Mucopolysaccharidosis VII Disease Monitoring Program (MPS VII DMP)

First published: 25/10/2018

Last updated: 14/03/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS25082

Study ID

49288

DARWIN EU® study

No

Study countries

- Argentina
- Brazil
- France
- Germany
- Netherlands
- Portugal

Spain

United States

Study description

The objectives of this study are to characterize MPS VII disease presentation and progression and assess long-term effectiveness and safety, including hypersensitivity reactions and immunogenicity of vestronidase alfa.

Study status

Ongoing

Research institutions and networks

Institutions

Ultragenyx Germany

First published: 01/02/2024

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Institution

Contact details

Study institution contact

Study Director Ultragenyx

clinicaltrialtransparency@ultragenyx.com

Study contact

clinicaltrialtransparency@ultragenyx.com

Primary lead investigator

Study Director Ultragenyx

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 23/01/2018

Actual: 23/01/2018

Study start date

Planned: 29/01/2018

Actual: 29/01/2018

Date of final study report

Planned: 15/05/2033

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ultragenyx

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)

Other study registration identification numbers and links

NCT03604835

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

Disease Monitoring Program

Main study objective:

The objectives of this study are to characterize MPS VII disease presentation and progression and assess long-term effectiveness and safety, including

hypersensitivity reactions and immunogenicity of vestronidase alfa.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

This DMP is a global, prospective, multicenter, longitudinal protocol focused on clinical presentation, heterogeneity, and disease progression. This is not a randomized study and both treated and untreated patients will be enrolled.

Study drug and medical condition

Medical condition to be studied

Mucopolysaccharidosis VII

Population studied

Age groups

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)
- Infants and toddlers (28 days – 23 months)
- Preterm newborn infants (0 – 27 days)
- Term newborn infants (0 – 27 days)
- 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

50

Study design details

Outcomes

1. Clinical Course of MPS VII Disease 2. Long-term Effectiveness of Vestronidase Alfa 3. Long-term Safety of Vestronidase Alfa

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Data analysis plan

The Full Analysis Set (FAS) will include all enrolled patients. All analyses will be done based on the FAS. With the small sample size and the heterogeneity among patients with MPS VII, no formal statistical testing is planned. Descriptive summary statistics will be generated depending on the sample size.

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