April 3, 2017

Amicus Therapeutics Completes Enrollment in ESSENCE Phase 3 Epidermolysis Bullosa Clinical Study

Target Exceeded with More than 160 Patients Enrolled

Top-Line Data on Track for 3Q17

CRANBURY, N.J., April 03, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a global biotechnology company at the forefront of therapies for rare and orphan diseases, has completed enrollment in the ongoing Phase 3 clinical study (ESSENCE) of the novel topical medicine SD-101 for patients with all 3 major types of epidermolysis bullosa (EB) (Simplex, Recessive Dystrophic, and Junctional non-Herlitz EB). With the achievement of full enrollment, top-line data from this study are expected in the third quarter of 2017.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "The completion of enrollment in our global Phase 3 ESSENCE study of SD-101 for epidermolysis bullosa is a significant accomplishment for our team at Amicus as well as for the EB community. This is the most advanced clinical study for EB, and we look forward to announcing top-line data from this study in the third quarter of this year."

The FDA has granted Breakthrough Therapy designation for SD-101 based on results from a Phase 2a study for the treatment of lesions in patients suffering with EB. SD-101 is the first-ever treatment in clinical studies to show improvements in wound closure across all major EB types.

John C. Browning, Chief of Dermatology at Children's Hospital of San Antonio, stated, "The full enrollment in this Phase 3 trial is a significant step forward as we look to advance new wound healing treatments for EB. With no currently approved treatment options, there is an urgent need among people living with EB, as well as their caregivers. There has been tremendous commitment among patients and families, advocacy organizations and study investigators in working alongside Amicus to raise awareness of and drive enrollment in this important study."

The ESSENCE Study is a Phase 3 double-blind, placebo-controlled study that enrolled more than 160 patients who have a documented diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB. To date, more than 95 percent of patients completing the 3-month primary treatment period have elected to continue in the open-label extension study.

About Epidermolysis Bullosa (EB)

EB is a rare, genetic disorder that manifests as blistering or erosion of the skin, and, in some cases, the epithelial lining of other organs. EB is chronic, potentially disfiguring, and in some cases fatal. Individuals with EB have painful wounds and blisters that can lead to infection and scarring. There are many genetic and symptomatic variations of EB, but all forms share the common symptom of fragile skin that blisters and tears, sometimes from the slightest friction or trauma. There is currently no approved treatment for EB. Current standard of care consists of pain management and the cleaning and bandaging of open wounds to prevent infection.

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 clinical development of our product candidate and the timing and reporting of results from our clinical trial. 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 potential goals, progress, and timing of results of our clinical trial, 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 our clinical study indicates that the product candidate is unsafe or ineffective; 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 the clinical study could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete our study. 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, 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.

FOLD-G

CONTACTS:

Investors/Media:

Amicus Therapeutics

Sara Pellegrino

Senior Director, Investor Relations

spellegrino@amicusrx.com

(609) 662-5044

Media:

MWW PR

Sid Dinsay

sdinsay@mww.com

(646) 381-9017

Primary Logo

Source: Amicus Therapeutics, Inc

News Provided by Acquire Media