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

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

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Institution

Contact details

Study institution contact

111-603 Program Director medinfo@bmrn.com

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

Medicinal product name

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

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