A Phase 2, Randomized, Double-Blind, Placebo-Controlled Study to Evaluate the Efficacy, Safety, and Tolerability of INCB000928 in Participants With Fibrodysplasia Ossificans Progressiva (PROGRESS)

Published: 29-11-2022
Last updated: 10-01-2025

This study has been transitioned to CTIS with ID 2023-504129-38-00 check the CTIS register for the current data. Primary:To determine the efficacy of INCB000928 for the prevention of new HO in participants with FOP.Key Secondary:To further evaluate...

Ethical review	Approved WMO
Status	Pending
Health condition type	Musculoskeletal and connective tissue disorders congenital
Study type	Interventional

Summary

ID

NL-OMON54122

Source
ToetsingOnline

Brief title
PROGRESS

Condition

- Musculoskeletal and connective tissue disorders congenital

Synonym
rare connective tissue disease

Research involving

Human

Sponsors and support

Primary sponsor: Incyte Corporation
Source(s) of monetary or material Support: Incyte Corporation

Intervention

Keyword: Fibrodysplasia, FOP, INCB000928, Ossificans

Outcome measures

Primary outcome

Total volume of new HO as assessed by low dose WBCT (excluding the head) at Week 24.

Secondary outcome

• Total number of new flares (annualized) from baseline to Week 24.

• Proportion of participants with a clinically meaningful improvement in the flare-related symptoms assessed by the FOP-PROMPT at Week 24.

• Frequency and severity of AEs and SAEs, including the results of vital signs, ECGs, echocardiograms, physical examinations, PFTs, BMD, laboratory data, and knee epiphyseal closure (>= 12 to < 21 years of age).

• Proportion of participants with new HO over the 24-week period.

• Total number of new HO lesions as assessed by low-dose WBCT (excluding the head) from baseline to Week 24.

• Total volume of new HO as assessed by low-dose WBCT (excluding the head) from Week 24 to Week 48 compared to baseline to Week 24 in participants randomized to placebo during the double-blind period.

• Total number of new flares (annualized) from Week 24 to Week 48 compared to

baseline to Week 24 in participants randomized to placebo during the

double-blind period.

• Proportion of participants with new HO from Week 24 to Week 48 compared to

baseline to Week 24 in participants randomized to placebo during the

double-blind period.

• Number of new HO lesions as assessed by low-dose WBCT (excluding the head)

from Week 24 to Week 48 compared to baseline to Week 24 in participants

randomized to placebo during the double-blind period.

• INCB000928 PK for plasma and or saliva: Cmax, tmax, Cmin, and AUCt.

Study description

Background summary

See protocol section 2.1

Study objective

This study has been transitioned to CTIS with ID 2023-504129-38-00 check the CTIS register for the current data.

Primary:
To determine the efficacy of INCB000928 for the prevention of new HO in participants with FOP.

Key Secondary:
To further evaluate the efficacy of INCB000928 in the reduction of flares and improvement in flare-related symptoms.

Secondary:
- To further evaluate the efficacy of INCB000928 in the reduction of flares and improvement in flare-related symptoms.
- To evaluate the safety and tolerability of INCB000928 in participants with FOP.
- To further evaluate the efficacy of INCB000928 in participants with FOP.
- To confirm the efficacy of INCB000928 at Week 48 in participants randomized

to placebo during the double-blind period.
- To characterize the PK of INCB000928 in participants with FOP.

Study design

This is a Phase 2, multicenter, randomized, double-blind, placebo-controlled study to evaluate the efficacy, safety, tolerability, and PK of INCB000928 over a 24-week treatment period followed by a 148-week, open-label extension period (including OLE 1 Treatment Period [52 Weeks] and OLE 2 Treatment Period [96 Weeks]);It is estimated that an individual will participate for approximately 3.5 years.

Intervention

Participants will be randomized 1:1 to the INCB000928 100 mg QD treatment group or the placebo group (~ 30 participants per group), stratified by joint function based on CAJIS score at baseline.
Up to 32 days for screening; continuous treatment in the double-blind, placebo-controlled period for 24 weeks; a 52-week, open-label extension period; and a 4-week follow-up period. It is estimated that an individual will participate for approximately 21 months.

Study burden and risks

See protocol section 2.3. Benefit/Risk Assessment,

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Elderly (65 years and older)

Inclusion criteria

-Female and male adults and adolescents >= 12 years of age.
-Clinical diagnosis of FOP (based on findings of congenital malformation of the great
toes, episodic soft-tissue swelling, and/or progressive HO).
- Participant-reported FOP disease activity within 1 year of the screening visit. This is
defined as pain, swelling, and other signs and symptoms associated with FOP flare-ups or
worsening of joint function or radiographic progression of HO (increase in site or number
of HO lesions) with or without an association with flare-up episodes.

Exclusion criteria

-CAJIS score >= 24.
-FOP disease severity that in the investigator's opinion precludes participation (eg,
ankyloses of most or all joints, symptomatic thoracic insufficiency syndrome, or
recurrent respiratory infections).
-History of uncontrolled or unstable cardiovascular, respiratory, renal, gastrointestinal,
endocrine, hematopoietic, psychiatric, and/or neurological disease within 6 months of
screening.

Study design

Design

Study phase:	2
Study type:	Interventional
Intervention model:	Other
Allocation:	Randomized controlled trial
Masking:	Double blinded (masking used)
Control:	Placebo
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-12-2023
Enrollment:	5
Type:	Anticipated

Medical products/devices used

Product type:	Medicine
Brand name:	INCB000928
Generic name:	Not available

Ethics review

Approved WMO Date:	29-11-2022
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO Date:	28-06-2023
Application type:	First submission
Review commission:	METC Amsterdam UMC
Approved WMO	

Date:	10-10-2023
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	19-02-2024
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	21-03-2024
Application type:	Amendment
Review commission:	METC Amsterdam UMC
Approved WMO Date:	08-05-2024
Application type:	Amendment
Review commission:	METC Amsterdam UMC

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EU-CTR	CTIS2023-504129-38-00
EudraCT	EUCTR2021-002286-17-NL
ClinicalTrials.gov	NCT05090891
CCMO	NL78845.029.22