Observational follow-up study to evaluate the long term efficacy of (HOLO-UP)

First published: 13/06/2017

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Study Ongoing

Administrative details

EU PAS number

EUPAS19486

Study ID

25844

DARWIN EU® study

No

Study countries

☐ Italy

Study description

This is an observational, non-interventional, long-term follow-up study, where the long-term efficacy and safety of autologous cultivated limbal stem cells transplantation (ACLSCT) will be assessed in a prospective (efficacy and safety)

and retrospective fashion (safety), respectively. The study will be conducted in a single site in Italy (Hospital San Raffaele, Milan) which participated as the main site (i.e. most patients treated here) in the previous retrospective HLSTM01 study.All patients who meet all the Inclusion Criteria will be contacted and invited for a follow-up visit.Each patient reached and consenting to participate will undergo to one ophtalmologic visit including pictures collection from the treated eye. The pictures will be then evaluated centrally (Independent Assessors) to provide a final and independent long-term treatment outcome.

Study status

Ongoing

Research institutions and networks

Institutions

Chiesi Farmaceutici

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Paolo Rama

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/02/2017

Actual: 09/06/2017

Study start date

Planned: 03/07/2017

Actual: 24/08/2017

Data analysis start date

Planned: 24/10/2018

Date of final study report

Planned: 07/02/2019

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Chiesi Farmaceutici SpA

Study protocol

CCD-GPLSCD01-07_study protocol_v 1.0_08Mar2017_Signed CHIESI.pdf(756.14 KB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Other study registration identification numbers and links

Sponsor code: CCD-GPLSCD01-07

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Main study objective:

To collect long term follow-up clinical data on patients who received transplantation of autologous cultured limbal stem cells (ACLSCT) to confirm efficacy and safety of the treatment in restoring a normal corneal epithelium in patients suffering from limbal stem cell deficiency (LSCD) secondary to ocular burns.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Long-term Follow-up study

Study drug and medical condition

Name of medicine

HOLOCLAR

Medical condition to be studied

Limbal stem cell deficiency

Population studied

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Estimated number of subjects

70

Study design details

Outcomes

% of patients with successful ACLSCT outcome, defined as CNV assessed by Independent Assessors in less than 2 quadrants and totally absent in central cornea as well as absence of mild to severe epithelial defects, % of patients with successful ACLSCT outcome defined as CNV assessed by investigator in less than 2 quadrants and totally absent in central cornea as well as absence of mild to severe epithelial defects, % of patients with ACLSCT outcome considered a success by investigator based on overall clinical status, % of patients by superficial corneal neo-vascularization score, Nr of related AE

Data analysis plan

General descriptive statistics for numeric variables will include the n (number of observed values), the mean, the standard deviation, the median, the minimum, and the maximum values. For categorical variables, the number and percent of subjects with a specific level of the variable will be presented.

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Electronic healthcare records (EHR)

Other

Data sources (types), other

Prospective patient-based data collection

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No