

# 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

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### Study ID

29320

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### DARWIN EU® study


No

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### Study countries

 France

 Germany

 Netherlands

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## 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

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## Study status

Planned

## Research institutions and networks

### Institutions

#### Recordati

**First published:** 01/02/2024

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Institution

#### Rare Diseases

## Contact details

### Study institution contact

Céline Plisson [plisson.c@recordati.com](mailto:plisson.c@recordati.com)

Study contact

[plisson.c@recordati.com](mailto:plisson.c@recordati.com)

## Primary lead investigator

Céline Plisson

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 31/10/2018

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### Study start date

Planned: 20/05/2019

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### 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

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### 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

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**Main study objective:**

To assess whether there 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

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**Non-interventional study design, other**

Descriptive and longitudinal single-arm study

### Study drug and medical condition

**Medicinal product name**

### **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)

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### **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)

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### **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](#)

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### 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

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No