An International, Multicenter, Open-label, Long Term Extension Study Evaluating the Safety of Diacerein 1% Ointment Topical Formulation in Subjects with Epidermolysis Bullosa Simplex (EBS)

Published: 06-12-2017
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The primary objective of this study is to evaluate the long-term safety and tolerability of Diacerein 1% Ointment for 2 treatment cycles in subjects with EBS that were previously enrolled in studies CCP-020-301 or CCP-020-101.

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

Summary

ID

NL-OMON48978

Source
ToetsingOnline

Brief title
CCP-020-302

Condition

- Epidermal and dermal conditions

Synonym
Epidermolysis Bullosa Simplex

Research involving
Human

Sponsors and support

Primary sponsor: Medpace
Source(s) of monetary or material Support: Castle Creek Pharmaceuticals LLC

Intervention

Keyword: Diacerein, Epidermolysis Bullosa Simplex

Outcome measures

Primary outcome

Safety will be evaluated in terms of the occurrence of AEs and changes in

clinical laboratory parameters, clinical examination findings, vital signs,

weight, and urine measurements.

Secondary outcome

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Study description

Background summary

Epidermolysis bullosa simplex (EBS) is a rare, genetic skin disease characterized by fragility of the skin and mucous membranes resulting in painful blisters and erosions after minor trauma, and is associated with significant morbidity and mortality. EBS is both a pediatric and an adult disease that tends to affect younger patients most severely. Diacerein 1% Ointment is a topical ointment containing diacerein (4,5-bis[acetyloxy]-9,10-dihydro-9,10dioxo-2-anthracene carboxylic acid, also known as diacetyl rhein), a highlypurified anthraquinone derivative, and is being developed for the treatment of EBS. Diacerein in the topical formulation is hydrolyzed to rhein in the epidermis and dermis following administration. Diacerein and rhein have been shown to inhibit the in vitro and in vivo production and activity of interleukin-1* (IL-1*) and other proinflammatory cytokines. IL-1* is a pro inflammatory cytokine that has been linked to a number of inflammatory and autoimmune diseases, including rheumatoid arthrtis (RA), OA, hemophilic arthropathy, gouty arthritis, type 2 diabetes mellitus (T2DM), diabetic nephropathy (DN), and EBS. In vitro and in vivo animal studie have shown that both diacerein and its active metabolite rhein inhibit the produiction and

activity of pro-inflammatory and procatabolic cytokines such as IL-1 and IL-6, and the expression of inducible nitric oxide synthase (iNOS) and tumor necrosis factor-* (TNF-*).

Study objective

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Study design

The study enrolls subjects from two feeder studies, CCP-020-301 and CCP-020-101. Subjects that enroll will, after a screening, undergo a 16 week cycle if they have developed blisters, in which they will be on the study drug for the first 8 weeks, and off the study drug for the next 8 weeks. This cycle can be done a maximum of two times, and subjects cannot start a new cycle after week 36.

Intervention

Subjects who are enrolled will apply diacerein 1% ointment daily for 8 weeks in a 16 week cycle.

Study burden and risks

Risks: Possible side effects of the study medication
Burden: Blood draws, instructions on study drug, filling in questionnaires

Contacts

Public
Medpace

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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. In the opinion of the Investigator, the subject is capable of understanding and complying with protocol requirements., 2. The subject or, when applicable, the subject's legally acceptable representative signs and dates a written, informed consent/assent form and any required privacy authorization prior to the initiation of any study procedures., 3. Subject has a documented genetic mutation consistent with EBS. , 4. Subjects who participated in the CCP-020-301 or the CCP-020-101 study are eligible to be rolled into the CCP-020-302 open label extension study, regardless of their completion status on the feeder study. , 5. Subject/caregiver agrees to report use of any topical therapies applied to EBS lesions (e.g. medicated cleansers, bleach cleansers, bleach baths, topical antiseptics, topical disinfectants, etc.)., 6. If the subject is a woman of childbearing potential, she has a negative urine pregnancy test and agrees to use an approved effective method of birth control, as defined by this protocol, for the duration of the study., 7. Subject is non-lactating and is not planning for pregnancy during the study period., 8. Subject is willing and able to follow all study instructions and to attend all study visits.

Exclusion criteria

1. Subject has EBS lesions to be treated that are infected (i.e., EBS lesions that require topical antibiotic therapy to treat an infection)., 2. Subject has evidence of a systemic infection or has used systemic antibiotics within 7 days prior to Baseline., 3. The subject was discontinued from the feeder study due

to an adverse event judged to be related or possibly related to the study medication., 4. Subject has experienced a change in clinical status from the feeder study that, in the investigator's opinion, puts the subject at undue risk to participate.

Study design

Design

Study phase:	2
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Completed
Start date (anticipated):	16-05-2018
Enrollment:	6
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Diacerein 1% Ointment
Generic name:	diacerein

Ethics review

Approved WMO Date:	06-12-2017
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	22-02-2018

Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	11-04-2018
Application type:	First submission
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	31-05-2018
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	31-08-2018
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	17-10-2018
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	06-11-2018
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	12-06-2019
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Groningen (Groningen)
Approved WMO Date:	11-11-2019
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	EUCTR2017-003757-41-NL
CCMO	NL64012.042.17

Study results

Date completed:	02-07-2019
Results posted:	11-08-2020
Actual enrolment:	3

First publication
01-01-1900