Cerliponase alfa Observational Study

First published: 26/03/2019

Last updated: 10/07/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS29031

Study ID

46783

DARWIN EU® study

No

Study countries

- [ ] Denmark
- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] Netherlands
- [ ] Romania
- [ ] Sweden

☐ United Kingdom

Study description

This is a voluntary, multicenter, multinational, cerliponase alfa observational study for patients with a confirmed diagnosis of neuronal ceriod lipofuscinosis type 2 (CLN2 disease), also known as TPP1 deficiency, who intend to be or are currently being treated with cerliponase alfa.

Study status

Ongoing

Research institutions and networks

Institutions

ICON Commercialisation & Outcomes

☐ Germany

☐ Ireland

First published: 19/03/2010

Last updated: 05/07/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Contact details

Study institution contact

Program 190-504 Director medinfo@bmrn.com

Study contact

medinfo@bmrn.com

Primary lead investigator

Program 190-504 Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 14/03/2019

Actual: 19/03/2019

Study start date

Planned: 28/10/2019

Actual: 10/10/2019

Date of final study report

Planned: 27/04/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin Pharmaceutical

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Main study objective:

To evaluate long-term safety of cerliponase alfa in patients with neuronal ceroid lipofuscinosis Type 2 (CLN2 disease).

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational

Study drug and medical condition

Medicinal product name

BRINEURA

Study drug International non-proprietary name (INN) or common name

CERLIPONASE ALFA

Anatomical Therapeutic Chemical (ATC) code

(A16AB17) cerliponase alfa

cerliponase alfa

Medical condition to be studied

Neuronal ceroid lipofuscinosis

Additional medical condition(s)

Neuronal ceroid lipofuscinosis type 2 (CLN2)

Population studied

Age groups

- Term newborn infants (0 – 27 days)
- Infants and toddlers (28 days – 23 months)
- 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)

Special population of interest

Hepatic impaired

Immunocompromised

Renal impaired

Estimated number of subjects

45

Study design details

Data analysis plan

All analysis will be descriptive. Assessments are collected based on the country, institution, and individual patient standard of care.

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

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