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Amicus Therapeutics Receives Rare Pediatric Disease Designation for SD-101 for Patients with Epidermolysis Bullosa

Designation Covers Broad Treatment of Epidermolysis Bullosa (EB)

Top-Line Phase 3 Data on Track for 3Q17

CRANBURY, N.J., May 31, 2017 (GLOBE NEWSWIRE) -- Amicus Therapeutics, Inc. (Nasdaq:FOLD), has received Rare Pediatric Disease designation[1] from the U.S. Food and Drug Administration (FDA) for the novel topical medicine SD-101 for the treatment of epidermolysis bullosa (EB). The Rare Pediatric Disease designation for SD-101 covers the broad treatment of EB. In addition to the Rare Pediatric Disease designation, SD-101 has Orphan Drug designation and was one of the first treatments to receive the FDA's Breakthrough Therapy designation.

The FDA grants Rare Pediatric Disease designation for diseases that primarily affect children ages 18 years or younger and fewer than 200,000 persons in the U.S. If a new drug application (NDA) for SD-101 is approved, Amicus is eligible to receive a priority review voucher that may be sold or transferred.

John F. Crowley, Chairman and Chief Executive Officer of Amicus Therapeutics, Inc. stated, "This Rare Pediatric Disease designation is significant in its broad coverage for the treatment of EB, and adds to our previous Orphan Drug and Breakthrough Therapy designations for SD-101 from the FDA. We believe that these important designations highlight the urgent need for a treatment for this devastating rare disease. SD-101, which we are developing for all three major types of EB, was the first drug to enter Phase 3 development for EB, and has the potential to be the first FDA-approved therapy. SD-101 has the potential to provide meaningful clinical benefit to patients and their caregivers and we eagerly await the results of our Phase 3 study."

Amicus is on track to report top-line data from the Phase 3 ESSENCE study of SD-101 during the third quarter of 2017. 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. 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 https://www.fda.gov/ForIndustry/DevelopingProductsforRareDiseasesConditions/RarePediatricDiseasePriorityVoucherProgram/default.htm

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