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

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CRANBURY, N.J., June 26, 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 for AT-GAA for Pompe disease following guidance from regulators in the European Union as well as guidance on manufacturing and bio-comparability from German regulatory authorities (BfArM).

Regulatory & Clinical:

During the second quarter, Amicus met with the Scientific Advice Working Party (SAWP) within the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA). The purpose of the discussions were two-fold: 1) to gain alignment on the design of a pivotal study for full approval for AT-GAA as well as other supplemental clinical studies in Pompe patients; 2) to discuss whether Amicus may pursue a pathway for AT-GAA that includes a conditional marketing approval (CMA) application in Europe. Amicus has concluded this series of interactions and has now received written guidance from the SAWP.

With respect to the pivotal study, Amicus will incorporate SAWP feedback on key elements of the nature and design of the pivotal study. The pivotal study is planned to focus on up to 80 ERT-switch patients with 6-minute walk as the primary endpoint and a primary treatment period up to 12 months. Patients enrolled in the current prospective observational study being conducted by Amicus (STRIDE Study 003) would be entered into the pivotal treatment study. This pivotal study is expected to commence in 2H 2018, pending feedback from the Type C meeting with FDA which is on track for 3Q18.

The SAWP was also supportive of studying additional patient populations, including pediatric Pompe patients and ERT-treatment naïve Pompe patients. Amicus expects to include these patient populations in studies to initiate in 2019, in addition to the pivotal study in ERT-switch patients.

With respect to a pathway for conditional approval, while the SAWP specifically noted that the efficacy data for AT-GAA to date appear “promising” the SAWP indicated that the current clinical package is not sufficient for a Conditional Marketing Authorization Application at this time. Amicus intends to continue a dialogue on a potential pathway for conditional approval with the EMA authorities in 2019 with further data on both efficacy and safety to include:

- 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)

As the clinical data continue to develop, Amicus will also be able to commence the Process Performance Qualification (PPQ) runs at the 1,000 Liter (commercial scale). These PPQ manufacturing runs will be essential for any marketing application under any pathway in both the EU and United States.

Manufacturing Comparability:

In a separate meeting with the German regulatory authorities (BfArM), Amicus received scientific advice indicating general agreement with the manufacturing strategy for ATB200, including on the strategy to demonstrate comparability between drug substance and drug product manufactured at the 1,000 liter scale and drug substance and drug product manufactured at the 250 liter scale.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “We continue to take steps forward in this vitally important program. As we have indicated previously, we also continue to believe that the evolving regulatory path will include a series of further iterative discussions with regulators as the program advances and as additional data are collected. Our commitment remains the same as it has always been since we initiated the development of our Pompe cell line - to deliver this promising new treatment regimen to as many people living with Pompe disease as soon as possible. We look forward to the Type-C meeting with FDA on this program in the third quarter and to incorporating their feedback along with that of the CHMP to initiate the pivotal trial this year.”

About Scientific Advice:
Scientific Advice is a procedure offered by the EMA to stakeholders for clarification of questions arising during development of medicinal products. The scope of Scientific Advice is limited to scientific issues, i.e. to quality, non-clinical and clinical aspects of the concerned medicinal product not yet unequivocally covered by published scientific guidelines. Scientific Advice is legally non-binding and is based on the current scientific knowledge which may be subject to future changes.

About AT-GAA (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.

AT-GAA is an investigational product and is not approved by regulatory authorities in any jurisdiction.

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-dedicated 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 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 and Japan, with additional approvals granted and pending in several geographies. The lead biologics program in the Amicus pipeline is AT-GAA, 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 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 it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities, including the FDA, EMA, and PMDA, 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. 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.

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