

# 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

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### Study ID

50251

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### DARWIN EU® study

No






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### Study countries

 Austria

 Belgium

 Czechia

-  France
  -  Germany
  -  Italy
  -  Portugal
  -  Spain
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## 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.

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## 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 [medinfo@bmrn.com](mailto:medinfo@bmrn.com)

Study contact

[medinfo@bmrn.com](mailto: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

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### Study start date

Planned: 31/03/2023

Actual: 17/04/2023

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### 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

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## 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

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#### **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

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**Medical condition to be studied**

Osteochondrodysplasia

## Population studied

**Age groups**

- Children (2 to < 12 years)
  - Adolescents (12 to < 18 years)
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**Estimated number of subjects**

330

## Study design details

**Outcomes**

Incidence of new bone-related safety events of interest.

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**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](#)

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### 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

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No