A Phase 3, Multi-center, Randomized, Double-Blind, Placebo Controlled Study of the Efficacy and Safety of SD-101 Cream in Patients with Epidermolysis Bullosa

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The primary objective is to compare the efficacy and safety of SD-101-6.0vs. SD- 101-0.0 (placebo) in patients with Simplex, RecessiveDystrophic, or Junctional non Herlitz Epidermolysis Bullosa.The primary endpoint is the complete closure of the...

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Epidermal and dermal conditions
Study type	Interventional

Summary

ID

NL-OMON44714

Source
ToetsingOnline

Brief title
SD-005 protocol

Condition

- Epidermal and dermal conditions

Synonym
inherited connective tissue disease; Genetic skin disorder

Research involving
Human

Sponsors and support

Primary sponsor: Scioderm Inc.
Source(s) of monetary or material Support: Scioderm Inc.

Intervention

Keyword: Junctional non Herlitz EB, SD-101, Simplex Epidermolysis Bullosa (EB) Recessive Dystrophic EB

Outcome measures

Primary outcome

Primary Efficacy Endpoints

The primary efficacy endpoints for this study are

* Time to complete target wound closure within 3 months

* The proportion of patients experiencing complete closure of their

target wound within 3 months

Secondary outcome

Key Secondary Efficacy Endpoints

The secondary measures of efficacy include:

* Proportion of patients experiencing complete closure of their target

wound within 2 months

* Proportion of patients experiencing complete closure of their target

wound within 1 month

* Change in lesional skin based on BSAI estimates at Month 3,

compared to Baseline

* Estimation of Total Body Wound Burden based on BSAI at Month 3, compared to

Baseline

* Change in itching assessed at Day 7, compared to Baseline

* Change in pain assessed at Day 7, compared to Baseline

Study description

Background summary

Epidermolysis Bullosa (EB) is a rare group of inherited disorders that typically manifest at birth as blistering and lesion formation on the skin and, in some cases, the epithelial lining of other organs, in response to little or no apparant trauma. In consequence, the skin is extremely fragile which can result in shearing of the skin, causing a high risk of infection. All forms of EB are both debilitating and life threatening. There are no standard of care products available to treat the dermal manifestations of EB, and there is no approved drug for EB in either Europe or United States. There have been numerous studies published on potential treatments for skin manifestations associated with EB. No controlled studies showed clinical benefit of any therapy. Newer exploratory treatments including skin grafts, bioengineered skin products, and gene therapy have been unsuccessful to date. In an open label study patients treated with SD-101 cream showed significant improvements in the complete healing of lesions, clinically meaningful reductions in the extent of total skin surface involvement with active disease, and reduced pain and itching. The aim of the study is to evaluate the efficacy and safety of SD-101 cream vs Placebo in patients with EB.

Study objective

The primary objective is to compare the efficacy and safety of SD-101-6.0vs. SD- 101-0.0 (placebo) in patients with Simplex, Recessive Dystrophic, or Junctional non Herlitz Epidermolysis Bullosa.The primary endpoint is the complete closure of the patient*s target wound within 2 months.

Study design

Phase 3, multi-center, randomized, double-blind, placebo controlled, study to assess the efficacy and safety of SD-101 vs placebo on lesions in patients with Simplex, Recessive Dystrophic, or Junctional non-Hertlitz Epidermolysis Bullosa.

Topical application of study cream (i.e. SD-101 or placebo) during 90 days, once daily.

Intervention

Topical application of SD-101 skin cream for 90 days (once daily)

Study burden and risks

In a previous study with SD-101, some cases of mild redness of the skin have been reported after application of SD-101 cream. Furthermore, patients will be asked to complete pain and itching scales, and diaries. All patients will have to use study cream (SD-101 or placebo) once daily for 90 days and visit the hospital/clinic more frequently.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)
Elderly (65 years and older)

Inclusion criteria

1. Informed Consent form signed by the patient or patient's legal representative; also, if the patient is under the age of majority but capable of providing assent, signed assent from the patient.
2. Patient (or caretaker) must be willing to comply with all protocol requirements.
3. Diagnosis of Simplex, Recessive Dystrophic, or Junctional non-Herlitz EB.
4. Patient must have 1 target wound (size 10 to 50 cm2).
5. Patients 1 month and older.
6. Target wound must be present for 21 days or more

Exclusion criteria

1. Patients who do not meet the entry criteria outlined above.
2. Selected target wound cannot have clinical evidence of local infection.
3. Use of any investigational drug within the 30 days before enrollment.
4. Use of immunotherapy or cytotoxic chemotherapy within the 60 days before enrollment.
5. Use of systemic or topical steroidal therapy within the 30 days before enrollment. (Inhaled steroids and ophthalmic drops containing steroids are allowed)
6. Use of systemic antibiotics within the 7 days before enrollment.
7. Current or former malignancy.
8. Arterial or venous disorder resulting in ulcerated lesions.
9.Pregnancy or breastfeeding during the study. (A urine pregnancy test will be performed at screening and every 30 days until the final visit for female patients of childbearing potential)
10. Females of childbearing potential who are not abstinent and not practicing a medically acceptable method of contraception.

Study design

Design

Study phase:	3
Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	15-03-2015
Enrollment:	14
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Zorblisa
Generic name:	ALLANTOIN

Ethics review

Approved WMO Date:	03-07-2014
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	23-12-2014
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	06-02-2015
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	21-04-2015
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	22-05-2015
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO	

Date:	24-06-2015
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	19-11-2015
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	10-12-2015
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	06-12-2016
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	03-02-2017
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	26-04-2017
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	21-06-2017
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2014-002288-14-NL
CCMO	NL49780.042.14