Amicus Therapeutics’ Fabry Monotherapy Granted Accelerated Assessment by European Regulators

First Investigational Therapy for Fabry Disease to be Granted Accelerated Assessment

Planned MAA Submission on Track for 2Q15 to Request Full Approval in European Union

CRANBURY, NJ May 26, 2015 – Amicus Therapeutics (Nasdaq: FOLD), a biopharmaceutical company at the forefront of therapies for rare and orphan diseases, today announced that the Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has granted Accelerated Assessment to the oral small molecule pharmacological chaperone migalastat HCl ("migalastat") monotherapy for Fabry patients who have amenable genetic mutations.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc., stated, “The designation of Accelerated Assessment in the European Union (EU) demonstrates that the EMA understands the current unmet medical need in Fabry disease as a major public health interest, and with this designation may accelerate the approval and our launch timelines to make migalastat available for patients very rapidly. This is excellent news for so many citizens in the EU and in other geographies around the world. As part of our global strategy, we also plan to submit our new drug application for U.S. approval in the second half of this year. We are committed to getting this personalized medicine approved as quickly as possible for Fabry patients with amenable genetic mutations around the world."

Migalastat is the first investigational Fabry drug to be granted Accelerated Assessment. Amicus requested Accelerated Assessment in accordance with the EMA guidelines, which justify Accelerated Assessment for therapies that are expected to be of major public health interest and therapeutic innovation, addressing the greater unmet needs for maintaining and improving the health of the Community.¹ Over the past three years, positive opinions were granted after an Accelerated Assessment for a total of just 14 therapies.² The most recent innovative treatment options to have been granted Accelerated Assessment include therapies for rare diseases such as Morquio A Syndrome (MPS IVA), Lysosomal Acid Lipase Deficiency (LAL D), and Cystic Fibrosis (CF).

Under Accelerated Assessment, the CHMP may shorten the Marketing Authorisation Assessment (MAA) review period from 210 days, under standard review, to 150 days under Accelerated Assessment. The CHMP opinion is then reviewed by the European Commission, which generally issues a final decision on EU approval within three months. If approved, Amicus would then begin the country-by-country reimbursement approval process. Amicus is on track to submit the MAA to request full approval for migalastat monotherapy in the EU in the second quarter of 2015.

Amicus previously reported positive Phase 3 data for migalastat in both treatment naïve (Study 011, or FACETS) and enzyme replacement therapy (ERT) switch patients (Study 012, or ATTRACT). Results from these studies have shown that treatment with migalastat has resulted in reductions in disease substrate, stability of kidney function, reduction in cardiac mass, and a positive impact on patient-reported outcomes in patients with amenable mutations.

About Fabry Disease
Fabry disease is an inherited lysosomal storage disorder caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A, referred to here as alpha-Gal). The primary biological function of alpha-Gal is to degrade specific lipids in lysosomes, including globotriaosylceramide (referred to here as GL-3 and also known as Gb3). Lipids that can be degraded by the action of alpha-Gal are called “substrates” of the enzyme. Reduced or absent levels of alpha-Gal activity lead to the accumulation of GL-3 in the affected tissues, including the central nervous system, heart, kidneys, and skin. This accumulation of GL-3 is believed to cause the various symptoms of Fabry disease, including pain, kidney failure, and increased risk of heart attack and stroke. It is currently estimated that Fabry disease affects approximately 5,000 to 10,000 people worldwide.

About Amicus Therapeutics
Amicus Therapeutics (Nasdaq:FOLD) is a biopharmaceutical company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage diseases. Amicus’ lead programs in development
including the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, as well as next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS I.


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 Amicus’ candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus’ candidate drug products and the projected cash position for the Company. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “potential,” “plan,” “targets,” “likely,” “may,” “will,” “would,” “should” and “could,” and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus 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 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 the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical 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 may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical 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, 2014. 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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