DIGITAL JOURNAL

Scioderm, Inc. receives Positive Opinion from European Orphan Medicinal Products Committee for Novel Topical Therapy to Treat Epidermolysis Bullosa

PR Newswire

DURHAM, N.C., Dec. 17, 2013

Scioderm, Inc., today announced that the Committee for Orphan Medicinal Products (COMP) issued a positive opinion on the application for orphan designation for SD-101 for the treatment of patients with Epidermolysis Bullosa, (EB), a rare orphan pediatric disease. The "Orphan Medicinal Product Designation" is designed to encourage the development of drugs which may provide significant benefit to patients suffering from rare diseases identified as "life-threatening or chronically debilitating" conditions. Scioderm has previously received Orphan Drug Designation for SD-101 in the United States for the treatment of skin blistering and erosions associated with inherited EB.

Orphan drugs benefit from 10 years market exclusivity in the European Union (EU) after marketing approval. Additional benefits for sponsor companies include reduced fees for various centralized activities including applications for marketing authorization, inspections and protocol assistance, as well as possible eligibility for EU grants and other R&D-supporting initiatives. An additional two years of exclusivity could be obtained if the drug development has complied with an agreed Pediatric Investigation Plan (PIP), for a total of twelve years of market exclusivity in the EU.

"We are very pleased to receive a positive opinion from the COMP for orphan designation of SD-101," said Robert Ryan., Ph.D., President and Chief Executive Officer of Scioderm. This designation recognizes the potential of SD-101 by the COMP to address a great unmet medical need."

Scioderm will be initiating in January 2014 a Phase 2B Randomized, Double-Blind, Vehicle Controlled Study with SD-101 in patients with either Simplex, Recessive Dystrophic, or Junctional Non-Herlitz EB. A

total of 48 patients will be enrolled in the study across seven sites in the US. Further information on the study design and site locations can be found at www.clinicaltrials.gov.

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, or Junctional EB. 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, with daily administration up to 3 months.

About Epidermolysis Bullosa (EB)

Epidermolysis Bullosa (EB) is a rare genetic connective tissue condition that, in all of its forms, share the prominent manifestation of extremely fragile skin that blisters or tears with 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. Daily wound care, pain management and preventative bandaging are the only options available for caregivers, who are usually the parents or other family members. The more severe forms of the disease lead to scarring, disfigurement, disability and early death, usually before the age of 30.

About Scioderm

Scioderm is a privately held, late 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. In 2013, Scioderm, Inc. 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 Epidermolysis Bullosa. 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. Additional information about Scioderm can be found at www.sderm.com.

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.

Investor and Media Contact
Robert Coull
Chief Operating Officer
robert.coull@sderm.com
(919)-824-2594
SOURCE Scioderm, Inc.

Read more: http://www.digitaljournal.com/pr/1644229#ixzz2t8GBpUWR