Clinical trial results:
A Phase Ib/IIa, randomized, double blind, placebo controlled, multicenter clinical trial to evaluate the safety, pharmacokinetics and efficacy of oral treatment with OST-122 in patients with moderate to severe ulcerative colitis

Summary

EudraCT number	2019-004090-50
Trial protocol	ES
Global end of trial date	27 December 2022

Results information

Result version number	v1 (current)
This version publication date	17 June 2026
First version publication date	17 June 2026

Trial information

Trial identification

Sponsor protocol code	CT-OST-122-02

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04353791
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Oncostellae S.L
Sponsor organisation address	Edificio FEUGA D5. Rúa Lope Gómez de Marzoa s/n, Santiago de Compostela, Spain, 15705
Public contact	Guido Kurz, Oncostellae S.L., +34 617 640 327, guido.kurz@oncostellae.com
Scientific contact	Guido Kurz, Oncostellae S.L., +34 617 640 327, guido.kurz@oncostellae.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 December 2023
Is this the analysis of the primary completion data?	Yes
Primary completion date	27 December 2022
Global end of trial reached?	Yes
Global end of trial date	27 December 2022
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The trial primary goal will be to evaluate the safety and tolerability of OST-122 in patients with moderate to severe active ulcerative colitis disease over 28 days of trearment.

Protection of trial subjects:

The study was conducted in compliance with the ICH Guidelines for Good Clinical Practice (GCP), the ethical principles derived from the Declaration of Helsinki, applicable local regulatory requirements, and the study protocol. All candidates taking part in the study were individually and fully informed about the nature of the study (aims, type of treatment, methodology) and the drug tested (expected risk/benefits, possible side effects) as well as their rights and responsibilities if they decided to participate. All were required to read a Patient Information Sheet and sign an Informed Consent Form (ICF).

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	30 September 2020
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	Spain: 21
Country: Number of subjects enrolled	Ukraine: 16
Worldwide total number of subjects	37
EEA total number of subjects	21

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)	37
From 65 to 84 years	0

85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

Subjects were eligible if aged 18 to 75 years with moderate to severe ulcerative active colitis (Total Mayo score 5-10, Endoscopic score ≥2) and with inadequate/loss of response, or intolerance to conventional (steroids and immunomodulators) and/or advanced therapy (ie. anti-TNFα, anti-integrin, anti-interleukin 12/23).

Period 1

Period 1 title	Overall trial (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Assessor

Arms

Are arms mutually exclusive?	Yes
Arm title	OST-122 400mg

Arm description:

Subjects received 400mg of OST-122 in a single oral daily dose for 28 days

Arm type	Experimental
Investigational medicinal product name	OST-122
Investigational medicinal product code	OST-122
Other name	
Pharmaceutical forms	Capsule, hard
Routes of administration	Oral use

Dosage and administration details:

Size 0 hard gelatine capsules filled with OST-122 and cellulose microcrystalline packed in blisters containing each one 200mg of OST-122.
OST-122 was administered in the morning, under fasting conditions and with 240 mL of water. Fasting conditions were kept for an additional hour and a half after medication intake.

Arm title	Placebo

Arm description:

Subjects received 400mg of Placebo in a single oral daily dose for 28 days

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule, hard
Routes of administration	Oral use

Dosage and administration details:

Size 0 hard gelatine capsules filled with Placebo and cellulose microcrystalline packed in blisters containing each one 200mg. It was administered in the morning, under fasting conditions and with 240 mL of water. Fasting conditions were kept for an additional hour and a half after medication intake.

Number of subjects in period 1	OST-122 400mg	Placebo
Started	28	9
Completed	27	9
Not completed	1	0
Adverse event, non-fatal	1	-

Baseline characteristics

Reporting groups	
Reporting group title	OST-122 400mg
Reporting group description:	
Subjects received 400mg of OST-122 in a single oral daily dose for 28 days	
Reporting group title	Placebo
Reporting group description:	
Subjects received 400mg of Placebo in a single oral daily dose for 28 days	

Reporting group values	OST-122 400mg	Placebo	Total
Number of subjects	28	9	37
Age categorical Units: Subjects			
In utero	0	0	0
Preterm newborn infants (gestational age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-11 years)	0	0	0
Adolescents (12-17 years)	0	0	0
Adults (18-64 years)	28	9	37
From 65-84 years	0	0	0
85 years and over	0	0	0
Age continuous Units: years			
arithmetic mean	43.82	47.89	
standard deviation	± 13.82	± 13.92	-
Gender categorical Units: Subjects			
Female	11	4	15
Male	17	5	22
Montreal classification - Ulcerative colitis extent Units: Subjects			
E1- Proctitis	4	4	8
E2- Left-sided colitis	13	5	18
E3- Extensive colitis	11	0	11
Montreal classification- Ulcerative colitis severity Units: Subjects			
S1 - Mild	2	0	2
S2 - Moderate	22	8	30
S3 - Severe	4	1	5
Ethnicity Units: Subjects			
Caucasian	23	9	32
Black	1	0	1
Other	4	0	4

Total Mayo Score			
Units: score			
arithmetic mean	8.07	6.67	
standard deviation	± 1.11	± 1.22	-

End points

End points reporting groups

Reporting group title	OST-122 400mg
Reporting group description:	
Subjects received 400mg of OST-122 in a single oral daily dose for 28 days	
Reporting group title	Placebo
Reporting group description:	
Subjects received 400mg of Placebo in a single oral daily dose for 28 days	

Primary: Safety and tolerability of OST-122

End point title	Safety and tolerability of OST-122[1]
End point description:	
Safety evaluations included the incidence, severity, and relationship of adverse events (AEs), as well as clinically significant changes in vital signs, physical examination findings, laboratory parameters, and electrocardiograms (ECGs).	
End point type	Primary
End point timeframe:	
Safety and tolerability of OST-122 was assessed from baseline to Day 43 (end of follow-up)	

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 number of subjects with at least one adverse event was summarised by proportion and 95% confidence interval using exact methods based on the binomial Clopper-Pearson method, independently of treatment groups and by type of AE. Comparison between treatment groups in the number (%) of subjects reporting one or more treatment-emergent AEs by preferred term was performed using Fisher's exact test.

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	28	9		
Units: percent				
number (not applicable)				
Any adverse event	71.4	77.8		
Any treatment-related adverse event	3.6	0		
Any serious adverse event	0	0		
Any treatment-related serious adverse event	0	0		
Any adverse event with outcome of death	0	0		
Any adverse event leading to discontinuation	3.6	0		
Any treatment-related adverse event leading to dis	0	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving clinical response at Day 28

End point title	Percentage of participants achieving clinical response at Day 28

End point description:

Clinical Response is defined as an improvement in the Total Mayo Score by ≥ 3 points and a decrease of 30% versus baseline, plus a decrease in rectal bleeding by ≥ 1 points and an absolute rectal bleeding score of 0 or 1.

End point type	Secondary

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	27	9		
Units: percent				
number (confidence interval 95%)	44.4 (25.5 to 64.7)	11.1 (0.3 to 48.2)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants achieving clinical remission at Day 28

End point title	Percentage of participants achieving clinical remission at Day 28

End point description:

Clinical remission is defined as a Total Mayo Score ≤ 2 and no individual subscore >1 plus a rectal bleeding score of 0

End point type	Secondary

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	27	9		
Units: percent				
number (confidence interval 95%)	22.2 (8.6 to 42.3)	0 (0 to 28.3)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with an improvement of the Mayo rectal bleeding subscore by ≥1 point at Day 28

End point title	Percentage of participants with an improvement of the Mayo rectal bleeding subscore by ≥1 point at Day 28

End point description:

The rectal bleeding score is one of the four subscores of the total Mayo score and assesses the severity of rectal bleeding. It is measured on a scale from 0 to 3, where 0 = no blood seen; 1 = streaks of blood with stool less than half the time; 2 = obvious blood with stool most of the time; and 3 = blood alone passed

End point type	Secondary

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	27	9		
Units: percent				
number (confidence interval 95%)	63 (42.4 to 80.6)	33.3 (7.5 to 70.1)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with an improvement of the Mayo stool frequency subscore by ≥ 1 point al Day 28

End point title	Percentage of participants with an improvement of the Mayo stool frequency subscore by ≥ 1 point al Day 28

End point description:

The stool frequency score is one of the four subscores of the total Mayo score and assesses the increase in stool frequency relative to normal. It is measured on a scale from 0 to 3, where 0 = normal number of stools; 1 = 1–2 stools more than normal; 2 = 3–4 stools more than normal; and 3 = ≥5 stools more than normal.

End point type	Secondary

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	27	9		
Units: percent				
number (confidence interval 95%)	51.9 (31.9 to 71.3)	33.3 (7.5 to 70.1)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with improvement of PRO-2 by ≥2 points

End point title	Percentage of participants with improvement of PRO-2 by ≥2 points

End point description:

The PRO-2 is a patient-reported outcome derived from the stool frequency score and rectal bleeding score components of the total Mayo score and is defined as the sum of these two subscores.

End point type	Secondary

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	27	9		
Units: percent				
number (confidence interval 95%)	48.1 (28.7 to 68.1)	11.1 (0.3 to 48.2)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with an improvement of the Mayo endoscopic subscore by ≥1 point at Day 28

End point title	Percentage of participants with an improvement of the Mayo endoscopic subscore by ≥1 point at Day 28

End point description:

The endoscopic subscore is one of the four subscores of the total Mayo score and assesses mucosal appearance on endoscopy. It is measured on a scale from 0 to 3, where 0 = normal or inactive disease; 1 = mild disease (erythema, decreased vascular pattern, mild friability); 2 = moderate disease (marked erythema, absent vascular pattern, friability, erosions); and 3 = severe disease (spontaneous bleeding, ulceration).

End point type	Secondary

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	27	9		
Units: Percentage				
number (confidence interval 95%)	29.6 (13.2 to 48.7)	33.3 (7.5 to 70.1)		

Statistical analyses

No statistical analyses for this end point

Other pre-specified: Pharmacokinetics

End point title	Pharmacokinetics[2]

End point description:

The following pharmacokinetic parameters were calculated on Day 1 and Day 28 (endo of treatment, EoT):
Cmax: Maximum observed plasma concentration, obtained directly from the data – without interpolation.
Area under the curve (AUC): Cumulative area under the plasma concentration time curve calculated from 0 to the last observed concentration above the Lower Limit Of Quantification (LLOQ), using the mixed model linear log trapezoidal rule.
Tmax: Time of maximum observed plasma concentration; obtained directly from the data, without interpolation. If it occurs at more than one time point, Tmax was defined as the first time point with this value.
Ctrough: concentration reached by a drug immediately before the next dose is administered.

End point type	Other pre-specified

End point timeframe:

Blood samples for parent compound determination were collected at baseline [pre-dose], [+ 10 min], [+ 20 min], [+ 40 min], [+ 60 min], [+ 1.5h], [+ 2 h], [+ 2.5 h], [+ 3h], [+ 4h], and [+ 6h], post-medication on Days 1 and 28 (End of treatment).

Notes:

[2] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: Justification: Placebo arms do not have parental compound, therefore descriptive statistics are not applicable.

End point values	OST-122 400mg			
Subject group type	Reporting group			
Number of subjects analysed	28			
Units: Pk parameters				
arithmetic mean (standard deviation)				
Cmax (ng/mL) - Treatment Day 1	0.769 (± 0.696)			
Tmax (h) - Treatment Day 1	3.34 (± 1.54)			
Ctrough (ng/mL) - Treatment Day 1	0.001 (± 0)			
AUC - Treatment Day 1	2.49 (± 2.36)			
Cmax (ng/mL) - Treatment Day 28 (EoT)	0.91 (± 0.59)			
Tmax (h) - Treatment Day 28 (EoT)	3.62 (± 2)			
Ctrough (ng/mL) - Treatment Day 28 (EoT)	0.47 (± 0.45)			
AUC - treatment Day 28 (EoT)	4.07 (± 2.7)			

Statistical analyses

No statistical analyses for this end point

Post-hoc: Percentage of participants achieving histological response at Day 28

End point title	Percentage of participants achieving histological response at Day 28

End point description:

Histological response is defined as a Robarts Histopathology Index ≤ 9 at Day 28 and baseline RHI > 9, with subscores of 0 for neutrophils in epithelium and without ulcers or erosions (in R1 or R2 location).

End point type	Post-hoc

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	25	9		
Units: Percentage				
number (confidence interval 95%)	16 (4.5 to 36.1)	0 (0 to 28.3)		

Statistical analyses

No statistical analyses for this end point

Post-hoc: Percentage of participants achieving histological remission at Day 28

End point title	Percentage of participants achieving histological remission at Day 28

End point description:

Defined as the percentage of subjects with Robarts Histopathological Index (RHI) ≤ 3 at Day 28 and baseline RHI > 3, with subscores of 0 for neutrophils in lamina propria and epithelium and without ulcers or erosions (in region R1 or R2).

End point type	Post-hoc

End point timeframe:

Day 28

End point values	OST-122 400mg	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	25	9		
Units: Percentage				
number (confidence interval 95%)	16 (4.5 to 36.1)	0 (0 to 28.3)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Adverse events (AEs) were collected from the time when the subject signed the consent form until the last final follow-up visit (or at the time of early discontinuation of the subject from the study)

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	25.1

Reporting groups

Reporting group title	OST-122 400mg
Reporting group description: -	
Reporting group title	Placebo

Reporting group description: -

Serious adverse events	OST-122 400mg	Placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 28 (0.00%)	0 / 9 (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	OST-122 400mg	Placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	20 / 28 (71.43%)	7 / 9 (77.78%)	
Vascular disorders			
Hypertension			
subjects affected / exposed	2 / 28 (7.14%)	0 / 9 (0.00%)	
occurrences (all)	3	0	
Cardiac disorders			
Sinus tachycardia			
subjects affected / exposed	0 / 28 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Nervous system disorders			
Headache			

subjects affected / exposed	4 / 28 (14.29%)	3 / 9 (33.33%)	
occurrences (all)	4	3	
Syncope			
subjects affected / exposed	0 / 28 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	3 / 28 (10.71%)	0 / 9 (0.00%)	
occurrences (all)	4	0	
General disorders and administration site conditions			
Hyperthermia			
subjects affected / exposed	0 / 28 (0.00%)	2 / 9 (22.22%)	
occurrences (all)	0	2	
Ear and labyrinth disorders			
Meniere's disease			
subjects affected / exposed	0 / 28 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 28 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Abdominal pain upper			
subjects affected / exposed	1 / 28 (3.57%)	1 / 9 (11.11%)	
occurrences (all)	3	1	
Flatulence			
subjects affected / exposed	1 / 28 (3.57%)	1 / 9 (11.11%)	
occurrences (all)	3	1	
Colitis ulcerative			
subjects affected / exposed	8 / 28 (28.57%)	2 / 9 (22.22%)	
occurrences (all)	9	2	
Skin and subcutaneous tissue disorders			
Hyperhidrosis			
subjects affected / exposed	0 / 28 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	
Infections and infestations			

COVID-19			
subjects affected / exposed	1 / 28 (3.57%)	1 / 9 (11.11%)	
occurrences (all)	1	1	
Nasopharyngitis			
subjects affected / exposed	2 / 28 (7.14%)	1 / 9 (11.11%)	
occurrences (all)	2	1	
Oral herpes			
subjects affected / exposed	0 / 28 (0.00%)	1 / 9 (11.11%)	
occurrences (all)	0	1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
08 January 2021	Due to the COVID-19 pandemic, mitigation measures were implemented including the replacement of selected on-site visits with telephone contacts to minimise patient travel while ensuring adequate monitoring. Additionally, inclusion of patients with inadequate response or intolerance to Ustekinumab, Tofacitinib or other JAK inhibitors was allowed, subject to a 60-day washout period.
19 August 2021	The number of participants in the 400 mg cohort was increased while maintaining the overall sample size, with removal of the 800 mg cohort. This adjustment strengthens the robustness and interpretability of results and supports the assessment of safety, tolerability, pharmacokinetics and preliminary efficacy in line with the exploratory nature of this proof-of-concept study.
23 December 2021	The washout period for prior medications was reduced to allow patients with moderate to severe active ulcerative colitis, inadequately controlled with current therapy, to access IMP as early as possible. This is particularly relevant given that OST-122 is a selective JAK3/TYK2 inhibitor designed to act exclusively at the gastrointestinal tract level, with limited oral absorption and rapid hepatic metabolism. This pharmacokinetic profile makes a prolonged washout period unnecessary from a safety perspective.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported