



## **Amicus Therapeutics Announces Positive Preliminary Results from Ongoing Phase 2 Chaperone-Enzyme Replacement Therapy (ERT) Study for Fabry Disease**

### ***Co-Administration Increases Levels of Active Enzyme Compared to ERT Alone in First Six Patients***

**CRANBURY, NJ, USA, January 6, 2012** – Amicus Therapeutics (Nasdaq: FOLD), today announced preliminary results from an ongoing, open-label Phase 2 drug-drug interaction study ([Study 013](#)) to evaluate the safety and pharmacokinetic (PK) effects of two doses of migalastat HCl (150 mg and 450 mg) co-administered with ERT (agalsidase beta or agalsidase alfa) in up to 24 males diagnosed with Fabry disease.

Amicus and GSK are developing migalastat HCl, an investigational oral pharmacological chaperone, as part of a global collaboration for Fabry disease. Migalastat HCl is in Phase 3 development ([Study 011](#) and [Study 012](#)) for use as a monotherapy for patients with Fabry disease identified as having alpha-galactosidase A mutations amenable to chaperone therapy.

When co-administered with ERT, migalastat HCl is designed to bind to and stabilize the enzyme in the circulation, in any patient receiving ERT. In preclinical studies, the co-administration of migalastat HCl and ERT led to stabilization of the ERT and increased uptake of active enzyme into key organs of disease, including kidney, heart, and skin, when compared to ERT alone. This increased enzyme uptake in Fabry mouse models also led to further reductions in globotriaosylceramide (GL-3), the substrate that accumulates in kidney, heart and skin in Fabry disease.

Data are currently available for the first six subjects in Study 013, who received their current dose and regimen of agalsidase beta alone at one infusion followed by oral migalastat HCl 150 mg administered two hours prior to agalsidase beta at their next infusion. Due to the supply shortage of agalsidase beta, four of these subjects had been receiving 0.5 mg/kg infused every two weeks and two subjects had been receiving a dose of 1.0 mg/kg infused every four weeks.

#### **Preliminary Results - Migalastat HCl 150 mg Co-Administered with Agalsidase Beta (n=6)**

- Increases in levels of active enzyme in plasma and skin demonstrate a positive drug-drug interaction between migalastat HCl 150 mg and agalsidase beta
- In the four patients who received agalsidase beta at 0.5 mg/kg co-administered with migalastat HCl 150 mg, levels of active enzyme in plasma ranged from 2.0 to 4.2-fold higher than with ERT alone, as measured by total area under the curve (AUC). In skin biopsies from three patients, increases in levels of active enzyme in the skin ranged from

1.1 to 3.9-fold higher at day two, but no higher at day seven, following co-administration compared to ERT alone.

- In the two patients who received agalsidase beta at 1.0 mg/kg co-administered with migalastat HCl 150 mg, levels of active enzyme in plasma were 1.6 and 2.2-fold higher than with ERT alone, as measured by total AUC. In skin biopsies, increases in levels of active enzyme in the skin were 1.6 and 2.1-fold higher at day two, and 1.2 and 1.7-fold higher at day seven, following co-administration compared to ERT alone.
- Preliminary data to be presented as a “late breaking” abstract at 8<sup>th</sup> Annual Lysosomal Disease Network WORLD Symposium ([LDN WORLD](#)) in San Diego, February 8-10, 2012.
- Independent data safety monitoring board approved dose escalation to migalastat HCl 450 mg as per study protocol. Amicus and GSK expect to complete Study 013 in the first half of 2012.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics said, “These very encouraging data in Fabry patients represent an important first step in validating the potential of pharmacological chaperones to enhance the stability and tissue uptake of enzyme replacement therapy products in Fabry disease. We look forward to completing Study 013, including obtaining data on the higher dose of migalastat HCl as well as data on co-administration with agalsidase alfa. We also look forward to evaluating the complete data set and collaborating with GSK on the best path forward for extending this co-administration use of our core technology as a treatment option for people living with Fabry disease.”

### **About Amicus Therapeutics**

Amicus Therapeutics (Nasdaq: FOLD) is a biopharmaceutical company at the forefront of developing therapies for rare diseases. The Company is developing orally-administered, small molecule drugs called pharmacological chaperones, a novel, first-in-class approach to treating a broad range of diseases including lysosomal storage disorders and diseases of neurodegeneration. Amicus' lead program migalastat HCl is in Phase 3 for the treatment of Fabry disease.

### **About Migalastat HCl**

Migalastat HCl is an investigational oral pharmacological chaperone for the treatment of Fabry disease being developed in collaboration with GlaxoSmithKline (GSK). Under the terms of the collaboration, GSK has an exclusive worldwide license to develop, manufacture and commercialize migalastat HCl. Amicus and GSK are conducting two Phase 3 global registration studies ([Study 011](#) and [Study 012](#)) of migalastat HCl monotherapy, along with a Phase 2 study

([Study 013](#)) evaluating migalastat co-administered with enzyme replacement therapy (ERT) for the treatment of Fabry disease.

## **About Fabry Disease**

Fabry disease is an inherited lysosomal storage disease that is currently estimated to affect approximately 5,000 to 10,000 people worldwide. Fabry Disease is caused by deficiency of an enzyme called alpha-galactosidase A (alpha-Gal A). The role of alpha-Gal A within the body is to break down a complex lipid called globotriaosylceramide (GL-3). Reduced or absent levels of alpha-Gal A activity leads 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 disorders and stroke.

## **Forward-Looking Statements**

This press release contains “forward-looking statements” within the meaning of the Private Securities Litigation Reform Act of 1995 relating to clinical development of Amicus’ candidate drug products and the timing and reporting of results from clinical trials evaluating Amicus’ candidate drug products. Words such as, but not limited to, “look forward to,” “believe,” “expect,” “anticipate,” “estimate,” “intend,” “plan,” “targets,” “likely,” “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 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. 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, 2010. 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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