An International Observational Registry Study to Further Describe Long-term Safety and Effectiveness of Palovarotene in Patients with Fibrodysplasia Ossificans Progressiva (FOP) (FOPaI)

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Study | Ongoing

Administrative details

EU PAS number

EUPAS1000000060

Study ID

1000000060

DARWIN EU® study

No

Study countries

Australia

Brazil

Canada

United States

Study description

The participants in this registry study will have fibrodysplasia ossificans progressiva (FOP). FOP is an ultra-rare, severely disabling disease characterized by new bone formation in areas of the body where bone is not normally present (heterotopic ossification (HO)). HO is often preceded by painful, recurrent episodes of soft tissue swelling (flare-ups).

This registry study will take place in countries where the treatment, known as palovarotene has been approved for use. Participants will already be receiving palovarotene as prescribed by their treating physician according to locally approved product information.

The main aim of this study will be to collect and assess real-world safety data on children and adult participants with FOP treated with palovarotene.

This study will also describe the effectiveness of this treatment, including the effect on everyday activities and physical performance.

Study status

Ongoing

Research institutions and networks

Institutions

Ipsen Pharma

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Institution

Contact details

Study institution contact

Ipsen Pharma clinical.trials@ipsen.com

Study contact

clinical.trials@ipsen.com

Primary lead investigator

Ipsen Medical Director

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 09/05/2020

Study start date

Planned: 30/05/2024

Actual: 05/12/2024

Data analysis start date

Planned: 30/05/2026

Date of final study report
Planned: 30/08/2036

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Approximate 100% funding: Ipsen Pharma

Study protocol

CLIN-60120-453_16.1.1 Protocol 2023SEP07_Redacted (1).pdf (6.31 MB)

CLIN-60120-453_16.1.1 Protocol V8.0 2025APR16_Redacted.pdf (8.49 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

Other study registration identification numbers and links

NCT06089616

Link to ClinicalTrials.gov

Methodological aspects

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Primary data collection

Study design:

The main aim of this study will be to collect and assess real-world safety data on children and adult participants with FOP treated with palovarotene. This study will also describe the effectiveness of this treatment, including the effect on everyday activities and physical performance.

Main study objective:

To collect and assess real-world safety data in patients with FOP treated with palovarotene.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name, other

Palovarotene / Sohonos

Anatomical Therapeutic Chemical (ATC) code

(M09AX11) palovarotene

palovarotene

Medical condition to be studied

Fibrodysplasia ossificans progressiva

Population studied

Age groups

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Special population of interest

Pregnant women

Estimated number of subjects

100

Study design details

Outcomes

Primary outcomes: Incidence and description of all TEAEs; of all serious and nonserious treatment-related TEAEs; of all serious TEAEs; all nonserious TEAEs; whether or not they are considered as related to the palovarotene during treatment period up to 30 days after the last dose.

Secondary outcomes: Use of Assistive Devices and Adaptions for Daily Living, Physical and Mental Function, investigator-reported flare-up outcomes, incidence of new bone growth, exposure.

Data analysis plan

Analysis will be primarily descriptive. Descriptive summary statistics will include number of documented data, mean, standard deviation, 95% confidence intervals (CI) of the mean/median, median, minimum, maximum, or frequency counts of the data collected. Percentages will be based on the number of non missing observations. Subgroup analyses will be performed by age group, gender. Interim effectiveness and safety descriptive analyses are planned to be performed every 2 years for publication purposes as required. For the first interim analysis, a minimum of 30 participants will have to be enrolled.

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
- Routine primary care electronic patient registry

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