A multicentre, non-interventional study to evaluate long-term safety in patients with achondroplasia treated with Voxzogo® (vosoritide) (BMN 111-603)

First published: 20/10/2022

Last updated: 23/04/2024

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/50251

EU PAS number

EUPAS47514

Study ID

50251

DARWIN EU® study

No

Study countries

- [ ] Austria
- [ ] Belgium
- [ ] Czechia
- [ ] France
- [ ] Germany
- [ ] Italy
- [ ] Portugal
- [ ] Spain

Study description

This post-authorisation safety study (PASS, BMN 111-603) will evaluate the long-term safety of treatment with Voxzogo in subjects with achondroplasia (ACH) in a real-world setting. This study is designed to monitor long-term safety in an observational setting in line with standard of care, with a focus on long term skeletal effects. It aims to supplement long-term safety data which is being collected in a series of ongoing interventional, clinical trials.

Study status

Ongoing

Research institutions and networks

Institutions

Quintiles

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

111-603 Program Director

Study contact

medinfo@bmrn.com

Primary lead investigator

Director 111-603 Program

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 11/02/2022

Study start date

Planned: 31/03/2023

Actual: 17/04/2023

Date of final study report

Planned: 31/12/2035

Sources of funding

- Other

More details on funding

BioMarin International Ltd.

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

Main study objective:

To evaluate the long-term impact of treatment with Voxzogo on adverse bone-related safety events in subjects with ACH in a real-world setting.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

VOXZOGO

Medical condition to be studied

Osteochondrodysplasia

Population studied

Age groups

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Estimated number of subjects

330

Study design details

Outcomes

Incidence of new bone-related safety events of interest.

Data analysis plan

The primary endpoint will include the exposure-adjusted incidence rate (EAIR, derived as the number of new events divided by the total exposure person-time at risk) of all new bone-related safety events of interest which have not

previously been observed in the subject, which emerge during the course of the study. When calculating the exposure and person-time follow-up of events of interest, the exposure time period for each subject begins with the start of treatment index date and continues until the occurrence of the protocol-defined safety event, permanent discontinuation of treatment, death, or loss to follow-up. The induction period for risk with exposure is assumed to be immediate with exposure.

Data management

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