Scioderm Initiates Phase 2B Study of SD-101, A Novel Topical Therapy, for Patients with Epidermolysis Bullosa

Durham, N.C. (January 6, 2014) --- Scioderm, Inc. today announced the initiation of a Phase 2B study designed to evaluate the efficacy and safety of SD-101, a novel topical therapy, for the treatment of non-healing wounds in patients with Epidermolysis Bullosa (EB), a rare, genetic connective tissue disorder that typically manifests at birth or early childhood.

“People affected by EB suffer skin blisters, chronic wounds, almost constant pain and itching, and scarring,” said Robert Ryan., Ph.D., President and Chief Executive Officer of Scioderm. “The current standard of care is palliative only and focuses primarily on daily wound care, bandaging and pain management. We believe SD-101 has the potential to initiate and continue healing of lesions in this patient population, and Scioderm is committed to develop SD-101 as expeditiously as possible as a treatment option for patients suffering from this devastating condition.”

SD-101 for the treatment of EB has been granted an orphan drug designation in the US and in December 2013 received a positive opinion by the Committee for Orphan Medicinal Products (COMP) in the EU. In addition, Scioderm was the first biotech to receive “Breakthrough Therapy” designation for SD-101 from the Food and Drug Administration (FDA) for the treatment of skin effects in patients with EB. A breakthrough therapy designation conveys all of the fast track program features, as well as more intensive FDA guidance on an efficient drug development program. In addition, Scioderm was recently selected as a 2013 “Fierce Top 15” company by FierceBiotech, considered as one of the top 15 emerging companies in the biotech industry.

The Phase 2B study (SD-003) is a multi-site, prospective, randomized, placebo-controlled trial evaluating the efficacy and safety of SD-101 to close selected chronic cutaneous wounds and reduction in body surface area (BSA) coverage of lesional areas on the skin in patients with EB (Simplex, Recessive Dystrophic, or Junctional (non-Herlitz)). In addition, improvement on pain and itching will also be assessed. Approximately 48 subjects aged six months and older are planned to enroll in the trial, which is being conducted in 7 sites across the US. The study will comprise application of SD-101 cream over the entire body daily for a period of three months. Patients completing the study will be eligible to continue receiving SD-101 once the study is completed. Additional information about Study SD-003 can be found at www.clinicaltrials.gov.

“Our patients with EB have few therapeutic options available and none have demonstrated evidence of accelerated closure of chronic wounds, said the study’s Principal Investigator, Amy Paller, MD, Walter J. Hamlin Professor and Chair of the Department of Dermatology, Northwestern University Feinberg School of Medicine. “We look forward to testing this new potential intervention in the double-blind, randomized trial. If the lives of the EB patients are improved with topical use of SD-101 through faster wound healing, as well as decreased pain and itchiness, this cream would be welcomed by our affected families.”
“We are very excited that Scioderm has developed this potential therapy to treat some of the more profound symptoms of EB,” said Brett Kopelan, MA, Executive Director of the Dystrophic Epidermolysis Bullosa Research Association of America (debra of America). “The impact on the quality of life of those with EB would be immeasurable if SD-101 was to be proven safe and effective.”

About Epidermolysis Bullosa (EB)

Epidermolysis Bullosa (EB) is a rare genetic connective tissue disorder, with many genetic and symptomatic variations. All forms of EB share the common symptom of fragile skin that blisters and tears from the slightest friction or trauma. This particular manifestation has led to EB patients being known as “butterfly children” due to the analogous nature of the fragility of the skin to the wings of a butterfly. As of today there is no cure or effective treatment. The more severe forms of the disease lead to scarring, disfigurement, disability and early death, usually before the age of 30.

About Scioderm, Inc.

Scioderm is a privately held, clinical-stage pharmaceutical company focused on developing innovative therapies to address diseases with critical unmet medical needs, including orphan products. The company is headquartered in Durham, North Carolina. Additional information about Scioderm can be found at www.sderm.com.

About SD-101

SD-101 is a topical cream that has previously demonstrated potential to provide improvement in treating the severe skin effects seen in patients across all EB subtypes. An open-label Phase II study was conducted previously in children with either Simplex, Recessive Dystrophic (RDEB), or Junctional EB. SD-101 was applied topically over the entire body daily for a period of three months. The primary outcome measurements were assessment of target wound reduction and closure, and reduction in body surface area (BSA) coverage of blisters and lesions. In the clinical trial, SD-101 application resulted in complete closure of 88% of target chronic lesions within one month, in addition to a 57% reduction in BSA coverage of blisters and lesions after 3 months of daily treatment. SD-101 was well tolerated by the children.

Forward Looking Statement

Except for the historical information contained herein, the matters discussed in this press release are forward-looking statements that involve risks and uncertainties, including: our dependence on third parties for the development, regulatory approval and successful commercialization of our products, the inherent risk of failure in developing product candidates based on new technologies, risks associated with the costs of clinical development efforts, as well as other risks. Actual results may differ materially from those projected. These forward-looking statements represent our judgment as of the date of the release. Scioderm disclaims any intent or obligation to update these forward-looking statements.
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