Clinical trial results:
Treatment of patients with Lichen planus with the JAK-Inhibitor Upadacitinib (Rinvoq®) – a mono-centered double-blinded placebo controlled randomized pilot study (investigator-initiated trial)

Summary

EudraCT number	2021-006031-25
Trial protocol	DE
Global end of trial date	17 December 2024

Results information

Result version number	v1 (current)
This version publication date	11 December 2025
First version publication date	11 December 2025

Trial information

Trial identification

Sponsor protocol code	UPALI

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Charité - Universitätsmedizin Berlin
Sponsor organisation address	Charitéplatz 1, Berlin, Germany, 10117
Public contact	Studienzentrale, AG Worm, Charité - Universitätsmedizin Berlin, Allergy Center Charité, +49 30 450518305, acc-studien@charite.de
Scientific contact	Studienzentrale, AG Worm, Charité - Universitätsmedizin Berlin, Allergy Center Charité, +49 30 450518305, acc-studien@charite.de

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	06 January 2025
Is this the analysis of the primary completion data?	Yes
Primary completion date	17 December 2024
Global end of trial reached?	Yes
Global end of trial date	17 December 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the efficacy of upadacitinib (Rinvoq®) therapy in patients with Lichen planus.

Protection of trial subjects:

The conduct of this study met all legal and regulatory requirements and in accordance with ethical principles of the Declaration of Helsinki.

Background therapy:

Patients with Lichen planus (LP) show a presence of a strong Th1/Th17 signature. Preliminary data revealed an overactivation of the JAK/STAT Pathway in LP, with hyperactivation of various STAT proteins (STAT1, STAT2, STAT3, STAT4, STAT5). These encouraging results enforce the concept that the use of Janus kinase (JAK) inhibitors in LP could help patients for which present treatments are often not sufficient and help physicians by offering a new possible treatment solution for an often recalcitrant and frustrating disorder.
JAKs are intracellular enzymes that transmit signals from cytokines and growth factors involved in a variety of cellular processes such as inflammatory responses, hematopoiesis, and immune surveillance. The JAK enzyme family comprises four members - JAK1, JAK2, JAK3 and TYK2 - which phosphorylate and thereby activate STATs in pairs. This phosphorylation in turn modulates gene expression and cell function. JAK1 is important for inflammatory cytokine signaling pathways, while JAK2 is important for erythrocyte maturation, and JAK3 signals play a role in immune monitoring and lymphocyte function. Upadacitinib is a selective and reversible JAK inhibitor. In human cell-based assays, upadacitinib preferentially inhibits JAK1 or JAK1/3 signaling pathways compared to other cytokine signaling pathways that are mediated via JAK2 pairs.

Evidence for comparator: -	
Actual start date of recruitment	10 August 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	Germany: 14
Worldwide total number of subjects	14
EEA total number of subjects	14

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37	0

wk	
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)	13
From 65 to 84 years	1
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

The study was conducted at 1 study center in Germany between 10/08/2022 and 17/12/2024.

Pre-assignment

Screening details:

Patients with diagnosis of acute or chronic (>3 months) Lichen planus, with Investigator Global Assessment (IGA) score ≥ 3 and with a clinical presentation and histopathology consistent (performed within 6 months prior to screening) and with at least one target lesion of oral lichen planus were screened.

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	Investigator, Subject, Monitor, Data analyst, Carer

Arms

Are arms mutually exclusive?	Yes
Arm title	Verum

Arm description: -

Arm type	Experimental
Investigational medicinal product name	Upadacitinib
Investigational medicinal product code	L04AA44
Other name	Rinvoq®
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

Patients received 15 mg Upadacitinib per day about 12 weeks

Arm title	Placebo

Arm description: -

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

Patients received placebo per day about 12 weeks

Number of subjects in period 1	Verum	Placebo
Started	8	6
Completed	6	6
Not completed	2	0
Adverse event, non-fatal	1	-
Lost to follow-up	1	-

Baseline characteristics

Reporting groups	
Reporting group title	Verum
Reporting group description: -	
Reporting group title	Placebo
Reporting group description: -	

Reporting group values	Verum	Placebo	Total
Number of subjects	8	6	14
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)	7	6	13
From 65-84 years	1	0	1
85 years and over	0	0	0
Age continuous Units: years			
arithmetic mean	50	51	
standard deviation	± 9	± 10	-
Gender categorical Units: Subjects			
Female	6	2	8
Male	2	4	6
IGA			
Investigator Global Assessment (IGA) for atopic dermatitis.			
Units: Subjects			
IGA severe	4	1	5
IGA moderate	4	5	9
DLQI			
Dermatology Life Quality Index (DLQI) is a ten-question questionnaire used to measure the impact of skin disease on the quality of life of an affected person.			
Units: score			
arithmetic mean	8.5	4	
standard deviation	± 6.19	± 1.26	-
Itch NRS			
Itch NRS (Numerical Rating Scale) is a single item designed to capture information on self-reported severity of worst itching each day.			
Units: Score			
arithmetic mean	3.38	1.33	
standard deviation	± 4.0	± 3.27	-
Pain VAS			
Pain on Visual Analogue Scale (VAS)			

Units: Score			
arithmetic mean	35.57	35.0	
standard deviation	± 16.73	± 19.28	-
PSA			
Participant self-assessment			
Units: score			
median	4	3.5	
inter-quartile range (Q1-Q3)	3 to 4	1.75 to 5	-
PSAD			
Medical assessment of the disease surface.			
Units: score			
median	3.5	4	
inter-quartile range (Q1-Q3)	3 to 4.75	3 to 4.25	-

End points

End points reporting groups

Reporting group title	Verum
Reporting group description: -	
Reporting group title	Placebo
Reporting group description: -	

Primary: clinical response (IGA) in mucosal disease

End point title	clinical response (IGA) in mucosal disease[1]
End point description:	
End point type	Primary
End point timeframe:	
at week 12	

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: Due to the small number of patients, a statistical evaluation was only planned descriptively.

End point values	Verum	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: subjects				
IGA ≤ 1	3	0		
IGA >1	3	6		

Statistical analyses

No statistical analyses for this end point

Secondary: Change in DLQI

End point title	Change in DLQI
End point description:	
Change in Dermatology Life Quality Index (DLQI), evaluation DLQI: week 4, 8, and 12.	
End point type	Secondary
End point timeframe:	
from baseline up to 12 weeks	

End point values	Verum	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: score				
arithmetic mean (standard deviation)				
week 4	7.5 (± 5.58)	2.83 (± 2.32)		
week 8	5.17 (± 4.07)	4.5 (± 2.89)		
week 12	4.67 (± 3.89)	5.0 (± 3.29)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change in Itch

End point title	Change in Itch
End point description:	
Change in patient assessment of pruritus (itching) (NRS 1-10), evaluation: week 4,8,12	
End point type	Secondary
End point timeframe:	
from baseline up to 12 weeks	

End point values	Verum	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: score				
arithmetic mean (standard deviation)				
week 4	2.17 (± 3.06)	2.6 (± 3.71)		
week 8	1.33 (± 1.63)	2.5 (± 3.89)		
week 12	0.83 (± 0.98)	3.0 (± 4.69)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change in pain VAS

End point title	Change in pain VAS
End point description:	
Change in patient assessment of pain (VAS 0-100), evaluation: week 4, 8, 12	
End point type	Secondary
End point timeframe:	
from baseline up to 12 weeks	

End point values	Verum	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: score				
arithmetic mean (standard deviation)				
week 4	38 (± 28.94)	44.6 (± 24.52)		
week 8	28.2 (± 19.0)	44.67 (± 28.05)		
week 12	25 (± 14.36)	33.4 (± 20.86)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change in PSA

End point title	Change in PSA

End point description:

Change in Participant Self-Assessment (PSA), evaluation: week 4,8,12

End point type	Secondary

End point timeframe:

from baseline up to 12 weeks

End point values	Verum	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: Score				
median (inter-quartile range (Q1-Q3))				
week 4	4 (3.5 to 4.5)	4 (3 to 5)		
week 8	3.5 (1.75 to 4.25)	3 (1.75 to 4.25)		
week 12	2.5 (1 to 3.25)	3.5 (1.75 to 4.25)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change in PSAD

End point title	Change in PSAD

End point description:

Change in Physician Assessment of Surface Area of Disease (PSAD), evaluation: week 4,8,12

End point type	Secondary

End point timeframe:

from baseline up to 12 weeks

End point values	Verum	Placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: score				
median (inter-quartile range (Q1-Q3))				
week 4	3 (2.75 to 4.25)	4 (3 to 4.25)		
week 8	2.5 (1.5 to 4)	4 (2.75 to 4)		
week 12	2 (1.5 to 3.25)	3.5 (3 to 4.25)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: from baseline up to 12 weeks	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	24
Reporting groups	
Reporting group title	Verum
Reporting group description: -	
Reporting group title	Placebo
Reporting group description: -	

Serious adverse events	Verum	Placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 8 (0.00%)	0 / 6 (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: 1 %

Non-serious adverse events	Verum	Placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	8 / 8 (100.00%)	5 / 6 (83.33%)	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
epithelial dysplasia			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
General disorders and administration site conditions			
Flu like symptoms			
subjects affected / exposed	3 / 8 (37.50%)	3 / 6 (50.00%)	
occurrences (all)	4	4	
local skin reaction			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	

loose teeth			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Gastrointestinal disorders			
Gastritis			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Gastroesophageal reflux disease			
subjects affected / exposed	2 / 8 (25.00%)	0 / 6 (0.00%)	
occurrences (all)	2	0	
Cheilitis			
subjects affected / exposed	0 / 8 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Reproductive system and breast disorders			
Amenorrhea			
subjects affected / exposed	0 / 8 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Respiratory, thoracic and mediastinal disorders			
Sore throat			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Rash acneiform			
subjects affected / exposed	0 / 8 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Musculoskeletal and connective tissue disorders			
back pain			
subjects affected / exposed	1 / 8 (12.50%)	1 / 6 (16.67%)	
occurrences (all)	1	1	
Myalgia			
subjects affected / exposed	0 / 8 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Arthritis	Additional description: gout symptoms		

subjects affected / exposed	0 / 8 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Infections and infestations			
Herpes simplex reactivation			
subjects affected / exposed	0 / 8 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Covid 19	Additional description: SARS-CoV-2 test positive		
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
fungal infection			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
soft tissue infection			
subjects affected / exposed	1 / 8 (12.50%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Urinary tract infection			
subjects affected / exposed	1 / 8 (12.50%)	2 / 6 (33.33%)	
occurrences (all)	1	2	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported