A MULTICENTRE, NON-INTERVENTIONAL, RETROSPECTIVE STUDY TO EXPLORE THE EFFECTS OF TRANSITIONING FROM IMMEDIATE RELEASE TO EXTENDED RELEASE ORAL CYSTEAMINE THERAPY IN NORWEGIAN PATIENTS WITH NEPHROPATHIC CYSTINOSIS (CYSTRANSFER)

First published: 08/06/2020

Last updated: 01/02/2022

Study Ongoing

Administrative details

EU PAS number

EUPAS35491

Study ID

40977

DARWIN EU® study

No

Study countries

☐ Norway

Study description

The aim of this study is to evaluate the implementation of the Extended-Release(ER)-cysteamine therapy in patients already treated with Immidiate-Release(IR)-cysteamine in Norway and to assess the outcomes of this option in routine care for patients with nephropathic cystinosis. This retrospective non-interventional multi-centre study will assess the efficacy and safety of oral IR- and ER-cysteamine treatment in the Norwegian patient population. The study will be based on retrospective patient journal data.

Study status

Ongoing

Research institutions and networks

Institutions

Oslo University Hospital

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Institution | Educational Institution | Hospital/Clinic/Other health care facility

Rikshospitalet

Contact details

Study institution contact

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Primary lead investigator

Anna Bjerre

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 10/02/2020

Actual: 02/04/2020

Study start date

Planned: 01/06/2020

Actual: 09/06/2020

Data analysis start date

Planned: 01/07/2021

Date of final study report

Planned: 31/12/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Chiesi Pharma AB

Regulatory

Was the study required by a regulatory body?

No

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

Not applicable

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Main study objective:

To evaluate the long-term efficacy and safety of IR-cysteamine (Cystagon®) and ER-cysteamine (Procysbi®) therapy under routine clinical practice.

Study drug and medical condition

Name of medicine

PROCYSBI

CYSTAGON

Anatomical Therapeutic Chemical (ATC) code

(A16AA04) mercaptamine

mercaptamine

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

10

Study design details

Outcomes

Primary efficacy endpoints• WBCs cystine levels• eGFR:, Secondary efficacy endpoints• Growth• Total prescribed daily cysteamine dose

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Data analysis plan

Data analysis includes four periods: Screening, IR-treatment period and ER-treatment periods 1 and 2. Efficacy, safety, prescribed dose and other patient characteristics are collected.

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)

Disease registry

Drug dispensing/prescription data

Electronic healthcare records (EHR)

Other

Data sources (types), other

Prescription event monitoring

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