Open-label, longitudinal, post-authorisation safety study to assess the safety of Cystadrops in pediatric and adult cystinosis patients in long term use (CYT-DS-001)

First published: 14/12/2018

Last updated: 08/04/2019

Study Planned

Administrative details

EU PAS number

EUPAS25688

Study ID

29320

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany
- ☐ Netherlands

Study description

This is an European prospective, observational, non-interventional, longitudinal, multi-center, single-arm study designed mainly to collect safety data in long term use on patients treated with Cystadrops

Study status

Planned

Research institutions and networks

Institutions

Recordati

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Institution

Rare Diseases

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Céline Plisson

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/10/2018

Study start date

Planned: 20/05/2019

Date of final study report

Planned: 15/07/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Recordati Rare Diseases

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Main study objective:

To assess whether they are any potential risks to develop high severity degrees of local intolerance with CYstadrops or other serious AEs that could lead to potential consequences for compliance to treatment in the long term

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Descriptive and longitudinal single-arm study

Study drug and medical condition

Name of medicine

CYSTADROPS

Medical condition to be studied

Cystinosis

Population studied

Age groups

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

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

To assess the safety profile of Cystadrops in long term use by measuring the incidence of the safety events collected in the study for all patients, Benefit of Cystadrops by measuring photophobia, description of ophthalmological assessments (CCCS, Crystal thickness and BCVA)

Data analysis plan

Due to the limited number of patients in this rare disease, no formal statistical test will be done. Descriptive analysis will be performed for the total study

population (overall analysis) defined as all patients enrolled in the study and that received at least one dose of Cystadrops.

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)

Other

Data sources (types), other

Spontaneous reporting system, 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