MPS VI Clinical Surveillance Program (CSP)

First published: 25/05/2017

Last updated: 23/05/2022

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/38956

EU PAS number

EUPAS19286

Study ID

38956

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Belgium
- ☐ France
- ☐ Germany

- [ ] Ireland
- [ ] Lithuania
- [ ] Netherlands
- [ ] Norway
- [ ] Portugal
- [ ] Sweden
- [ ] United Kingdom
- [ ] United States

Study description

The Mucopolysaccharidosis VI (MPS VI) Clinical Surveillance Program (CSP) is being conducted in accordance with post-marketing commitments to the United States (US) Food and Drug Administration (FDA) and European Union (EU) European Medicines Agency (EMA) for Naglazyme. The data collected by this program will provide information to better characterize the natural history and progression of MPS VI in both treated and untreated patients. Data from periodic patient assessments, which are part of a patient's normal care, may be collected to provide long-term efficacy and safety data.

Study status

Ongoing

Research institutions and networks

Institutions

BioMarin Pharmaceuticals

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Institution

Contact details

Study institution contact

Program Director

Study contact

medinfoeu@bmrn.com

Primary lead investigator

Program Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 01/07/2005

Actual: 01/07/2005

Study start date

Planned: 12/09/2005

Actual: 12/09/2005

Date of final study report

Planned: 01/07/2021

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin International Limited

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

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Disease epidemiology

Main study objective:

Characterize & describe the MPS VI population as a whole, Help the MPS VI medical community with development of recommendations for monitoring patients reports and optimize patient care, Evaluate long-term effectiveness and safety of Naglazyme, Determine presence of Naglazyme in the infants of treated mothers, characterize effects of Naglazyme in pts <5 yrs enrolled in CSP (1mg/kg at least 1 year)

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Observational disease registry

Study drug and medical condition

Name of medicine

NAGLAZYME

Medical condition to be studied

Mucopolysaccharidosis VI

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)

Estimated number of subjects

176

Study design details

Data analysis plan

CSP data will be analyzed as per the program’s statistical analysis plan (SAP) and reported periodically. Physicians may obtain data on their individual patients and aggregate data on patients at their clinic. Longitudinal prospective and retrospective data may be collected. Demographic and baseline characteristics will be summarized. Frequencies will be presented for the categorical variables (eg, sex and race), and descriptive statistics will be presented for continuous variables (eg, height, weight, and age).

Data management

Data sources

Data sources (types)

Disease registry

Electronic healthcare records (EHR)

Other

Data sources (types), other

Prospective patient-based data collection, 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