February 14, 2017

Amicus Therapeutics Highlights New Fabry Program Data at WORLDSymposium™ 2017

New Phase 3 Retrospective Analysis Shows Correlation between Reduction in Disease Substrate (Kidney Interstitial Capillary GL-3) and Improved Diarrhea in Fabry Patients with Amenable Mutations treated with Migalastat

Supportive Study for Japanese New Drug Application (J-NDA) Demonstrates Migalastat Exposure is Similar in Japanese and non-Japanese Individuals

SAN DIEGO and CRANBURY, N.J., Feb. 14, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, today announced new positive data analyses for the oral small molecule pharmacological chaperone migalastat HCl ("migalastat") for Fabry disease at WORLD*Symposium*™ 2017 in San Diego, California.

Jay Barth, Chief Medical Officer of Amicus Therapeutics, Inc., stated, "The new analyses highlighted at this year's WORLD*Symposium* add to the already significant body of data which demonstrate the multiple benefits of treatment with migalastat in Fabry patients with amenable mutations. Here, the correlation of the reduction in disease substrate with the reduction in diarrhea symptoms provides further evidence that migalastat is having a positive effect on an important gastrointestinal symptom in Fabry. These findings in diarrhea symptoms support our current Fabry regulatory strategy for migalastat in the U.S., which is based upon improvement in diarrhea in this patient population. Also, we remain on track in Japan with our submission to the Pharmaceuticals and Medical Devices Agency based on completed Phase 1 and Phase 3 studies. We also remain committed to providing access to this important medicine to patients in the EU, where it is already approved, in addition to other major geographies."

Data Highlights for Migalastat for Fabry Disease at WORLD*Symposium* 2017

Phase 3 Retrospective Analysis - Correlation between Substrate Reduction and Reduction in Diarrhea

In a poster[1] from Study 011 (FACETS) in Fabry patients who were naïve to ERT, a retrospective analysis from baseline to month 6 demonstrated that migalastat reduces disease substrate (KIC GL-3) and improves diarrhea symptoms (GSRS-D) in patients with Fabry disease with amenable mutations. Key highlights were as follows:

- Migalastat demonstrated a statistically significant reduction in the combined endpoint of disease substrate (KIC GL-3 and diarrhea (GSRS-D) (p=0.009; 1-sided)
- Reduction in disease substrate was significantly correlated with an improvement in diarrhea
- 83% (15/18) of migalastat-treated patients demonstrated a reduction in disease substrate and/or improvement in diarrhea when either or both were elevated at baseline compared with 33% (5/15) of patients treated with placebo

To support full approval in the U.S., which represents approximately 25% of the global Fabry market, Amicus plans to confirm the clinical beneficial effects of migalastat in a GI symptom study. The GI study is anticipated to begin in 2017 in approximately 35 Fabry patients who are naïve to treatment and who have an amenable mutation and diarrhea and other GI symptoms. More than 50% of patients with Fabry disease report or show GI signs and symptoms, including diarrhea, abdominal pain, constipation, nausea, and vomiting.[2]

Supportive Pharmacokinetics (PK) Study for Japanese Regulatory Submission

As previously announced, Amicus plans to submit a J-NDA in Japan in the first half of 2017. The J-NDA will be based on data from completed clinical studies with migalastat, including two pivotal Phase 3 studies as well as a Phase 1 study that evaluated the pharmacokinetics (PK) of migalastat in Japanese volunteers. The results from this Phase 1 study are being highlighted in a poster[3] at WORLD*Symposium* 2017 with key highlights as follows:

- Exposure to migalastat was similar between Japanese and non-Japanese healthy volunteers
- The PK properties of migalastat were not different between Japanese and non-Japanese patients with Fabry disease

Japan represents the second largest Fabry market in the world by country, with approximately 13% of the $1.2B global Fabry ERT sales generated in Japan in 2015.[4] The Pharmaceuticals and Medical Devices Agency (PMDA) in Japan

previously confirmed that completed studies of migalastat meet J-NDA submission requirements without the need to conduct an additional clinical study in Japan. The PMDA took into account data from Japanese patients included in the Phase 3 program and the similar PK properties in Japanese and non-Japanese individuals.

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.

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

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]D. Germain, **WORLD*Symposium* 2017**, Effects of Treatment With Migalastat on the Combined Endpoint of Kidney Globotriaosylceramide Accumulation and Diarrhea in Patients With Fabry Disease: Results From the Phase 3 FACETS Study

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

[3]F. Johnson, **WORLD*Symposium* 2017**, Migalastat exposures in Japanese healthy volunteers and non-Japanese subjects provide evidence that they are similar to Japanese patients with Fabry disease

[4]Company filings and Amicus estimates

Forward Looking Statements

This press release contains "forward- looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995, including statements relating to data, future studies and ongoing regulatory strategies for migalastat. 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 data reported herein will not be predictive of future results, that later study results will not support further development of migalastat, or even if such later results are favorable, that the Company will not be able to successfully complete the development of, obtain regulatory approval for, or successfully commercialize migalastat. 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, 2015 and Quarterly Report on 10-Q for the Quarter ended September 30, 2016. As a consequence, actual results may differ materially from those set forth in this press release or the accompanying conference call or webcast. 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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Source: Amicus Therapeutics, Inc

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