November 28, 2016

Amicus Therapeutics Announces U.S. Regulatory Pathway for Migalastat for Fabry Disease

Regulatory Plan for Full Approval Pathway Based on Generation of Additional Gastrointestinal Symptoms Data

Intermediate Expanded Access Program Expected to Begin in 2017

Conference Call Today at 4:30pm ET

CRANBURY, N.J., Nov. 28, 2016 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, announces its planned regulatory pathway to collect additional data to support full approval for the oral precision medicine migalastat for Fabry disease. Following several collaborative discussions with the U.S. Food and Drug Administration (FDA), including the receipt of final written minutes from an in-person Type B meeting, the Company plans to collect additional data on gastrointestinal (GI) symptoms in Fabry patients who have an amenable mutation.

Key elements of the additional data generation required for full approval of migalastat in the United States may include:

- Randomized, 12-month, placebo-controlled pivotal "cross-over" study in treatment naïve Fabry patients who have an amenable mutation and GI symptoms
- Crossover study design provides sufficient powering with a small number of patients (~35 patients planned)
- Primary endpoint to assess diarrhea based upon established FDA irritable bowel syndrome (IBS) guidance[1]
- Amicus is working with FDA to finalize the clinical protocol and plans to initiate enrollment in 2017, with data expected in 2019
- Upon successful NDA filing, the review will be based upon these new data and the totality of the data from all prior migalastat studies
- Intermediate Expanded Access Program (EAP) planned to ensure short-term access to migalastat for U.S. patients who are currently on ERT and meet EAP requirements
- U.S. patients currently enrolled in ongoing clinical extension studies will continue to receive migalastat

During its review of the briefing document submitted and in discussions with Amicus, the FDA acknowledged that significant unmet medical need exists in Fabry disease. The agency also indicated that kidney globotriaosylceramide (GL-3) is currently not considered a basis for an accelerated approval under Subpart H.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "While we believe that the totality of the data from our studies with migalastat support the submission of a new drug application today, we acknowledge the FDA's position that accelerated approval based on kidney GL-3 reduction is not currently an option. We have thus defined a plan to collect additional GI data to support full approval for migalastat that we believe is feasible in a reasonable amount of time and with a high likelihood of success based on positive GI data generated in our previous Phase 3 Study 011. FDA has been flexible in allowing a crossover design and in our use of established GI endpoints to measure clinical benefit in Fabry patients. We are fully committed to the additional work necessary to move migalastat toward approval in the United States."

More than 50% of patients with Fabry disease report or show GI signs and symptoms, including diarrhea, abdominal pain, constipation, nausea, and vomiting.[2] Amicus previously presented positive GI data in a completed Phase 3 randomized, placebo controlled Study 011 (FACETS) in treatment-naïve Fabry patients with amenable mutations. The GI data from this study showed a significant decrease in diarrhea (unadjusted p=0.03) in patients with amenable mutations treated with migalastat versus placebo during the 6-month double-blind phase (Stage 1), which persisted after 18-24 months of treatment with migalastat.

"There are significant unmet needs and a lack of treatment choices for people living with Fabry disease in the U.S.," said Jack Johnson, Founder and Executive Director of the Fabry Support & Information Group (FSIG). "Amicus has been an

outstanding partner for the Fabry community for more than a decade, incorporating the patient's perspective throughout the entire migalastat development program and providing valuable research for the Fabry community. I look forward to the continued advancement of migalastat for patients with amenable mutations in the U.S."

The U.S. market for migalastat is estimated to be approximately 25% of the global opportunity.

Migalastat is designed to selectively and reversibly bind with high affinity to the active sites of certain mutant forms of alpha-Gal A, the genotypes of which are referred to as amenable mutations. On May 30, 2016, the European Commission granted full approval for migalastat, under the trade name Galafold™, as a first line therapy for long-term treatment of adults and adolescents aged 16 years and older with a confirmed diagnosis of Fabry disease (alpha-galactosidase A deficiency) and who have an amenable mutation. This EU approval may serve as the basis for regulatory approvals in more than two-thirds of the global Fabry market that is outside the U.S. Amicus has commenced the commercial launch of Galafold in Germany and is undergoing the EU country-by-country processes to launch in the majority of EU countries throughout 2016 and 2017. The Company has also initiated expanded access programs (EAP) in the EU and other territories outside the U.S. that provide this mechanism for reimbursed access prior to formal approval.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, November 28, 2016 at 4:30 p.m. ET. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./ Canada) or 678-224-7784 (international).

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/events.cfm, and will be archived for 30 days. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 7:30 p.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 28635855.

About Galafold™ and Amenable Mutations

Galafold™ (migalastat) is a first-in-class chaperone therapy approved in the EU as a monotherapy for Fabry disease in patients with amenable mutations. Galafold works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulation of disease substrate in patients who have amenable mutations. A proprietary *in vitro* assay (Galafold Amenability Assay) was used to classify more than 800 known GLA mutations as "amenable" or "not amenable" to treatment with Galafold. The current EU label includes 313 GLA mutations that have been identified and determined to be amenable based on the Galafold Amenability Assay, which represent between 35% and 50% of the currently diagnosed Fabry population.

Healthcare providers in the EU may access the website www.galafoldamenabilitytable.com to quickly and accurately identify which mutations are categorized as "amenable" or "not amenable" to Galafold. Amicus expects to submit updates to the label as additional GLA mutations are identified and tested in the Galafold Amenability Assay.

Important Safety Information from the EU Approval

Treatment with GALAFOLD should be initiated and supervised by specialists experienced in the diagnosis and treatment of Fabry disease. GALAFOLD is not recommended for use in patients with a nonamenable mutation.

- GALAFOLD is not intended for concomitant use with enzyme replacement therapy.
- GALAFOLD is not recommended for use in patients with Fabry disease who have severe renal impairment (< 30 mL/min/1.73 m^2). The safety and efficacy of GALAFOLD in children 0-15 years of age have not yet been established.
- No dosage adjustments are required in patients with hepatic impairment or in the elderly population.
- There is very limited experience with the use of this medicine in pregnant women. If you are pregnant, think you may be pregnant, or are planning to have a baby, do not take this medicine until you have checked with your doctor, pharmacist, or nurse.
- While taking GALAFOLD, effective birth control should be used. It is not known whether GALAFOLD is excreted in human milk.
- Contraindications to GALAFOLD include hypersensitivity to the active substance or to any of the excipients listed in the PRESCRIBING INFORMATION.
- It is advised to periodically monitor renal function, echocardiographic parameters and biochemical markers (every 6 months) in patients initiated on GALAFOLD or switched to GALAFOLD.
- OVERDOSE: General medical care is recommended in the case of GALAFOLD overdose.
- The most common adverse reaction reported was headache, which was experienced by approximately 10% of patients who received GALAFOLD. For a complete list of adverse reactions, please review the SUMMARY OF PRODUCT CHARACTERISTICS.
- Call your doctor for medical advice about side effects.

For further important safety information for Galafold, including posology and method of administration, special warnings, drug interactions and adverse drug reactions, please see the European SmPC for Galafold available from the EMA website at www.ema.europa.eu.

About Fabry Disease

Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A), which is the result of mutations in the GLA gene. The primary biological function of alpha-Gal A is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb_3). Lipids that can be degraded by the action of alpha-Gal A are called "substrates" of the enzyme. Reduced or absent levels of alpha-Gal A activity lead to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. Progressive accumulation of GL-3 is believed to lead to the morbidity and mortality of Fabry disease, including pain, kidney failure, heart disease, and stroke. The symptoms can be severe, differ from patient to patient, and begin at an early age. All Fabry disease is progressive and may lead to organ damage regardless of the time of symptom onset.

About Amicus Therapeutics

Amicus Therapeutics (Nasdaq:FOLD) is a global biotechnology company at the forefront of therapies for rare and orphan diseases. The Company has a robust pipeline of advanced therapies for a broad range of human genetic diseases. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, SD-101 for Epidermolysis Bullosa (EB), as well as novel enzyme replacement therapy (ERT) and biologic products for Fabry disease, Pompe disease, and other rare and devastating diseases.

[1] http://www.fda.gov/downloads/Drugs/.../Guidances/UCM205269.pdf

[2] Hoffmann B *et al.* **Clin Gastroenterol Hepatol**. 2007;5(12):1447-1453.

Forward-Looking Statements

This press release contains "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to preclinical and clinical development of our product candidates, the timing and reporting of results from preclinical studies and clinical trials, the prospects and timing of the potential regulatory approval of our product candidates, commercialization plans, financing plans, and the projected cash position for the Company. 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, timing, and results of preclinical studies and clinical trials, actual results 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 Galafold in Europe or our other 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; and the potential that we will need additional funding to complete all of our studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. 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, 2015 and Quarterly Report on Form 10-Q for the quarter ended September 30, 2016. 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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