



Clinical trial results:

A Phase 2, Multicenter, Randomized, Double-Blind, Placebo-Controlled, Parallel Dose Study to Evaluate the Efficacy and Safety of Oral SKI-O-703, SYK Inhibitor, in Patients with Persistent and Chronic Immune Thrombocytopenia (ITP)

Summary

EudraCT number	2018-003329-26
Trial protocol	PL ES GR
Global end of trial date	10 January 2023

Results information

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

Trial information

Trial identification

Sponsor protocol code	OSCO-P2101
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Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Oscotec Inc.
Sponsor organisation address	Korea Bio-Park, Building A, 9th Floor 700 Daewangpangyo-ro, Seongnam-si, Korea, Republic of, 13488
Public contact	Sungsil Lee, Oscotec Inc., 82 31 628 7627, sslee@oscotec.com
Scientific contact	Sungsil Lee, Oscotec Inc., 82 31 628 7627, sslee@oscotec.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	25 July 2023
Is this the analysis of the primary completion data?	Yes
Primary completion date	10 January 2023
Global end of trial reached?	Yes
Global end of trial date	10 January 2023
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and efficacy on the primary endpoint (platelet response) of select (200 mg BID and 400 mg BID) doses of SKI-O-703 compared to placebo in patients with persistent and chronic ITP, with a platelet count <30,000/ μ L on 2 occasions at least 7 days apart with the confirmatory count on the first day of treatment.

Protection of trial subjects:

The study was performed in accordance with the ethical principles that have their origin in the Declaration of Helsinki, ICH GCP, the protocol, and all applicable regulations.

Background therapy:

Subjects were allowed to receive stable doses of background medications in line with the current standard of care. The background medications allowed included corticosteroids (<20 mg prednisone equivalent per day) and immunosuppressive drugs such as azathioprine ($\leq$ 50 mg twice daily), mycophenolate mofetil ($\leq$ 500 mg twice daily), and cyclosporine ($\leq$ 100 mg twice daily). The dose of such background medications was fixed for at least 2 weeks before Day 1 and remained unchanged until 12 weeks of treatment was completed or unless rescue therapy was required.

Evidence for comparator:

This study is placebo controlled and placebo is used for comparator.

Actual start date of recruitment	06 December 2019
Long term follow-up planned	Yes
Long term follow-up rationale	Efficacy, Safety
Long term follow-up duration	1 Months
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Poland: 12
Country: Number of subjects enrolled	Spain: 16
Country: Number of subjects enrolled	Greece: 20
Country: Number of subjects enrolled	Korea, Republic of: 11
Country: Number of subjects enrolled	United States: 1
Worldwide total number of subjects	60
EEA total number of subjects	48

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)	39
From 65 to 84 years	19
85 years and over	2

Subject disposition

Recruitment

Recruitment details:

A total of 61 subjects were randomly assigned to receive the study drugs, of whom 60 subjects were included in the ITT set and the safety set each. Note: 1 subject was randomly assigned to the 400 mg BID group but did not receive any dose of study drug as the subject was withdrawn due to noncompliance with the protocol.

Pre-assignment

Screening details:

The study included up to 4 weeks of screening period. A total of 85 subjects were screened, of whom 24 subjects were screen failures.

Period 1

Period 1 title	12-Week Treatment Period (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Monitor, Data analyst, Carer, Assessor

Arms

Are arms mutually exclusive?	Yes
Arm title	SKI-O-703 200 mg BID

Arm description:

2 capsules of 100 mg SKI-O-703 BID (twice per day)

Arm type	Experimental
Investigational medicinal product name	SKI-O-703 200 mg BID
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule
Routes of administration	Oral use

Dosage and administration details:

200 mg BID group: 2 capsules of 100 mg SKI-O-703 + 2 capsules of placebo

Arm title	SKI-O-703 400 mg BID
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Arm description:

4 capsules of 100 mg SKI-O-703 BID (twice per day)

Arm type	Experimental
Investigational medicinal product name	SKI-O-703 (100 mg)
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Capsule
Routes of administration	Oral use

Dosage and administration details:

400 mg BID group: 4 capsules of 100 mg SKI-O-703 + 0 capsule of placebo

Arm title	Placebo
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Arm description:

4 capsules of placebo BID (twice per day)

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

Dosage and administration details:

Placebo group: 4 capsules of placebo (contained only microcrystalline cellulose and magnesium stearate)

Number of subjects in period 1	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo
Started	26	22	12
Completed	22	20	10
Not completed	4	2	2
Subject withdrew consent	-	1	-
Adverse event, non-fatal	2	-	1
Other	1	-	-
Investigator decision	1	1	1

Baseline characteristics

Reporting groups

Reporting group title	SKI-O-703 200 mg BID
Reporting group description: 2 capsules of 100 mg SKI-O-703 BID (twice per day)	
Reporting group title	SKI-O-703 400 mg BID
Reporting group description: 4 capsules of 100 mg SKI-O-703 BID (twice per day)	
Reporting group title	Placebo
Reporting group description: 4 capsules of placebo BID (twice per day)	

Reporting group values	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo
Number of subjects	26	22	12
Age categorical Units: Subjects			
Adults (18-64 years)	18	16	5
From 65-84 years	8	6	5
85 years and over	0	0	2
Age continuous Units: years			
arithmetic mean	56.5	54.3	61.1
standard deviation	± 14.98	± 16.30	± 22.24
Gender categorical Units: Subjects			
Female	13	16	5
Male	13	6	7

Reporting group values	Total		
Number of subjects	60		
Age categorical Units: Subjects			
Adults (18-64 years)	39		
From 65-84 years	19		
85 years and over	2		
Age continuous Units: years			
arithmetic mean	-		
standard deviation	-		
Gender categorical Units: Subjects			
Female	34		
Male	26		

End points

End points reporting groups

Reporting group title	SKI-O-703 200 mg BID
Reporting group description: 2 capsules of 100 mg SKI-O-703 BID (twice per day)	
Reporting group title	SKI-O-703 400 mg BID
Reporting group description: 4 capsules of 100 mg SKI-O-703 BID (twice per day)	
Reporting group title	Placebo
Reporting group description: 4 capsules of placebo BID (twice per day)	

Primary: Platelet count $\geq 30,000/\mu\text{L}$ and doubling the baseline (average of 2 previous counts)

End point title	Platelet count $\geq 30,000/\mu\text{L}$ and doubling the baseline (average of 2 previous counts)
End point description: To evaluate the safety and efficacy on the primary endpoint (platelet response) of selected (200 mg BID and 400 mg BID) doses of SKI-O-703 compared to placebo in subjects with persistent and chronic ITP, with a platelet count $<30,000/\mu\text{L}$ on 2 occasions at least 7 days apart with the confirmatory count being taken during screening	
End point type	Primary
End point timeframe: Up to week 12	

End point values	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	26	22	12	
Units: Count of Participants				
number (not applicable)	12	14	4	

Statistical analyses

Statistical analysis title	Fisher's Exact test
Comparison groups	SKI-O-703 200 mg BID v Placebo
Number of subjects included in analysis	38
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.504
Method	Fisher exact
Parameter estimate	ORR difference
Point estimate	0.13

Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.225
upper limit	0.433

Primary: Platelet count $\geq$ 30,000/ μ L and doubling the baseline (average of 2 previous counts)

End point title	Platelet count $\geq$ 30,000/ μ L and doubling the baseline (average of 2 previous counts)
End point description:	
End point type	Primary
End point timeframe:	
Up to week 12	

End point values	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	26	22	12	
Units: percent				
arithmetic mean (confidence interval 95%)				
ORR=overall platelet response rate	46.2 (26.59 to 66.63)	63.6 (40.66 to 82.80)	33.3 (9.92 to 65.11)	

Statistical analyses

Statistical analysis title	Fisher's Exact test
Comparison groups	SKI-O-703 400 mg BID v Placebo
Number of subjects included in analysis	34
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.151
Method	Fisher exact
Parameter estimate	ORR difference
Point estimate	0.3
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.061
upper limit	0.607

Primary: Platelet Response Rate based on Rescue Medication with 28-day effective window (NRI)

End point title	Platelet Response Rate based on Rescue Medication with 28-day effective window (NRI)
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End point description:

End point type	Primary
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End point timeframe:

Up to week 12

End point values	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	26	22	12	
Units: Count of Participants				
number (not applicable)	12	14	5	

Statistical analyses

Statistical analysis title	Fisher's Exact test
Comparison groups	SKI-O-703 200 mg BID v Placebo
Number of subjects included in analysis	38
Analysis specification	Pre-specified
Analysis type	superiority
P-value	> 0.999
Method	Fisher exact
Parameter estimate	ORR difference
Point estimate	0.04
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.31
upper limit	0.369

Primary: Platelet Response Rate based on Rescue Medication with 28-day effective window (NRI)

End point title	Platelet Response Rate based on Rescue Medication with 28-day effective window (NRI)
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End point description:

End point type	Primary
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End point timeframe:

Up to week 12

End point values	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	26	22	12	
Units: percent				
arithmetic mean (confidence interval 95%)	46.2 (26.59 to 66.63)	63.6 (40.66 to 82.80)	41.7 (15.17 to 72.33)	

Statistical analyses

Statistical analysis title	Fisher's Exact test
Comparison groups	SKI-O-703 400 mg BID v Placebo
Number of subjects included in analysis	34
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.288
Method	Fisher exact
Parameter estimate	ORR difference
Point estimate	0.22
Confidence interval	
level	95 %
sides	2-sided
lower limit	-0.145
upper limit	0.54

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Up to 16 weeks

Adverse event reporting additional description:

Adverse events were assessed and reported from the time the subject signed the ICF until end of study visit (Week 16).

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	21.0
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Reporting groups

Reporting group title	SKI-O-703 200 mg BID
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Reporting group description: -

Reporting group title	SKI-O-703 400 mg BID
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Reporting group description: -

Reporting group title	Placebo
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Reporting group description: -

Serious adverse events	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 26 (0.00%)	2 / 22 (9.09%)	3 / 12 (25.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Investigations			
Neutrophil count decreased			
subjects affected / exposed	0 / 26 (0.00%)	1 / 22 (4.55%)	0 / 12 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Guillain-Barre syndrome			
subjects affected / exposed	0 / 26 (0.00%)	0 / 22 (0.00%)	1 / 12 (8.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Thrombocytopenia			
subjects affected / exposed	0 / 26 (0.00%)	1 / 22 (4.55%)	1 / 12 (8.33%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 26 (0.00%)	0 / 22 (0.00%)	1 / 12 (8.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Coronavirus infection			
subjects affected / exposed	0 / 26 (0.00%)	0 / 22 (0.00%)	1 / 12 (8.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

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

Non-serious adverse events	SKI-O-703 200 mg BID	SKI-O-703 400 mg BID	Placebo
Total subjects affected by non-serious adverse events			
subjects affected / exposed	15 / 26 (57.69%)	17 / 22 (77.27%)	8 / 12 (66.67%)
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	2 / 26 (7.69%)	2 / 22 (9.09%)	0 / 12 (0.00%)
occurrences (all)	2	2	0
Aspartate aminotransferase increased			
subjects affected / exposed	2 / 26 (7.69%)	1 / 22 (4.55%)	0 / 12 (0.00%)
occurrences (all)	2	1	0
Vascular disorders			
Haematoma			
subjects affected / exposed	1 / 26 (3.85%)	1 / 22 (4.55%)	1 / 12 (8.33%)
occurrences (all)	1	1	1
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 26 (3.85%)	0 / 22 (0.00%)	2 / 12 (16.67%)
occurrences (all)	2	0	2
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 26 (0.00%)	2 / 22 (9.09%)	2 / 12 (16.67%)
occurrences (all)	0	3	2
Gastrointestinal disorders			

Gingival bleeding subjects affected / exposed occurrences (all)	0 / 26 (0.00%) 0	3 / 22 (13.64%) 3	1 / 12 (8.33%) 1
Nausea subjects affected / exposed occurrences (all)	1 / 26 (3.85%) 1	2 / 22 (9.09%) 2	0 / 12 (0.00%) 0
Diarrhoea subjects affected / exposed occurrences (all)	0 / 26 (0.00%) 0	2 / 22 (9.09%) 3	2 / 12 (16.67%) 2
Respiratory, thoracic and mediastinal disorders Epistaxis subjects affected / exposed occurrences (all)	1 / 26 (3.85%) 6	4 / 22 (18.18%) 6	0 / 12 (0.00%) 0
Skin and subcutaneous tissue disorders Petechiae subjects affected / exposed occurrences (all)	4 / 26 (15.38%) 4	3 / 22 (13.64%) 3	3 / 12 (25.00%) 5
Infections and infestations Coronavirus infection subjects affected / exposed occurrences (all)	3 / 26 (11.54%) 3	0 / 22 (0.00%) 0	1 / 12 (8.33%) 1
Metabolism and nutrition disorders Hyperglycaemia subjects affected / exposed occurrences (all)	0 / 26 (0.00%) 0	2 / 22 (9.09%) 2	0 / 12 (0.00%) 0

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
03 April 2019	Protocol Amendment 1 Version 2.0, dated 03 Apr 2019
30 April 2019	Protocol Amendment 2 Version 3.0, dated 30 Apr 2019
22 April 2020	Protocol Amendment 3 Version 4.0, dated 22 Apr 2020

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.

None reported

Notes: