Clinical trial results:
A MULTICENTER, OPEN-LABEL, EXTENSION STUDY TO ASSESS THE LONG-TERM SAFETY AND EFFICACY OF CTP-543 IN ADULT PATIENTS WITH MODERATE TO SEVERE ALOPECIA AREATA

Summary

EudraCT number	2021-002365-18
Trial protocol	DE ES HU
Global end of trial date	18 July 2024

Results information

Result version number	v1 (current)
This version publication date	02 August 2025
First version publication date	02 August 2025

Trial information

Trial identification

Sponsor protocol code	CP543.5002

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05041803
WHO universal trial number (UTN)	-
Other trial identifiers	IND: 131,423

Notes:

Sponsors

Sponsor organisation name	Sun Pharmaceutical Industries, Inc.
Sponsor organisation address	2 Independence Way, Princeton, NJ, United States, 08540
Public contact	Manager, Punit Patel, Clinical.Trial@sunpharma.com
Scientific contact	Associate Director, Aryany Sanchez, Clinical.Trial@sunpharma.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	18 July 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	18 July 2024
Global end of trial reached?	Yes
Global end of trial date	18 July 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The overall objectives of the study are to evaluate long-term safety of CTP-543 and to assess long-term effects of CTP-543 on treating hair loss in adult patients with moderate to severe alopecia areata

Protection of trial subjects:

Prior to performing any study-related activities under the study protocol, written informed consent with the approved Informed Consent Form was obtained from the subject. All study subjects were required to read and sign an Informed Consent Form.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	27 September 2021
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	Poland: 107
Country: Number of subjects enrolled	Spain: 72
Country: Number of subjects enrolled	France: 91
Country: Number of subjects enrolled	Germany: 124
Country: Number of subjects enrolled	Hungary: 13
Worldwide total number of subjects	407
EEA total number of subjects	407

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)	0
Adolescents (12-17 years)	0
Adults (18-64 years)	403

From 65 to 84 years	4
85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Subjects who previously participated in a qualifying phase 3 study with CTP-543(CP543.3001 or CP543.3002), and who completed a 24-week Treatment Period on study drug (active or placebo), had the opportunity to enroll in this OLE study. A total of 407 subjects were enrolled: 114 subjects from CP543.3001 and 293 (72.0%) subjects from CP543.3002.

Period 1

Period 1 title	Overall trial: Open label Extension (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Not blinded

Blinding implementation details:

The study was not blinded. Initially, subjects were assigned to receive the same daily treatment at a dose of 8 mg or 12 mg CTP-543 BID as in the qualifying study. Subjects who received placebo in the qualifying study were randomized in a 1:1 ratio to CTP543 8 mg BID or CTP-543 12 mg BID.

Arms

Are arms mutually exclusive?	Yes
Arm title	CTP-543 8 mg BID

Arm description:

Participants received CTP-543 8 mg tablets, orally, twice a day

Arm type	Experimental
Investigational medicinal product name	CTP-543
Investigational medicinal product code	
Other name	Deuruxolitinib
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

CTP-543 8 mg tablets, orally, twice a day

Arm title	CTP-543 12 mg BID

Arm description:

Participants received CTP-543 12 mg tablets, orally, twice a day

Arm type	Experimental
Investigational medicinal product name	CTP-543
Investigational medicinal product code	
Other name	Deuruxolitinib
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Deuruxolitinib (CTP-543) 12 mg tablets, orally, twice a day

Arm title	CTP-543 8 mg BID to CTP-543 12 mg BID

Arm description:

Participants received CTP-543 12 mg tablets, orally, twice a day, after they received CTP-543 8 mg tablets, orally, twice a day

Arm type	Experimental

Investigational medicinal product name	CTP-543
Investigational medicinal product code	
Other name	Deuruxolitinib
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

CTP-543 8 mg tablets, orally, twice a day

Investigational medicinal product name	CTP-543
Investigational medicinal product code	
Other name	Deuruxolitinib
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Deuruxolitinib (CTP-543) 12 mg tablets, orally, twice a day

Arm title	CTP-543 12 mg BID to CTP-543 8 mg BID

Arm description:

Participants received CTP-543 8 mg tablets, orally, twice a day, after they received CTP-543 12 mg tablets, orally, twice a day

Arm type	Experimental
Investigational medicinal product name	CTP-543
Investigational medicinal product code	
Other name	Deuruxolitinib
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

CTP-543 8 mg tablets, orally, twice a day

Investigational medicinal product name	CTP-543
Investigational medicinal product code	
Other name	Deuruxolitinib
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Deuruxolitinib (CTP-543) 12 mg tablets, orally, twice a day

Number of subjects in period 1	CTP-543 8 mg BID	CTP-543 12 mg BID	CTP-543 8 mg BID to CTP-543 12 mg BID
Started	137	46	115
Completed	111	27	87
Not completed	26	19	28
Consent withdrawn by subject	15	14	18
Physician decision	3	-	6
Other	1	-	-
Pregnancy	2	1	-
Lost to follow-up	2	1	3
Treatment Emergement or Worsening Adverse Event	3	3	1

Number of subjects in period 1	CTP-543 12 mg BID to CTP-543 8 mg BID
Started	109
Completed	103
Not completed	6
Consent withdrawn by subject	3
Physician decision	1
Other	1
Pregnancy	-
Lost to follow-up	1
Treatment Emergement or Worsening Adverse Event	-

Baseline characteristics

Reporting groups	
Reporting group title	CTP-543 8 mg BID
Reporting group description:	
Participants received CTP-543 8 mg tablets, orally, twice a day	
Reporting group title	CTP-543 12 mg BID
Reporting group description:	
Participants received CTP-543 12 mg tablets, orally, twice a day	
Reporting group title	CTP-543 8 mg BID to CTP-543 12 mg BID
Reporting group description:	
Participants received CTP-543 12 mg tablets, orally, twice a day, after they received CTP-543 8 mg tablets, orally, twice a day	
Reporting group title	CTP-543 12 mg BID to CTP-543 8 mg BID
Reporting group description:	
Participants received CTP-543 8 mg tablets, orally, twice a day, after they received CTP-543 12 mg tablets, orally, twice a day	

Reporting group values	CTP-543 8 mg BID	CTP-543 12 mg BID	CTP-543 8 mg BID to CTP-543 12 mg BID
Number of subjects	137	46	115
Age categorical Units: Subjects			
Adults (18-64 years)	136	45	115
From 65-84 years	1	1	0
Age continuous Units: years			
arithmetic mean	38.55	39.20	39.84
standard deviation	± 12.588	± 14.784	± 12.351
Gender categorical Units: Subjects			
Female	96	21	83
Male	41	25	32

Reporting group values	CTP-543 12 mg BID to CTP-543 8 mg BID	Total	
Number of subjects	109	407	
Age categorical Units: Subjects			
Adults (18-64 years)	107	403	
From 65-84 years	2	4	
Age continuous Units: years			
arithmetic mean	37.31		
standard deviation	± 11.794	-	
Gender categorical Units: Subjects			
Female	70	270	

Male	39	137	

End points

End points reporting groups

Reporting group title	CTP-543 8 mg BID
Reporting group description:	
Participants received CTP-543 8 mg tablets, orally, twice a day	
Reporting group title	CTP-543 12 mg BID
Reporting group description:	
Participants received CTP-543 12 mg tablets, orally, twice a day	
Reporting group title	CTP-543 8 mg BID to CTP-543 12 mg BID
Reporting group description:	
Participants received CTP-543 12 mg tablets, orally, twice a day, after they received CTP-543 8 mg tablets, orally, twice a day	
Reporting group title	CTP-543 12 mg BID to CTP-543 8 mg BID
Reporting group description:	
Participants received CTP-543 8 mg tablets, orally, twice a day, after they received CTP-543 12 mg tablets, orally, twice a day	

Primary: Relative Change from Pre-zero Baseline (to 108 weeks) in Total SALT Scores

End point title	Relative Change from Pre-zero Baseline (to 108 weeks) in Total SALT Scores[1]
End point description:	
SALT is a quantitative assessment of scalp hair loss with scores ranging from 0 (no scalp hair loss) to 100 (complete scalp hair loss). This outcome measures the relative Change from Pre-zero Baseline in Total SALT Scores. Relative change from baseline was defined as 100*(post-baseline value - baseline)/baseline.	
End point type	Primary
End point timeframe:	
108 weeks	

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: The primary efficacy endpoint was presented with descriptive statistics with no statistical hypothesis testing. It was based on the Efficacy Population. Pre-zero baseline was defined as the last observation obtained prior to first dose of active drug in the study or a previous qualifying study.

End point values	CTP-543 8 mg BID	CTP-543 12 mg BID	CTP-543 8 mg BID to CTP-543 12 mg BID	CTP-543 12 mg BID to CTP-543 8 mg BID
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	97	0[2]	48	99
Units: score on a scale				
arithmetic mean (standard deviation)	-96.38 (± 8.790)	()	-88.57 (± 18.715)	-90.25 (± 18.860)

Notes:

[2] - 12 mg BID dose was discontinued

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 108 Weeks	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	23.1

Reporting groups	
Reporting group title	CTP-543 8 mg BID
Reporting group description: -	
Reporting group title	CTP-543 12 mg BID
Reporting group description: -	

Serious adverse events	CTP-543 8 mg BID	CTP-543 12 mg BID	
Total subjects affected by serious adverse events			
subjects affected / exposed	16 / 367 (4.36%)	5 / 273 (1.83%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events			
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Gastric cancer stage IV			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Tongue neoplasm malignant stage unspecified			
subjects affected / exposed	0 / 367 (0.00%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pregnancy, puerperium and perinatal conditions			
Abortion spontaneous			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Immune system disorders			
Anaphylactic reaction			

subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Reproductive system and breast disorders			
Bartholin's cyst			
subjects affected / exposed	0 / 367 (0.00%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Menorrhagia			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory, thoracic and mediastinal disorders			
Asthma			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pulmonary embolism			
subjects affected / exposed	0 / 367 (0.00%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Injury, poisoning and procedural complications			
Humerus fracture			
subjects affected / exposed	0 / 367 (0.00%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Spinal fracture			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Tibia fracture			

subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac disorders			
Tricuspid valve incompetence			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Intracranial hypotension			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	1 / 367 (0.27%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 1	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Musculoskeletal and connective tissue disorders			
Osteoarthritis			
subjects affected / exposed	2 / 367 (0.54%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Synovial cyst			
subjects affected / exposed	0 / 367 (0.00%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Hepatitis E			

subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infectious mononucleosis			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Tonsillitis			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Wound infection			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bartholin's abscess			
subjects affected / exposed	0 / 367 (0.00%)	1 / 273 (0.37%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Metabolism and nutrition disorders			
Type 1 diabetes mellitus			
subjects affected / exposed	1 / 367 (0.27%)	0 / 273 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	CTP-543 8 mg BID	CTP-543 12 mg BID	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	176 / 367 (47.96%)	127 / 273 (46.52%)	
Investigations			
Blood creatine phosphokinase increased			
subjects affected / exposed	42 / 367 (11.44%)	24 / 273 (8.79%)	
occurrences (all)	53	27	

Weight increased			
subjects affected / exposed	14 / 367 (3.81%)	14 / 273 (5.13%)	
occurrences (all)	15	14	
Nervous system disorders			
Headache			
subjects affected / exposed	23 / 367 (6.27%)	9 / 273 (3.30%)	
occurrences (all)	27	10	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	13 / 367 (3.54%)	14 / 273 (5.13%)	
occurrences (all)	16	17	
Thrombocytosis			
subjects affected / exposed	20 / 367 (5.45%)	26 / 273 (9.52%)	
occurrences (all)	30	28	
Infections and infestations			
Asymptomatic COVID-19			
subjects affected / exposed	83 / 367 (22.62%)	58 / 273 (21.25%)	
occurrences (all)	92	65	
COVID-19			
subjects affected / exposed	78 / 367 (21.25%)	44 / 273 (16.12%)	
occurrences (all)	81	46	
Upper respiratory tract infection			
subjects affected / exposed	30 / 367 (8.17%)	14 / 273 (5.13%)	
occurrences (all)	50	16	
Folliculitis			
subjects affected / exposed	4 / 367 (1.09%)	14 / 273 (5.13%)	
occurrences (all)	4	14	
Nasopharyngitis			
subjects affected / exposed	76 / 367 (20.71%)	48 / 273 (17.58%)	
occurrences (all)	118	71	
Metabolism and nutrition disorders			
Hypercholesterolaemia			
subjects affected / exposed	41 / 367 (11.17%)	29 / 273 (10.62%)	
occurrences (all)	46	32	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
28 September 2021	Protocol amendment included the following changes: 1. The addition of pregnancy testing on a monthly basis. 2. Amended vital sign collection to allow for flexibility with the type of assessment measure for temperature.
21 October 2022	1. Amended to include 1 additional year of treatment with CTP-543 for responders (up to 108 weeks)
30 May 2023	Following updates we made to the protocol: 1. Name change due to acquisition of Concert Pharmaceuticals by Sun Pharmaceutical Industries, Inc. 2. Due to an urgent Safety Measure, the 12 mg BID dose was discontinued and all patients previously assigned to the 12 mg BID dose were reduced to 8 mg BID dose, if they elected to continue in the trial

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? Yes

Date	Interruption	Restart date
28 April 2023	Dosing with 12 mg BID was interrupted due to an urgent safety measure	-

Notes:

Limitations and caveats

None reported