

Clinical Trials Information #47

Principal Investigator

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Study Title:

Rollover Study; Multicentre, Phase III, Open-label Study to Further Evaluate the Safety and Efficacy of Palovarotene Capsules in Male and Female Participants Aged ≥ 14 Years with Fibrodysplasia Ossificans Progressiva (FOP) Who Have Completed Study PVO-1A-301 or PVO-1A-202/PVO-1A-204 and May Benefit from Palovarotene Therapy. (PIVOINE)

Purpose:

Treatment

Brief Description

The main objective of this study is to further evaluate the safety and efficacy of palovarotene in adult and pediatric participants with FOP.

The aim of the study is also to ensure treatment continuity to participants who have completed one of the parent studies (Study PVO-1A-301, Study PVO-1A-202 and Study PVO-1A-204) and who, in the investigator's judgement, may benefit from palovarotene therapy.

Eligibility

Inclusion Criteria:

Participant has completed the EOS or End of Treatment Visit of Study PVO-1A-301 or PVO-1A-202 (PVO-1A-202 Parts C and D correspond to Study PVO-1A-204 in France) and did not previously withdraw consent from any of the parent studies to be eligible for Study CLIN-60120-452.

Participant must be ≥ 14 years of age (aligned with the age of treated participants in the ongoing parent studies PVO-1A-301 and PVO-1A-202/PVO-1A-204) and qualify as 100% skeletally mature (if < 18 years, based on assessments carried out at parent EOS Visit; if ≥ 18 years, automatically considered 100% skeletally mature) or have reached final adult height based on investigator's assessment, at the time the Study CLIN- 60120-452 informed consent is signed.

Exclusion Criteria:

History of allergy or hypersensitivity to retinoids, gelatin, lactose (note that lactose intolerance is not exclusionary) or palovarotene, or unresponsiveness to prior treatment with palovarotene.

Uncontrolled cardiovascular, hepatic, pulmonary, gastrointestinal, endocrine, metabolic, ophthalmologic, immunologic, psychiatric, or other significant disease.

Symptomatic vertebral fracture.

Intercurrent known or suspected non-healed fracture at any location;

Any other medical condition/clinically significant abnormalities that would expose the participant to undue risk or interfere with study assessments.

Amylase or lipase $> 2 \times$ above the upper limit of normal (ULN) or with a history of chronic pancreatitis.

Elevated aspartate aminotransferase (AST) or alanine aminotransferase (ALT) $> 2.5 \times$ ULN.

Fasting triglycerides > 400 mg/dL with or without therapy.

Suicidal ideation (type 4 or 5) or any suicidal behaviour at the Inclusion Visit as defined by the Columbia-Suicide Severity Rating Scale (C-SSRS).

Current use of vitamin A or beta carotene, multivitamins containing vitamin A or beta carotene, or herbal preparations, fish oil, and unable or unwilling to discontinue use of these products during palovarotene treatment.

Exposure to synthetic oral retinoids other than palovarotene within 4 weeks of the Inclusion Visit.

Concurrent treatment with tetracycline or any tetracycline derivatives due to the potential increased risk of pseudotumor cerebri.

Use of concomitant medications that are strong inhibitors or inducers of cytochrome P450 (CYP450) 3A4 activity; or kinase inhibitors such as imatinib.

Palovarotene is reimbursed in the country where the study is being conducted.

Any reason that, in the opinion of the investigator, would lead to the inability of the participant and/or family to

comply with the protocol.

Which section would you like the trial listed under?	FOP Clinical Trials
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