Morquio A Registry Study (MARS)

First published: 04/07/2014

Last updated: 17/05/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS6835

Study ID

49863

DARWIN EU® study

No

Study countries

- [ ] Australia
- [ ] Austria
- [ ] Belgium
- [ ] Canada
- [ ] Czechia
- [ ] Denmark
- [ ] France

- [ ] Ireland
- [ ] Italy
- [ ] Malaysia
- [ ] Netherlands
- [ ] Poland
- [ ] Portugal
- [ ] Puerto Rico
- [ ] Taiwan
- [ ] United Kingdom
- [ ] United States

Study description

A multicenter, multinational, observational Morquio A Registry Study (MARS) will be established to characterize and describe the MPS IVA population as a whole, including the heterogeneity, progression, and natural history of MPS IVA and to track the clinical outcomes of patients with MPS IVA treated with Vimizim®. The Registry will enroll and collect data on patients over a period of at least 8 years from the time of the first marketing approval globally and data on individual patients will continue to be collected for at least 2 years from the time the last patient was enrolled or until the Registry is terminated. It is not required that patients receive Vimizim to be eligible to participate in this Registry, however, they must have confirmed diagnosis of MPS IVA. Patients currently participating in a BMN 110 (elosulfase alfa) clinical trial will not meet inclusion criteria, but will be able to enroll after withdrawal from the clinical trial. The Registry will collect medical history, clinical, and safety assessments at least every six months or as indicated in the Recommended Schedule of Events, for up to 10 years. In addition this Registry will collect additional data on patients who have completed the MOR-005 and MOR-007 clinical trials. The MOR-005 and MOR-007 clinical trial patients will be enrolled into the appropriate Registry Substudy for a minimum of 5 years from the time of the patient's enrollment in the MOR-

005 or MOR-007 clinical study. After the 5-year period, these patients should remain in MARS until the Registry is complete. Relevant retrospective data may also be collected. Registry data collected using a validated web-based application will be analyzed as per the Registry's statistical analysis plan (SAP) and reported annually. The Morquio A Registry Study (MARS) will provide the necessary data to further characterize the spectrum of clinical signs and symptoms of the disease, and to further characterize the safety profile of Vimizim.

Study status

Ongoing

Contact details

Study institution contact

Sherry Kaye sherry.kaye@bmrn.com

Study contact

sherry.kaye@bmrn.com

Primary lead investigator

Director Program

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 28/04/2014

Actual: 28/04/2014

Study start date
Planned: 30/09/2014

Actual: 27/09/2014

Data analysis start date
Actual: 13/02/2015

Date of interim report, if expected
Planned: 30/06/2022

Actual: 22/06/2022

Date of final study report
Planned: 31/03/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioMarin International Limited

Study protocol

Morquio_A_Registry_Study_(MARS)_EU_PASS_Protocol_Version_5_15Jun2018_Final_.pdf (898.5 KB)

MARS Protocol Amendment 2, EU PASS V6 23Feb2021.pdf (1.01 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 1 (imposed as condition of marketing authorisation)

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:

1. To characterize and describe the MPS IVA population as a whole, including the heterogeneity, progression, and natural history of MPS IVA.
2. To evaluate the long-term effectiveness and safety of Vimizim, including but not limited to the occurrence of serious hypersensitivity reactions, anaphylaxis, and changes in antibody status.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Prescription event monitoring

Study drug and medical condition

Medicinal product name

VIMIZIM

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

ELOSULFASE ALFA

Anatomical Therapeutic Chemical (ATC) code

(A16AB12) elosulfase alfa

elosulfase alfa

Medical condition to be studied

Mucopolysaccharidosis IV

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

419

Study design details

Data analysis plan

Efficacy analysis will include all patients in the Efficacy Population, and will be conducted annually and over the duration of MARS. All efficacy variables will be summarized descriptively for baseline and post-baseline. When applicable for the specific efficacy variable, the change from the baseline to post-baseline (post–baseline at annual or semi-annual timepoints, depending on the specific efficacy variable), and/or its percent change will be summarized descriptively. The analyses of safety will include all patients in the Safety Population. Safety data, including vital signs, findings from physical examinations, concomitant medications, and other safety assessments, will be summarized descriptively. Incidence rate calculations will be completed. Where applicable, descriptive statistics will include the number of patients and mean, median, standard deviation, minimum, and maximum values for continuous variables and count and percent for categorical variables.

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

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