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### Study Update: Phase 3, The FACETS Study



While the majority of patients have been enrolled in the ongoing FACETS Study (AT1001-011), this trial is *still recruiting patients* at 38 sites worldwide and is now expected to complete enrollment in the first half of 2011. Amicus plans to report top-line results in the second half of 2011.

As a reminder, among those potentially eligible for the FACETS Study are males and females with Fabry disease, ages 16-74, with confirmed AT1001-responsive *GLA* mutations, who have never received enzyme replacement therapy (ERT) or have not received ERT for 6 months before the first study visit. Additional information, including detailed inclusion criteria, can be obtained from your healthcare provider, by contacting a study site, calling toll-free at 1-888-55-FABRY, visiting the FACETS website at [www.fabrystudy.com](http://www.fabrystudy.com) or [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and searching for “Fabry AT1001-011” or by emailing [clinicaltrials@amicustherapeutics.com](mailto:clinicaltrials@amicustherapeutics.com).

The FACETS Study is divided into two stages. In the first stage, participants receive either placebo (a sugar pill) or study drug (AT1001) for 6 months. In Stage 2, all participants receive AT1001 for an additional 6 months, with an optional 12-month treatment extension period offered to participants who complete both Stages 1 and 2.

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### Study Update: Phase 3, The ATTRACT Study

## THE ATTRACT STUDY

AT1001 Therapy Compared to Enzyme Replacement in Fabry Patients with AT1001-responsive Mutations: a Global Clinical Trial

Investigational study site initiation progresses for the ATTRACT Study (AT1001-012), Amicus' global Phase 3 clinical trial assessing the safety and efficacy of AT1001 in individuals currently receiving ERT.

The ATTRACT Study will enroll males and females with Fabry disease, ages 16-74, with confirmed AT1001-responsive *GLA* mutations who have initiated treatment with ERT at least 12 months prior to the first study visit. Individuals must be on a stable ERT regimen for 3 months prior to the first study visit and receiving at least 80 percent of the currently labeled dose during this time. In the United States, participants must be receiving Fabrazyme<sup>®</sup>. Outside the U.S., participants can be receiving either Fabrazyme or Replagal<sup>®</sup>. In the ATTRACT Study, half of the 60 participants will remain on their physician-prescribed ERT regimen while the other half will be switched onto AT1001, Amicus' investigational oral drug. The treatment period will be approximately 18 months with an optional 12-month extension period during which AT1001 will be offered to all participants. No biopsies are part of the ATTRACT Study protocol.

**More detailed inclusion criteria and additional information is available by speaking to your healthcare provider, visiting [www.fabrystudy.com](http://www.fabrystudy.com) or [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and searching for “Fabry AT1001-012” or contacting Amicus at [clinicaltrials@amicustherapeutics.com](mailto:clinicaltrials@amicustherapeutics.com)**

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## **Study Update: AT1001-013 Co-Administration Therapy (AT1001 + ERT)**

The first patient was recently dosed in the Phase 2 clinical trial designed to evaluate the safety and efficacy of co-administering the pharmacological chaperone AT1001 with enzyme replacement therapy (ERT). Five sites will enroll a total of 18 participants in the 013 Study. Preliminary results are expected to be released in the second half of 2011.

Participants in the study will receive one oral dose of AT1001 approximately two hours before their regular ERT infusion. Participation in the study may last up to four months. Study participants include males with Fabry disease 18-65 years of age, who are on a stable (even if not a full) ERT dose for at least one month. U.S. participants must be on Fabrazyme, whereas ex-U.S. participants may be on either Replagal or Fabrazyme. Participants in this Phase 2 study will not need to have an AT1001-responsive *GLA* mutation. Participation in this study does not preclude participation in future longer-term ERT plus AT1001 co-administration studies.

**For more detailed inclusion criteria and other information, please visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) and search for “Fabry AT1001-013” or contact [clinicaltrials@amicustherapeutics.com](mailto:clinicaltrials@amicustherapeutics.com)**

Amicus has presented encouraging results from its preclinical co-administration experiments at scientific and medical conferences and symposia. Recently, posters on preclinical co-administration experiments were presented at the Annual Society for Human Genetics 60<sup>th</sup> Annual Conference in November 2010, and also in February 2011 at the Lysosomal Disease Network WORLD Symposium in Las Vegas.

On March 19<sup>th</sup>, 2011, Amicus Therapeutics, will present additional pre-clinical data during a platform presentation “Pharmacological Chaperones Increase the Stability of ERTs and Lead to Greater Uptake and Substrate Reduction in Patient-derived Cells and Mouse Models of LSDs” at the 20th American College of Medical Genetics Annual Conference, in Vancouver, Canada.

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## **Amicus Partnership with GlaxoSmithKline**

In 2010, GlaxoSmithKline (GSK) announced the formation of the company’s new rare disease division and commitment to the rare disease community. Marc Dunoyer, Global Head of GSK Rare Diseases, stated, “In addition to our existing discovery effort, alternative opportunities need to be explored to make treatments available for rare diseases ... this new unit has the potential to deliver multiple therapies responding to high medical needs of underserved populations of patients.” GSK’s commitment to rare diseases and unique interest in learning more about those who make up the rare disease community was a strong basis of the Amicus/GSK Fabry partnership. In October 2010, GSK and Amicus announced an agreement to develop and commercialize AT1001 (migalastat hydrochloride), currently in Phase 3 for the treatment of Fabry disease. In this partnership, Amicus can utilize GSK’s clinical, regulatory, and commercial expertise and Amicus can help GSK better understand the rare diseases community. John Crowley, Chairman and CEO of Amicus Therapeutics, stated in a press release announcing the collaboration, “the completion of this agreement with GSK is a transformational event for Amicus. It provides a strong validation of the potential for AT1001 to become an important new treatment option for people living with Fabry disease and for our pharmacological chaperone technology broadly. GSK has extremely impressive global clinical, regulatory and commercial expertise and a strong

commitment to the development of treatments for rare diseases. We look forward to working in close partnership with them.”

Under the terms of the agreement, GSK received an exclusive worldwide license to develop, manufacture and commercialize AT1001. Additionally, as part of the agreement, GSK and Amicus are advancing clinical studies exploring the co-administration of AT1001 with ERT for the treatment of Fabry disease.

Since announcing the partnership, Amicus and GSK teams have been working together to advance the development of AT1001.

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## Fabry Patient Advisory Board Addresses Community Concerns

Fabry Patient Advisory Board (PAB) members from the U.S. and Canada gathered in New Jersey in December 2010 for their third round of meetings. The group convenes regularly to provide the company with important insights from the patient community. Discussions focused on details about the Amicus/GSK Rare Diseases partnership for Fabry disease, updates on Amicus’ three AT1001 studies, and input from the PAB around packaging, clinical research, general outreach and education in the Fabry community. Additionally, the PAB discussed concerns in the patient community regarding treatment, access to care, and conferences and meeting structure. Amicus Patient Advocacy also organizes periodic PAB meetings for the Pompe and Gaucher communities.

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## Key Role of Amicus at Professional Medical and Community Meetings

During the second half of 2010, Amicus participated in a variety of Fabry meetings for patients and healthcare professionals such as the regional Fabry family meetings in New Jersey, Kentucky, Chicago, and California. Amicus was well represented at the Society of the Study of Inborn Errors of Metabolism (SSIEM) Conference in Turkey, the American Society of Human Genetics (ASHG) 60<sup>th</sup> Annual Meeting in Washington, D.C., the National Society of Genetic Counselors (NSGC) and the American Society of Nephrology (ASN) meetings in Texas. In December, an educational gathering of GADYTEF and AADELFA, the two main groups of Fabry physicians in Argentina, along with other specialty and referring physicians discussed Fabry treatments.

Amicus’ participation in the global Fabry community continues in the first half of 2011. This February, Amicus was well represented at the Lysosomal Disease Network WORLD Symposium in Las Vegas with platform presentations and multiple posters, the Renal Pathology Society (RPS) meeting, the Society for Inherited Metabolic Disorders (SIMD), and the Fabry International Network (FIN) Annual General Meeting and Conference. Amicus will play a key role at other professional conferences, including the American College of Medical Genetics (ACMG) 20<sup>th</sup> anniversary conference and at many meetings planned throughout the Fabry patient community.

**If you are interested in a regional meeting for individuals with Fabry, their family members and healthcare professionals in your area, please contact [patientadvocacy@amicustherapeutics.com](mailto:patientadvocacy@amicustherapeutics.com)**

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