



August 31, 2015

Amicus Therapeutics to Acquire Rare Disease Company Scioderm, Inc.

Scioderm's Lead Drug Candidate Zorblisa™ in Phase 3 Study for Rare Disease Epidermolysis Bullosa (EB) Granted FDA Breakthrough Therapy Designation

FDA Has Agreed to Rolling NDA Submission for Zorblisa Beginning in 4Q15

Acquisition Advances Amicus Vision to Create One of the World's Leading Rare Disease Biotechnology Companies

Conference Call Today at 8:00 a.m. ET

CRANBURY, N.J., Aug. 31, 2015 (GLOBE NEWSWIRE) -- Amicus Therapeutics (Nasdaq:FOLD), a biotechnology company at the forefront of therapies for rare and orphan diseases, and Scioderm, Inc. a privately-held biopharmaceutical company focused on developing innovative therapies for treating diseases with high unmet need, have signed a definitive agreement under which Amicus will acquire 100% of the capital stock of Scioderm, Inc.

Transaction highlights

- Excellent strategic fit with Amicus' patient-centric vision to develop and commercialize advanced therapies for devastating rare and orphan diseases
- Leverages Scioderm development team's EB expertise with Amicus' global clinical infrastructure to advance Zorblisa toward regulatory approvals and Amicus' commercial, patient advocacy and medical affairs infrastructure to support a successful global launch
 - Potential first-to-market therapy to address estimated \$1 billion+ global commercial opportunity
 - FDA breakthrough therapy designation based on positive Phase 2 proof-of-concept data
 - Phase 3 pivotal study ([SD-005](#)) is currently enrolling pediatric and adult EB patients across all major subtypes to support global regulatory approvals - data anticipated in 1H16
 - Well-defined global regulatory pathway - agreement on rolling NDA in U.S. and pediatric investigation plan (PIP) in Europe
- Creates a leading rare disease portfolio that is well-positioned to bring substantial value to patients and shareholders - potential for Fabry commercial product launch, EB marketing submissions, and Pompe Phase 3 study in 2016

"This acquisition is a major step forward toward our strategic vision and is transformative for the Epidermolysis Bullosa, or EB, community as well as the shareholders of Amicus and Scioderm," said John F. Crowley, Chairman and Chief Executive Officer of Amicus and Board Member of Scioderm. "EB is a disorder that is utterly devastating and painful as it causes extremely fragile skin that blisters and tears from minor friction or trauma. In many children it leads to severe complications and a very early death. Amicus is committed now to advancing the tremendous mission of Scioderm's Co-Founder and CEO Dr. Robert Ryan, who we are proud to welcome to our senior leadership team at Amicus. We believe we are well-positioned to rapidly complete the clinical development of Zorblisa and to make Zorblisa commercially available for all EB patients as quickly as possible. When combined with migalastat for Fabry disease and ATB200 for Pompe disease, this acquisition solidly positions Amicus as a leading global rare disease company dedicated to bringing substantial value to patients and shareholders."

Scioderm's lead product candidate Zorblisa is a novel, late-stage, proprietary topical cream and potential first-to-market therapy for EB. Zorblisa has established positive proof-of-concept in Phase 2 studies for the treatment of lesions in patients suffering with EB, and is currently being investigated in a Phase 3 study to support global regulatory approvals. Zorblisa was one of the first products to receive FDA breakthrough therapy designation in 2013, and was the first-ever treatment in EB clinical studies to show significant benefit in wound closure across all major EB subtypes.

Amicus estimates that EB may represent a potential \$1 billion+ global market opportunity based on third party market research. The current standard of care is palliative treatments which cost \$10,000 to \$15,000 per month, and mainly consist of bandaging, treating the open wounds to prevent infection and trying to manage patients' pain. An estimated 30,000 to 40,000+ people are currently diagnosed with EB in major markets.

"Amicus is a champion of the rare disease community that, together with Scioderm, understands our sense of urgency to see a treatment approved for EB," said Brett Kopelan, Executive Director of the Dystrophic Epidermolysis Bullosa Research

Association of America (DebRA). "The EB community will be well-served by the experience and broad, global capabilities that Amicus adds to Scioderm."

"Both Amicus and Scioderm are wholly and passionately focused on patients with rare diseases, and share a common vision and similar values," said Robert Ryan, Ph.D., President and Chief Executive Officer of Scioderm. "John Crowley has been a dedicated board member providing valuable counsel to Scioderm over the past several years, during which time he has been deeply involved with the EB community. This combination of Amicus and Scioderm is a major win for EB patients. With the added resources and expertise that Amicus provides for the Zorblisa program, we are more confident than ever in our potential for success and our ability to deliver significant benefits to patients and families living with the devastating effects of EB."

The Transaction

Amicus will acquire Scioderm in a cash and stock transaction. At closing, Amicus will pay Scioderm shareholders \$229 million, of which \$125 million will be paid in cash and \$104 million will be paid through the issuance of 7 million newly issued Amicus shares. Amicus has agreed to pay up to an additional \$361 million to Scioderm shareholders in cash or stock upon achievement of certain clinical and regulatory milestones and \$257 million to Scioderm shareholders in cash or stock upon achievement of certain sales milestones. If Zorblisa is approved, EB qualifies as a rare pediatric disease and a Priority Review Voucher will be requested. If the Priority Review voucher is obtained and subsequently sold, Amicus will pay Scioderm shareholders the lesser of \$100 million or 50% of the proceeds of such sale. The transaction is subject to customary conditions, including the expiration or termination of the waiting period under the Hart-Scott-Rodino Antitrust Improvements Act. The Boards of both companies have approved the transaction and the companies currently anticipate that the transaction will be completed in the third quarter of 2015.

Amicus intends to finance the acquisition through cash on hand and has a \$50 million debt commitment from Redmile Group. Leerink Partners LLC is acting as financial advisor to Amicus. Skadden, Arps, Slate, Meagher & Flom LLP is acting as legal counsel to Amicus. J.P. Morgan is acting as financial advisor to Scioderm. Cooley LLP is acting as legal advisor to Scioderm.

Based on the closing of the Scioderm acquisition, the anticipated debt financing and the forecasted spending on Zorblisa development, Amicus expects to end 2015 with \$200 million to \$225 million of cash on hand. Pro-forma cash post-closing is expected to fund the current operating plan (including Zorblisa) into 2017.

Conference Call and Webcast

Amicus Therapeutics will host a conference call and audio webcast today, August 31, 2015 at 8:00 a.m. ET to discuss the proposed acquisition of Scioderm. Interested participants and investors may access the conference call at 8:00 a.m. ET by dialing 877-303-5859 (U.S./Canada) or 678-224-7784 (international).

An audio webcast and slide presentation can also be accessed via the Investors section of the Amicus Therapeutics corporate web site at <http://www.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 8:00 p.m. ET today. Access numbers for this replay are 855-859-2056 (U.S./Canada) and 404-537-3406 (international); participant code 28588321.

About Epidermolysis Bullosa (EB)

Epidermolysis Bullosa (EB) is a chronic, rare genetic connective tissue disorder with no approved treatment options. EB is debilitating, disfiguring, and potentially fatal. There are many genetic and symptomatic variations of EB that all share the prevalent manifestation of fragile skin that blisters and tears from minor friction or trauma. Patients with the more severe forms of EB have generalized blistering and lesions affecting a substantial percentage of their bodies that can lead to infection and scarring, and, in severe cases, death. Internal organs and bodily systems can also be severely affected by the secondary complications and illnesses. There is currently no FDA approved treatment for EB. Current standard of care consists of bandaging and bathing the open wounds to prevent infection and trying to manage patients' pain. EB affects all racial, ethnic and genders equally.

About Zorblisa Phase 3 Clinical Trial (SD-005)

A Phase 3 multi-center, randomized, double-blind, placebo-controlled study ([SD-005](#)) in the U.S. and Europe is currently underway and expected to support registration globally. The study is currently enrolling individuals who are 1 month and older with a diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB who have at least 1 target wound present for 21 days or more. Half the patients receive Zorblisa cream (also known as SD-101) and the other half receive placebo cream, applied topically once daily to the entire body for 90 days. The primary outcome measure is complete target wound closure within 2 months. Secondary outcome measures include 1) median time to complete target wound closure; 2) change in lesional skin at Month 2; 3) change in itching at Day 7; and 4) change in pain at Day 7. Patients who complete the 90-day primary

treatment period will be eligible to receive Zorblisa in an open-label extension study (SD-006). For more information please visit Scioderm's website at www.sderm.com.

About Scioderm

Scioderm is a privately held, clinical-stage biopharmaceutical company focused on developing innovative therapies to address diseases with high unmet need, including rare diseases. Scioderm was financed initially in 2013 by Morgenthaler Ventures and Technology Partners, followed by a subsequent financing that was led by Redmile Group and included the initial investors.

Ralph (Chris) Christoffersen, Ph.D., Chairman of the Board, noted that, "Robert Ryan and the Scioderm team have done an outstanding job in bringing Zorblisa through both preclinical and clinical studies. It is a real pleasure to join with the excellent team at Amicus to continue the development and commercialization of this product which we believe will have a significant positive impact on the lives of EB patients and their families."

The company's lead therapy, Zorblisa (SD-101), is in Phase 3 development for treatment of the skin effects associated with Epidermolysis Bullosa (EB), a rare genetic connective tissue disorder. Scioderm was selected as a 2013 "Fierce Top 15" company by FierceBiotech, and considered one of the top 15 emerging companies in the biotech industry. The company is headquartered in Durham, North Carolina. Additional information about Scioderm can be found at www.sderm.com.

About Amicus Therapeutics

[Amicus Therapeutics](http://www.amicus-therapeutics.com) (Nasdaq:FOLD) is a biotechnology company at the forefront of therapies for rare and orphan diseases. The Company is developing novel, first-in-class treatments for a broad range of human genetic diseases, with a focus on delivering new benefits to individuals with lysosomal storage disorders. Amicus' lead programs in development include the small molecule pharmacological chaperone migalastat as a monotherapy for Fabry disease, as well as next-generation enzyme replacement therapy (ERT) products for Fabry disease, Pompe disease, and MPS I.

Forward-Looking Statements

This press release contains, and the accompanying conference call and slide presentation will contain, "forward-looking statements" within the meaning of the Private Securities Litigation Reform Act of 1995 relating to the planned acquisition of Scioderm, the expected financial impact and benefits to Amicus of such acquisition, and anticipated milestones and other expectations regarding Scioderm's product development activities, clinical trials and commercialization, preclinical and clinical development of Amicus' candidate drug products, the timing and reporting of results from preclinical studies and clinical trials evaluating Amicus' candidate drug products, financing plans, and the projected cash position for the Company. Words such as, but not limited to, "look forward to," "believe," "expect," "anticipate," "estimate," "intend," "potential," "plan," "targets," "likely," "may," "will," "would," "should" and "could," and similar expressions or words identify forward-looking statements. Such forward-looking statements are based upon current expectations that involve risks, changes in circumstances, assumptions and uncertainties. The inclusion of forward-looking statements should not be regarded as a representation by Amicus that any of its plans will be achieved. Any or all of the forward-looking statements in this press release may turn out to be wrong. They can be affected by inaccurate assumptions Amicus might make or by known or unknown risks and uncertainties. Important factors that could cause or contribute to such differences include risks relating to: the possibility that the transaction with Scioderm will not be completed; uncertainties as to the timing of the transaction; the possibility that the expected benefits of the transaction will not be fully realized by us or may take longer to realize than expected; future results of on-going or later clinical trials for Zorblisa; our ability to obtain regulatory approvals and commercialize Zorblisa following the closing; and market acceptance of Zorblisa. Also, with respect to statements regarding the goals, progress, timing and outcomes of discussions with regulatory authorities and the potential goals, progress, timing and results of preclinical studies and clinical trials, actual results may differ materially from those set forth in this release due to the risks and uncertainties inherent in the business of Amicus, including, without limitation: the potential that results of clinical or pre-clinical studies indicate that the product candidates are unsafe or ineffective; the potential that it may be difficult to enroll patients in our clinical trials; the potential that regulatory authorities may not grant or may delay approval for our product candidates; the potential that preclinical and clinical studies could be delayed because we identify serious side effects or other safety issues; the potential that we will need additional funding to complete all of our studies and, our dependence on third parties in the conduct of our clinical studies. Further, the results of earlier preclinical studies and/or clinical trials may not be predictive of future results. With respect to statements regarding projections of the Company's cash position, actual results may differ based on market factors and the Company's ability to execute its operational and budget plans. 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, 2014 and our Form 10-Q for the quarter ended June 30, 2015. 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 Amicus undertakes no obligation to revise or update this news release to reflect events or circumstances after the date hereof. This caution is made under the safe harbor provisions of Section 21E of the Private Securities Litigation Reform Act of 1995.

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