November 11, 2016

Journal of Medical Genetics Publishes Pivotal Phase 3 ATTRACT Study of Migalastat for Patients with Fabry Disease

CRANBURY, N.J., Nov. 11, 2016 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, today announced that data from the pivotal Phase 3 Study 012 (ATTRACT) evaluating the efficacy and safety of the oral pharmacological chaperone migalastat compared with Enzyme Replacement Therapy (ERT) in individuals with Fabry disease were published online in the *Journal of Medical Genetics*[1].

"We are honored that the Journal of Medical Genetics has published our pivotal Phase 3 ATTRACT study in patients with Fabry disease who were switched from ERT to migalastat," said Jay Barth, MD, Chief Medical Officer of Amicus Therapeutics, Inc. "We believe that this 18-month, randomized, active-controlled study generated a strong clinical data set for our precision medicine migalastat. This Phase 3 study, together with our Phase 3 Study 011 in Fabry patients who were naïve to treatment, supported the European Commission's full approval for migalastat in the European Union as a first line therapy for Fabry disease in patients 16 years and older who have an amenable genetic mutation. This publication is a major accomplishment, and I would like to thank the physicians who were investigators in this study, as well as the patients and families who participated in the study."

Prof. Ulla Feldt-Rasmussen, Chief of Medical Endocrinology, Copenhagen University Hospital stated, "I have been treating patients with Fabry disease for 16 years, and I believe that significant unmet needs remain. Migalastat is an oral, first-in-class precision medicine with a unique mechanism of action that is based on a patient's genotype. As a lead author of the journal publication, as well as a principal investigator in Denmark for the migalastat clinical studies for seven years, I believe that migalastat is poised to become an important, differentiated treatment option for patients with Fabry disease who have an amenable mutation."

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. Amicus commenced the commercial launch on May 31, 2016 following the European Commission's 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.

About ATTRACT (Study 012)

Study 012 was a Phase 3 open-label study that compared oral migalastat to standard-of-care Enzyme Replacement Therapies (ERTs) for Fabry disease (agalsidase alfa or beta). The study enrolled 60 patients (26 males and 34 females) with Fabry disease with amenable mutations in a clinical trial assay who had been treated with ERT for a minimum of 12 months prior to study entry. These patients were randomized 1.5:1 to switch to migalastat (36 patients) or remain on ERT (24 patients) for the primary 18-month treatment period. The co-primary outcome measures were the mean annualized changes in estimated glomerular filtration rate (eGFR) and measured (iohexol) GFR (mGFR) assessed by descriptive comparisons of migalastat and ERT over 18 months. Secondary outcome measures included left ventricular mass index (LVMi), as well as a composite of Fabry-associated clinical events (i.e. renal, cardiac, or cerebrovascular). Upon completion, patients were eligible to roll over into a separate extension to continue migalastat.

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 all 269 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. The Committee for Medicinal Products for Human Use (CHMP) agreed to 44 new amenable mutations and the EU label is being updated to include a total of 313 amenable mutations

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.

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.

[1]Hughes, *et al.* Oral Pharmacological Chaperone Migalastat Compared With Enzyme Replacement Therapy in Fabry Disease: 18-Month Results from the Randomized Phase 3 ATTRACT Study, J Medical Genetics, online publication 10 November 2016. doi:10.1136/jmedgenet- 2016-104178

CONTACTS:

Investors/Media:
Amicus Therapeutics
Sara Pellegrino
Senior Director, Investor Relations
spellegrino@amicusrx.com
(609) 662-5044

Media:
MWW PR
Sean Conley
sconley@mww.com
(646) 381-9096

FOLD-G


Primary Logo

Source: Amicus Therapeutics, Inc

News Provided by Acquire Media