



Clinical trial results: Open-label Study to Evaluate the Pharmacokinetics, Safety, and Immunogenicity of Ustekinumab in Pediatric Participants Summary

EudraCT number	2021-005085-18
Trial protocol	Outside EU/EEA
Global end of trial date	26 January 2024

Results information

Result version number	v1 (current)
This version publication date	07 August 2024
First version publication date	07 August 2024

Trial information

Trial identification

Sponsor protocol code	CNT01275ISD1001
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05252533
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Janssen Research & Development, LLC
Sponsor organisation address	Turnhoutseweg 30, Beerse, Belgium, B-2340
Public contact	Clinical Registry Group, Janssen Research & Development, LLC, ClinicalTrialsEU@its.jnj.com
Scientific contact	Clinical Registry Group, Janssen Research & Development, LLC, ClinicalTrialsEU@its.jnj.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	01 March 2024
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	26 January 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective of trial was to evaluate pharmacokinetics (PK) of ustekinumab in juvenile psoriatic arthritis (jPsA) and pediatric psoriasis (PsO).

Protection of trial subjects:

This study was conducted in accordance with the ethical principles that have their origin in the Declaration of Helsinki and that are consistent with Good Clinical Practices and applicable regulatory requirements.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	24 May 2022
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 31
Worldwide total number of subjects	31
EEA total number of subjects	0

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	9
Adolescents (12-17 years)	22
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

A total of 31 subjects were enrolled in this study. Of these, 11 subjects were enrolled under disease cohort 1: Juvenile Psoriatic Arthritis (jPsA), and 20 subjects under disease cohort 2: Pediatric Psoriasis (PsO).

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Non-randomised - controlled
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
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Arm title	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)
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Arm description:

Subjects (aged greater than or equal to [$\geq$] 5 to less than [$<$] 18 years) who were previously diagnosed with jPsA and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating health care professional (HCP).

Arm type	Experimental
Investigational medicinal product name	Ustekinumab
Investigational medicinal product code	CNTO 1275
Other name	STELARA
Pharmaceutical forms	Injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Subjects who were previously diagnosed with jPsA and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating HCP.

Arm title	Cohort 2: Pediatric Psoriasis (PsO)
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Arm description:

Subjects (aged ≥ 5 to < 18 years) who were previously diagnosed with PsO and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating HCP.

Arm type	Experimental
Investigational medicinal product name	Ustekinumab
Investigational medicinal product code	CNTO 1275
Other name	STELARA
Pharmaceutical forms	Injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Subjects who were previously diagnosed with PsO and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating HCP.

Number of subjects in period 1	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	Cohort 2: Pediatric Psoriasis (PsO)
Started	11	20
Completed	9	19
Not completed	2	1
Unspecified	-	1
Lost to follow-up	1	-
Consent Withdrawn by Subject's Parent/Guardian	1	-

Baseline characteristics

Reporting groups

Reporting group title	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)
Reporting group description: Subjects (aged greater than or equal to [$\geq$] 5 to less than [$<$] 18 years) who were previously diagnosed with jPsA and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating health care professional (HCP).	
Reporting group title	Cohort 2: Pediatric Psoriasis (PsO)
Reporting group description: Subjects (aged ≥ 5 to < 18 years) who were previously diagnosed with PsO and treated with ustekinumab were enrolled this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating HCP.	

Reporting group values	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	Cohort 2: Pediatric Psoriasis (PsO)	Total
Number of subjects	11	20	31
Title for AgeCategorical Units: subjects			
Children (2-11 years)	0	9	9
Adolescents (12-17 years)	11	11	22
Title for AgeContinuous Units: years			
arithmetic mean	15.1	12.6	
standard deviation	± 1.51	± 3.27	-
Title for Gender Units: subjects			
Female	8	15	23
Male	3	5	8

End points

End points reporting groups

Reporting group title	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)
Reporting group description: Subjects (aged greater than or equal to [$\geq$] 5 to less than [$<$] 18 years) who were previously diagnosed with jPsA and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating health care professional (HCP).	
Reporting group title	Cohort 2: Pediatric Psoriasis (PsO)
Reporting group description: Subjects (aged ≥ 5 to <18 years) who were previously diagnosed with PsO and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating HCP.	

Primary: Observed Serum Concentration of Ustekinumab

End point title	Observed Serum Concentration of Ustekinumab ^[1]
End point description: Observed serum concentration of ustekinumab were reported. Pharmacokinetic (PK) analysis set included all enrolled subjects who received ustekinumab and had at least 1 sample drawn for PK analysis.	
End point type	Primary
End point timeframe: Cumulative PK samples from visits 1-4 (from Week 0 [baseline] up to Week 20)	
Notes: [1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point. Justification: No inferential statistics were performed for this endpoint.	

End point values	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	Cohort 2: Pediatric Psoriasis (PsO)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	11	20		
Units: micrograms per millilitre (mcg/mL)				
arithmetic mean (standard deviation)	3.346 ($\pm$ 3.2483)	2.006 ($\pm$ 1.7867)		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects with Treatment-emergent Serious Adverse Events (SAEs)

End point title	Number of Subjects with Treatment-emergent Serious Adverse Events (SAEs)
End point description: Number of subjects with treatment-emergent SAEs were reported. An adverse event (AE) is any	

untoward medical occurrence in a subject participating in a clinical study that does not necessarily have a causal relationship with the pharmaceutical/biological agent under study. An SAE is an AE resulting in any of the following outcomes or deemed significant for any other reason: death; initial or prolonged inpatient hospitalisation; life-threatening experience (immediate risk of dying); persistent or significant disability/incapacity; congenital anomaly/birth defect; suspected transmission of any infectious agent via a medicinal product or medically important. Treatment-emergent SAEs were those SAEs that occurred after signing of ICF up to end of study. Safety analysis set included all subjects enrolled in the study.

End point type	Secondary
End point timeframe:	
From Week 0 (Baseline) up to end of study (up to 20 weeks)	

End point values	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	Cohort 2: Pediatric Psoriasis (PsO)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	11	20		
Units: Subjects	0	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Subjects with Treatment-emergent Adverse Events (AEs)

End point title	Number of Subjects with Treatment-emergent Adverse Events (AEs)
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End point description:

Number of subjects with TEAEs were reported. An adverse event (AE) is any untoward medical occurrence in a subject participating in a clinical study that does not necessarily have a causal relationship with the pharmaceutical/biological agent under study. TEAEs were those AEs that occurred after signing of informed consent form (ICF) up to end of study. Safety analysis set included all subjects enrolled in the study.

End point type	Secondary
End point timeframe:	
From Week 0 (Baseline) up to end of study (up to 20 weeks)	

End point values	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	Cohort 2: Pediatric Psoriasis (PsO)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	11	20		
Units: Subjects	1	7		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From Week 0 (Baseline) up to end of study (up to 20 weeks)

Adverse event reporting additional description:

Safety analysis set included all subjects enrolled in the study.

Assessment type	Non-systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.1
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Reporting groups

Reporting group title	Cohort 2: Pediatric Psoriasis (PsO)
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Reporting group description:

Subjects (aged ≥ 5 to < 18 years) who were previously diagnosed with PsO and treated with ustekinumab were enrolled this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating HCP.

Reporting group title	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)
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Reporting group description:

Subjects (aged greater than or equal to ≥ 5 to less than < 18 years) who were previously diagnosed with jPsA and treated with ustekinumab were enrolled in this study and continued ustekinumab at the dose and frequency prescribed as per discretion of their treating health care professional (HCP).

Serious adverse events	Cohort 2: Pediatric Psoriasis (PsO)	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 20 (0.00%)	0 / 11 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	Cohort 2: Pediatric Psoriasis (PsO)	Cohort 1: Juvenile Psoriatic Arthritis (jPsA)	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 20 (35.00%)	1 / 11 (9.09%)	
Investigations			
HLA-B*27 positive			
subjects affected / exposed	0 / 20 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Nervous system disorders			

Headache			
subjects affected / exposed	1 / 20 (5.00%)	0 / 11 (0.00%)	
occurrences (all)	1	0	
Syncope			
subjects affected / exposed	1 / 20 (5.00%)	0 / 11 (0.00%)	
occurrences (all)	1	0	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	2 / 20 (10.00%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Nausea			
subjects affected / exposed	2 / 20 (10.00%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Vomiting			
subjects affected / exposed	3 / 20 (15.00%)	0 / 11 (0.00%)	
occurrences (all)	3	0	
Diarrhoea			
subjects affected / exposed	2 / 20 (10.00%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Reproductive system and breast disorders			
Dysmenorrhoea			
subjects affected / exposed	1 / 20 (5.00%)	0 / 11 (0.00%)	
occurrences (all)	1	0	
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	1 / 20 (5.00%)	0 / 11 (0.00%)	
occurrences (all)	1	0	
Nasal congestion			
subjects affected / exposed	1 / 20 (5.00%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Wheezing			
subjects affected / exposed	1 / 20 (5.00%)	0 / 11 (0.00%)	
occurrences (all)	1	0	
Skin and subcutaneous tissue disorders			
Psoriasis			

subjects affected / exposed occurrences (all)	1 / 20 (5.00%) 1	0 / 11 (0.00%) 0	
Musculoskeletal and connective tissue disorders Arthritis subjects affected / exposed occurrences (all)	0 / 20 (0.00%) 0	1 / 11 (9.09%) 1	
Infections and infestations Ear infection subjects affected / exposed occurrences (all) Influenza subjects affected / exposed occurrences (all) Nasopharyngitis subjects affected / exposed occurrences (all)	1 / 20 (5.00%) 1 1 / 20 (5.00%) 1 1 / 20 (5.00%) 1	0 / 11 (0.00%) 0 0 / 11 (0.00%) 0 0 / 11 (0.00%) 0	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 September 2022	The rational of this amendment was to address the recent Food and Drug Administration (FDA) approval of STELARA in juvenile psoriatic arthritis (jPsA) in children ages 6 and above, changes were made to align with the United States (US) label.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Limited safety data due to small sample size and short study duration. Limited interpretation of PK data due to the opportunistic study design (with variable dosing schedules), use of historical dosing records, and real-world dosing of ustekinumab.

Notes: