Clinical trial results:
A Randomized, Double-Blind, Placebo-Controlled, Multicenter, Phase 2 Study to Evaluate the Efficacy, Safety, and Pharmacokinetics of Rozanolixizumab in Adult Study Participants With Leucine-Rich Glioma Inactivated 1 Autoimmune Encephalitis

Summary

EudraCT number	2019-004778-25
Trial protocol	DE FR NL IT PT
Global end of trial date	26 April 2024

Results information

Result version number	v1
This version publication date	23 March 2025
First version publication date	23 March 2025

Trial information

Trial identification

Sponsor protocol code	AIE001

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04875975
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	UCB Biopharma SRL
Sponsor organisation address	Allée de la Recherche 60, Brussels, Belgium, 1070
Public contact	Clin Trial Reg & Results Disclosure, UCB BIOSCIENCES GmbH, clinicaltrials@ucb.com
Scientific contact	Clin Trial Reg & Results Disclosure, UCB BIOSCIENCES GmbH, clinicaltrials@ucb.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	29 May 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	08 March 2024
Global end of trial reached?	Yes
Global end of trial date	26 April 2024
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

Assess the efficacy of rozanolixizumab as measured by seizure freedom

Protection of trial subjects:

During the conduct of the study all participants were closely monitored.

Background therapy:

Corticosteroids was initiated prior to the start of the Screening Period, however, the study participant randomized within 42 days of corticosteroids initiation. As individual disease entities, IgG autoantibody-mediated conditions are relatively rare. Treatment of these disorders remains a difficult clinical problem, requiring in many of these conditions the long-term use of corticosteroids alone or combined with other immunomodulatory therapy.

Evidence for comparator:

Not applicable

Actual start date of recruitment	27 September 2021
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Belgium: 1
Country: Number of subjects enrolled	France: 3
Country: Number of subjects enrolled	Germany: 1
Country: Number of subjects enrolled	Italy: 1
Country: Number of subjects enrolled	Netherlands: 3
Country: Number of subjects enrolled	Spain: 1
Country: Number of subjects enrolled	United Kingdom: 2
Worldwide total number of subjects	12
EEA total number of subjects	10

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)	4
From 65 to 84 years	8
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

The study started to enroll participants in September 2021 and concluded in April 2024.

Pre-assignment

Screening details:

The Participant Flow refers to the Randomized Set (RS).

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Placebo

Arm description:

Participants received placebo as a subcutaneous (sc) infusion once a week (Q1W) for 25 weeks.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received placebo at pre-specified timepoints.

Arm title	Rozanolixizumab (RLZ)

Arm description:

Participants received rozanolixizumab as a sc infusion Q1W for 25 weeks.

Arm type	Experimental
Investigational medicinal product name	Rozanolixizumab
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received rozanolixizumab at pre-specified timepoints.

Number of subjects in period 1	Placebo	Rozanolixizumab (RLZ)
Started	6	6
Completed	2	0
Not completed	4	6
Permanently left due to new seizure recurrence	-	1

Adverse event, non-fatal	1	2
Relapse With Insults	-	1
Permanently left due to seizure recurrence	1	-
Lack of efficacy	2	2

Baseline characteristics

Reporting groups	
Reporting group title	Placebo
Reporting group description:	
Participants received placebo as a subcutaneous (sc) infusion once a week (Q1W) for 25 weeks.	
Reporting group title	Rozanolixizumab (RLZ)
Reporting group description:	
Participants received rozanolixizumab as a sc infusion Q1W for 25 weeks.	

Reporting group values	Placebo	Rozanolixizumab (RLZ)	Total
Number of subjects	6	6	12
Age Categorical Units: participants			
18 - <65 years	3	1	4
65 - <85 years	3	5	8
>=85 years	0	0	0
Age Continuous Units: Years			
arithmetic mean	60.7	70.7	
standard deviation	± 12.4	± 11.4	-
Sex: Female, Male Units: Participants			
Female	2	2	4
Male	4	4	8

End points

End points reporting groups

Reporting group title	Placebo
Reporting group description:	
Participants received placebo as a subcutaneous (sc) infusion once a week (Q1W) for 25 weeks.	
Reporting group title	Rozanolixizumab (RLZ)
Reporting group description:	
Participants received rozanolixizumab as a sc infusion Q1W for 25 weeks.	

Primary: Number of seizure free study participants at the end of the Treatment

End point title	Number of seizure free study participants at the end of the Treatment[1]
End point description:	
Seizure freedom was defined as a minimum of 28 consecutive days of no seizures of any type during the treatment and maintained until the end of the treatment (Week 25). The Randomized Set (RS) consisted of all enrolled study participants who were randomized to treatment arms.	
End point type	Primary
End point timeframe:	
From Baseline until the end of the Treatment (Week 25)	

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: Formal statistical hypothesis testing was planned for primary endpoint in this study. However, analysis was not feasible due to insufficient number of participants. Results were summarized as descriptive statistics only.

End point values	Placebo	Rozanolixizumab (RLZ)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: participants	1	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Repeatable Battery for the Assessment of Neuropsychological Status (RBANS) total scale index score at the end of the Treatment

End point title	Change from Baseline in Repeatable Battery for the Assessment of Neuropsychological Status (RBANS) total scale index score at the end of the Treatment

End point description:

The RBANS consists of 12 subtests that contribute to 5 age-based domain index scores (immediate memory, visuospatial/constructional, language, attention, delayed memory) that were aggregated for total scale index score. All index scores have age-based mean of 100, with standard deviation (SD) of 15. Total scale score was calculated by taking mean of sum of the five index scores. Total possible scale index scores range from 40-135. Higher scores reflect better neurocognitive performance. Total scale index score is the score typically used to reflect global neurocognitive status. Baseline (BL) of RBANS is defined as the screening (Visit 1, Week -1) value. 99999: Mean and standard deviation (SD) were not

calculated due to less number of participants. Randomized Set (RS). Number of participants analyzed = participants who were evaluable for this endpoint. n = participants who were evaluable at specified time points.

End point type	Secondary

End point timeframe:

From Baseline to Week 5, 13, 21 and 25

End point values	Placebo	Rozanolixizumab (RLZ)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	4	6		
Units: score on a scale				
arithmetic mean (standard deviation)				
Week 5 (n= 4, 6)	4.5 (± 2.6)	2.2 (± 11.3)		
Week 13 (n= 4, 4)	7.0 (± 2.9)	12.3 (± 7.9)		
Week 21 (n= 4, 2)	7.0 (± 7.3)	99999 (± 99999)		
Week 25 (n= 2, 1)	99999 (± 99999)	99999 (± 99999)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants with a favorable outcome in the Modified Rankin Scale (mRS) during the Treatment

End point title	Percentage of participants with a favorable outcome in the Modified Rankin Scale (mRS) during the Treatment

End point description:

Percentage of participants with favorable outcome in mRS, defined as no worsening for participants with BL mRS score of ≤1 or improvement of ≥1 point with BL mRS score of ≥2. mRS: scale for measuring degree of disability or dependence in daily activities of people who suffered stroke or other causes of neurological disability. Scale ranges from 0 (perfect health) to 6 (death). 0-No symptoms at all 1-No significant disability despite symptoms; able to carry out all usual activities 2-Slight disability; unable to carry out all previous activities, but able to look after own affairs without assistance 3-Moderate disability; requiring some help, but able to walk without assistance 4-Moderately severe disability; unable to walk andattend to own bodily needs without assistance 5-Severe disability; bedridden, requiring nursing care 6-Dead 99999: Due to early termination of study, insufficient data were available to perform statistical analyses as described in protocol. Analysis set: RS.

End point type	Secondary

End point timeframe:

From Baseline until the end of the Treatment (Week 25)

End point values	Placebo	Rozanolixizumab (RLZ)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: percentage of participants				
number (not applicable)	99999	99999		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of participants who required rescue medication due to an absence or loss of clinical benefit during the Treatment

End point title	Number of participants who required rescue medication due to an absence or loss of clinical benefit during the Treatment

End point description:

Study participants who required rescue medication due to an absence or loss of clinical benefit were discontinued blinded treatment and completed the assessments for the early discontinuation visit. The Randomized Set (RS) consisted of all enrolled study participants who were randomized to treatment arms.

End point type	Secondary

End point timeframe:

From Baseline until the end of the Treatment (Week 25)

End point values	Placebo	Rozanolixizumab (RLZ)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: participants	3	2		

Statistical analyses

No statistical analyses for this end point

Secondary: Time to first occurrence of seizure freedom during the Treatment

End point title	Time to first occurrence of seizure freedom during the Treatment

End point description:

The time to first occurrence of seizure freedom was defined by the number of days after randomization to the first day of the first 28 consecutive days without seizures during the treatment. Time to first occurrence of 28 consecutive days of seizure freedom (days) during the treatment was calculated as date of first day of occurrence of 28 consecutive days of seizure freedom – Date of Randomization + 1. The Randomized Set (RS) consisted of all enrolled study participants who were randomized to treatment arms.

End point type	Secondary

End point timeframe:

From Baseline until the end of the Treatment (Week 25)

End point values	Placebo	Rozanolixizumab (RLZ)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: Weeks				
median (full range (min-max))	0.1 (0.1 to 8.4)	0.1 (0.1 to 8.3)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants with Treatment-Emergent Adverse Events (TEAEs)

End point title	Percentage of Participants with Treatment-Emergent Adverse Events (TEAEs)

End point description:

An adverse event (AE) is any untoward medical occurrence in a patient or clinical study participant, temporally associated with the use of investigational medicinal product (IMP), whether or not considered related to the IMP. An AE could therefore be any unfavorable and unintended sign (including an abnormal laboratory finding), symptom, or disease (new or exacerbated) temporally associated with the use of IMP, whether or not related to the IMP. A TEAE was defined as an AE starting on or after the time of first administration of IMP or any unresolved event already present before the first administration of IMP that worsens in intensity following exposure to treatment up to the end of the Treatment (EOT) and including the 8-week (56 days) Safety-Follow Up (SFU). The Safety Set (SS) consisted of all randomized study participants who received at least one dose of IMP.

End point type	Secondary

End point timeframe:

From Baseline until the End of Study (Week 32)

End point values	Placebo	Rozanolixizumab (RLZ)		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	6	6		
Units: percentage of participants				
number (not applicable)	100	100		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From Baseline until the End of Study (up to Week 32)

Adverse event reporting additional description:

TEAE: AE starting on or after time of first administration of IMP or any unresolved event already present before first administration of IMP that worsens in intensity following exposure to treatment up to EOT and including 8-week (56 days) SFU. The Safety Set consisted of all randomized study participants who received at least one dose of IMP.

Assessment type	Non-systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	24.1

Reporting groups

Reporting group title	Rozanolixizumab (RLZ)

Reporting group description:

Participants received rozanolixizumab as a sc infusion Q1W for 25 weeks.

Reporting group title	Placebo

Reporting group description:

Participants received placebo as a subcutaneous (sc) infusion once a week (Q1W) for 25 weeks.

Serious adverse events	Rozanolixizumab (RLZ)	Placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	3 / 6 (50.00%)	3 / 6 (50.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Vascular disorders			
Deep vein thrombosis			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Peripheral sensory neuropathy			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Encephalitis autoimmune			

subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Psychiatric disorders			
Mania			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Suicidal ideation			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
COVID-19			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Psoas abscess			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Bacteraemia			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (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	Rozanolixizumab (RLZ)	Placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	6 / 6 (100.00%)	6 / 6 (100.00%)	
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Benign ear neoplasm			

subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Paraproteinaemia			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Vascular disorders			
Hypotension			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	2	0	
Fatigue			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Influenza like illness			
subjects affected / exposed	0 / 6 (0.00%)	2 / 6 (33.33%)	
occurrences (all)	0	2	
Swelling face			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Infusion site erythema			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Oedema peripheral			
subjects affected / exposed	4 / 6 (66.67%)	0 / 6 (0.00%)	
occurrences (all)	4	0	
Respiratory, thoracic and mediastinal disorders			
Dyspnoea			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Cough			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Lung opacity			

subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Oropharyngeal pain			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	2	
Rhinorrhoea			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Psychiatric disorders			
Affect lability			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	3	0	
Anxiety			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	2	0	
Insomnia			
subjects affected / exposed	2 / 6 (33.33%)	1 / 6 (16.67%)	
occurrences (all)	3	1	
Emotional disorder			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	2	
Impulse-control disorder			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Mania			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	2	0	
Sleep disorder			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Investigations			
Alanine aminotransferase increased			
subjects affected / exposed	2 / 6 (33.33%)	0 / 6 (0.00%)	
occurrences (all)	2	0	
Blood immunoglobulin M increased			

subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Aspartate aminotransferase increased			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Gamma-glutamyltransferase increased			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Blood alkaline phosphatase increased			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Injury, poisoning and procedural complications			
Fall			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Skin injury			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Spinal compression fracture			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Nervous system disorders			
Ataxia			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Balance disorder			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Depressed level of consciousness			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Encephalitis autoimmune			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Headache			

subjects affected / exposed	1 / 6 (16.67%)	1 / 6 (16.67%)	
occurrences (all)	1	4	
Parkinsonism			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Peripheral sensory neuropathy			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Seizure			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Ageusia			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Dysgeusia			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Blood and lymphatic system disorders			
Lymphopenia			
subjects affected / exposed	1 / 6 (16.67%)	1 / 6 (16.67%)	
occurrences (all)	1	2	
Eye disorders			
Vision blurred			
subjects affected / exposed	1 / 6 (16.67%)	1 / 6 (16.67%)	
occurrences (all)	1	1	
Lacrimation increased			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Gastrointestinal disorders			
Constipation			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Gingival pain			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Vomiting			

subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Dry mouth			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Abdominal pain upper			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Abdominal pain			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Diarrhoea			
subjects affected / exposed	3 / 6 (50.00%)	1 / 6 (16.67%)	
occurrences (all)	7	3	
Skin and subcutaneous tissue disorders			
Hyperhidrosis			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Eczema			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Pruritus			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Rash maculo-papular			
subjects affected / exposed	0 / 6 (0.00%)	2 / 6 (33.33%)	
occurrences (all)	0	2	
Musculoskeletal and connective tissue disorders			
Bursitis			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Periarthritis			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Muscle spasms			

subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Musculoskeletal stiffness			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Back pain			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Infections and infestations			
Anal abscess			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Cellulitis			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
COVID-19			
subjects affected / exposed	1 / 6 (16.67%)	1 / 6 (16.67%)	
occurrences (all)	1	1	
Suspected COVID-19			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Cytomegalovirus infection			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Tooth infection			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Otitis externa			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	
Lower respiratory tract infection			
subjects affected / exposed	0 / 6 (0.00%)	1 / 6 (16.67%)	
occurrences (all)	0	1	
Cystitis			
subjects affected / exposed	1 / 6 (16.67%)	0 / 6 (0.00%)	
occurrences (all)	1	0	

Urinary tract infection subjects affected / exposed occurrences (all)	0 / 6 (0.00%) 0	1 / 6 (16.67%) 1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
04 December 2020	Protocol Amendment 1 was dated 04 Dec 2020: Changes to the protocol were made to change the dose of rozanolixizumab from weight-tiered doses to fixed dose. Other corrections to ensure consistency across the rozanolixizumab clinical development program were also made. Minor grammatical, editorial, and formatting changes were also made for clarification purposes only.
07 September 2021	Protocol Amendment 2 was dated 07 Sep 2021: Changes to the protocol were made to amend the inclusion critera to ensure greater specificity for the target population, the addition of regular monitoring of lipid parameters, and to provide clarity on how the treatment blind maintained. This amendment also introduced blood sampling for antibody response to COVID-19 vaccination, and provided clarity on the management of study participants undergoing COVID-19 vaccination. Requests from regulatory authorities or ethics committees were incorporated, and country-specific revisions added. Minor grammatical, editorial, and formatting changes were also made for clarification purposes only.
14 January 2022	Protocol Amendment 3 was dated 14 Jan 2022: The overall rationale for the amendment was to address comments and queries from Food and Drug Administration (FDA) raised during the Investigational New Drug (IND) review. Minor grammatical, editorial, and formatting changes were also made for clarification purposes only.
19 October 2022	Protocol Amendment 4 was dated 19 Oct 2022: Changes to the protocol were made to update safety information in line with the revised IB dated 06 September 2022. Additional changes were also made to reduce the study burden for patients. Minor grammatical, editorial, and formatting changes were also made for clarification purposes only.
09 February 2023	Protocol Amendment 5 was dated 09 Feb 2023: In the past 3 years (since the initial protocol), there was an emerging understanding about the experiences of patients with Leucine-rich glioma inactivated 1 AIE (LGI1 AIE), changed in medical management of such patients, and more widespread use of the clinical outcome assessments supporting efficacy endpoints selected for this study. Therefore, changes to the protocol were made to update the secondary endpoints and associated statistical methods and better align the protocol with the current treatment practices and understanding of the disease. Minor grammatical, editorial, and formatting changes were also made for clarification purposes only.
18 July 2023	Protocol Amendment 6 was dated 18 Jul 2023: The overall rationale for the amendment was to address a request from FDA to re-introduce certain safety assessments to support the safety profile of rozanolixizumab in a new population. Since Amendment 5, there was no change in the benefit/risk profile of rozanolixizumab. Minor grammatical, editorial, and formatting changes were also made for clarification purposes only.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? Yes

Date	Interruption	Restart date

29 November 2023	UCB had determined that it is no longer feasible to complete the study within the targeted timelines and as a result made the decision to prematurely terminate the AIE001 study. This decision does not relate to any concern about the safety, tolerability, or efficacy of rozanolixizumab and the established benefit-risk profile remains unchanged.	-

Notes:

Limitations and caveats

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

Due to the early termination of the recruitment and insufficient participants, it was not feasible to carry out the statistical analyses as originally planned.

Notes: