September 13, 2017

Amicus Therapeutics Announces Top-Line Phase 3 Results for SD-101 in Epidermolysis Bullosa

Study Did Not Meet Primary Endpoints

Conference Call and Webcast Today at 8:30am ET

CRANBURY, N.J., Sept. 13, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics, Inc. (Nasdaq:FOLD) reported that top-line data from the randomized, double-blind, placebo-controlled Phase 3 clinical study (ESSENCE, SD-005) to assess the efficacy and safety of the novel topical wound-healing agent SD-101 did not meet the primary endpoints or secondary endpoints in participants with epidermolysis bullosa (EB).

The Phase 3 ESSENCE study randomized 169 participants with a documented diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz epidermolysis bullosa to SD-101 (n=82) or placebo (n=87) during a three-month primary treatment period. SD-101 did not show a statistically significant difference from placebo in the intent to treat (ITT) population (n=169). The first primary endpoint, the time to target wound closure within 3 months, was not different between groups (Hazard ratio=1.004, p=0.985).[1] The second primary endpoint, the percentage of patients achieving target wound closure by month 3, was also not different between groups (49% SD-101; 54% placebo; p=0.390).[2] Similarly, the secondary endpoints did not reach statistical significance versus placebo. Encouraging trends in wound closure were observed in certain sub-populations. Treatment-emergent adverse events (TEAEs) were similar across both the SD-101 and placebo groups. The most common TEAEs were nasopharyngitis (common cold), pruritis (itchy skin), and pyrexia (fever).

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "We are disappointed that this Phase 3 study of SD-101 did not meet the primary endpoints in epidermolysis bullosa, an utterly devastating rare genetic disorder with no approved treatment options. In keeping with our Amicus mission, we have a strong commitment to the EB community and will work closely with investigators and other leading experts to understand and to share these data. We would like to sincerely thank the patients, families, clinical investigators, regulators and our Amicus team involved in this EB program. In seeking to develop novel, high quality therapies for those living with devastating rare diseases we may sometimes fail. But we would rather be the first to fail, than the last to try. Our vision at Amicus remains steadfast and is focused on building a leading global biotechnology company that delivers significant benefits for people living with rare diseases."

Amicus plans to further analyze and share the Phase 3 ESSENCE results with key stakeholders in the epidermolysis bullosa community including physicians, patient organizations and regulators. In the interim, in consultation with their physicians, participants in the ongoing extension studies (SD-004 and -006) will have the opportunity to continue being treated with SD-101. Based on these top-line data Amicus has no current plans to invest in any additional clinical studies or commercial preparation activities for SD-101.

Conference Call and Webcast
Amicus Therapeutics will host a conference call and audio webcast today, September 13, 2017 at 8:30 a.m. ET to discuss the top-line Phase 3 ESSENCE study results. Interested participants and investors may access the conference call by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international); participant code 85441850.

An audio webcast can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at http://ir.amicusrx.com/, and will be archived for 30 days. Web participants are encouraged to go to the web site 15 minutes prior to the start of the call to register, download and install any necessary software. A telephonic replay of the call will be available for seven days beginning at 11:30 a.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 85441850.

About the ESSENCE Study
ESSENCE (SD-005) is a randomized, multicenter, double-blind, placebo-controlled Phase 3 study in patients with documented diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB. For study entry, target wounds were required to be chronic (at least 21 days old) and between 10 and 50 cm^2 in size. A total of 169 patients were randomized

on a 1:1 basis to SD-101 treatment (n=82) or placebo (n=87) for a three-month primary treatment period followed by an ongoing open-label extension study (SD-006) in which all patients receive SD-101 treatment.

About Epidermolysis Bullosa (EB)
Epidermolysis bullosa is a rare, devastating genetic disorder that leads to severe skin blistering and open wounds often beginning at birth. There are no approved therapies to treat epidermolysis bullosa, which affects tens of thousands of children and adults throughout their lifetimes. EB is chronic, potentially disfiguring, and in some cases fatal. 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 bandaging and cleaning of open wounds to prevent infection.

About Amicus Therapeutics
Amicus Therapeutics (Nasdaq:FOLD) is a 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, 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 the clinical results from, and our future development plans with respect to, our product candidate, SD-101 for Epidermolysis Bullosa. The inclusion of forward-looking statements should not be regarded as a representation by us that any of our plans will be achieved or will not change. Any or all of the forward-looking statements in this press release can be affected by assumptions we might make based on information available at this time, including our evaluation of the Phase 3 clinical results, or by other known or unknown risks and uncertainties. In addition, all forward-looking statements are subject to other risks and factors detailed in our Annual Report on Form 10-K for the year ended December 31, 2016 as well as our Quarterly Report on Form 10-Q for the quarter ended June 30, 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.

[1]Analyzed in all patients randomized (intent to treat (ITT)) using cox-proportional statistical model with baseline target wound size, target wound age, and EB type as covariates.

[2]Analyzed in ITT population using a logistic regression statistical model with baseline target wound size, target wound age, and EB type as covariates.

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Source: Amicus Therapeutics, Inc