufacture or supply sufficient clinical or commercial products; and the potential that we will need additional funding to complete all of our studies and manufacturing. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. 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, 2017 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018 filed August 7, 2018 with the Securities and Exchange Commission. 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.

GALAFOLD INDICATIONS AND USAGE
Galafold is indicated for the treatment of adults with a confirmed diagnosis of Fabry disease and an amenable galactosidase alpha gene (*GLA*) variant based on *in vitro* assay data.

This indication is approved under accelerated approval based on reduction in kidney interstitial capillary cell globotriaosylceramide (KIC GL-3) substrate. Continued approval for this indication may be contingent upon verification and description of clinical benefit in confirmatory trials.

GALAFOLD U.S. IMPORTANT SAFETY INFORMATION

ADVERSE REACTIONS
The most common adverse reactions reported with Galafold (≥10%) were headache, nasopharyngitis, urinary tract infection, nausea and pyrexia.

USE IN SPECIFIC POPULATIONS
There is insufficient clinical data on Galafold use in pregnant women to inform a drug-associated risk for major birth defects and miscarriage. Advise women of the potential risk to a fetus.

It is not known if Galafold is present in human milk. Therefore, the developmental and health benefits of breastfeeding should be considered along with the mother's clinical need for Galafold and any potential adverse effects on the breastfed child from Galafold or from the underlying maternal condition.

Galafold is not recommended for use in patients with severe renal impairment or end-stage renal disease requiring dialysis.

The safety and effectiveness of Galafold have not been established in pediatric patients.

To report Suspected Adverse Reactions, contact Amicus Therapeutics at 1-877-4AMICUS or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

For additional information about Galafold, including the full U.S. Prescribing Information, please visit https://www.amicusrx.com/pi/galafold.pdf.

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