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# Amicus Therapeutics Submits Japanese New Drug Application for Migalastat for Fabry Disease

## Represents World's Second Largest Diagnosed Global Fabry Patient Population

CRANBURY, N.J., June 28, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of rare and orphan diseases, has submitted a Japanese new drug application (J-NDA) to the Pharmaceuticals and Medical Devices Agency (PMDA) to request marketing authorization for migalastat, an oral precision medicine for Fabry disease.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, "On the heels of our initial commercial launch success for migalastat in the EU, we are pleased to submit our Japanese new drug application for migalastat. With more than 700 Fabry patients currently treated in Japan, we believe that a substantial number of Japanese Fabry patients could potentially benefit from migalastat as a differentiated precision oral therapy with a unique mechanism of action. Japan is a key part of our patient-focused vision to provide migalastat to amenable Fabry patients throughout the world as soon as possible. We look forward to working collaboratively with the PMDA toward a potential approval in the coming months."

Based on a previous meeting with and written correspondence from the PMDA, the J-NDA is based upon data from completed clinical studies with migalastat, including two pivotal Phase 3 studies, as well as a Phase 1 study that evaluated the pharmacokinetics of migalastat in Japanese volunteers. Migalastat has orphan drug designation in Japan which makes it eligible for priority review as well as 10 years of market exclusivity, if approved. Based on the priority review timeline, Amicus expects a decision from the PMDA in the first half of 2018.

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 2016.[1] Fabry disease is a rare genetic disease and potentially life-threatening condition caused by the accumulation of disease substrate (globotriaosylceramide, GL-3) in the lysosome due to a dysfunctional or deficient enzyme. Migalastat works by stabilizing the body's own dysfunctional enzyme, so it can clear the accumulated disease substrate in patients who have amenable mutations. An amenable mutation is one that is responsive to therapy with migalastat based on a proprietary *in vitro* assay (Galafold Amenability Assay). Amicus estimates that 35%-50% of Fabry patients globally may have amenable genetic mutations, and amenability rates within this range vary by geography.

Migalastat is currently reimbursed in 11 countries in the EU and other parts of the world, on a commercial basis or through expanded access programs (EAPs). 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. The EC approval was based on clinical data from two Phase 3 pivotal studies in both treatment-naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT), as well as ongoing long-term extension studies.

**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 global 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 additional 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) products for Fabry disease, Pompe disease, and other lysosomal storage disorders.

[1]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 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, 2016 and the Quarterly Report on Form 10-Q for the quarter ended March 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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CONTACTS:

Investors/Media:

Amicus Therapeutics

Sara Pellegrino, IRC

Senior Director, Investor Relations

spellegrino@amicusrx.com

(609) 662-5044


Primary Logo

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