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Amicus Therapeutics Announces Planned Analysis of Primary Endpoints in Phase 3 Epidermolysis Bullosa Study

Top-Line Data on Track for 3Q17

CRANBURY, N.J., May 19, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics, Inc. (Nasdaq:FOLD), has completed the analysis plan for the primary endpoints in the blinded ongoing Phase 3 clinical study (ESSENCE) of the novel topical medicine SD-101 for epidermolysis bullosa (EB). SD-101 was one of the first treatments to receive the FDA's Breakthrough Therapy designation. ESSENCE is a double-blind, placebo-controlled registration study that completed enrollment of more than 160 patients who have a documented diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB.

Based on discussions and written communication from the Dermatology Division of the U.S. Food and Drug Administration, Amicus will analyze the primary endpoints for the Phase 3 ESSENCE study as follows:

- The endpoint of "time to wound closure within 3 months" will be analyzed first. If the difference between SD-101 6% and placebo is statistically significant ($p \leq 0.05$), then the study will be considered a success.
- If the first endpoint is statistically significant, then "proportion of patients with target wound closure at month 3" will be analyzed and considered statistically significant at $p \leq 0.05$.

Time to Wound Closure is a clinically relevant endpoint in EB, given the constant and often chronic nature of EB wounds. Time to Wound Closure is as an acceptable efficacy endpoint for wound-related diseases, per FDA guidance.[1]

Jay Barth, MD, Chief Medical Officer of Amicus Therapeutics, Inc. stated, "We believe that the planned analysis of the primary endpoints increases our overall likelihood for success in the Phase 3 ESSENCE study of SD-101 for epidermolysis bullosa. Time to Wound Closure accounts for wound healing across all study time points, offering greater precision for discriminating treatment effects and treatment differences between SD-101 and placebo. We have been very pleased with the collaborative interactions with the Dermatology Division of the FDA under our breakthrough therapy designation for SD-101. We look forward to announcing data from this study."

Amicus is on track to report top-line data from the ESSENCE study during the third quarter of 2017. 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. Patients 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 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, 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, the timing and reporting of results from a clinical trial, the prospects and timing of the potential regulatory approval of our product candidates. 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 goals, progress, timing, and outcomes of discussions with regulatory authorities, and in particular 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 our business, including, without limitation: the potential that results of clinical studies indicate that the product candidates are 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 we may not be successful in commercializing our product candidates if and when approved; the potential that clinical studies could be delayed because we identify serious side effects or other safety issues; and the potential that we will need additional funding to complete all of our 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, 2016 as well as our Quarterly Report on Form 10-Q for the quarter ended March 31 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] http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm071324.pdf

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