Clinical trial results:
An Open-Label Extension Study to Evaluate Rozanolixizumab in Study Participants with Generalized Myasthenia Gravis

Summary

EudraCT number	2020-003230-20
Trial protocol	GB DE CZ FR PL HU DK BE IT
Global end of trial date	25 January 2024

Results information

Result version number	v3 (current)
This version publication date	03 May 2025
First version publication date	07 February 2025
Version creation reason	

Trial information

Trial identification

Sponsor protocol code	MG0007

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04650854
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	12 April 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	25 January 2024
Global end of trial reached?	Yes
Global end of trial date	25 January 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Assess the safety and tolerability of additional 6-week treatment cycles with rozanolixizumab in study participants with generalized myasthenia gravis (gMG)

Protection of trial subjects:

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

Background therapy:

Background therapy as permitted in the protocol.

Evidence for comparator:

Not applicable

Actual start date of recruitment	03 February 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	Canada: 19
Country: Number of subjects enrolled	Czechia: 2
Country: Number of subjects enrolled	Denmark: 5
Country: Number of subjects enrolled	France: 13
Country: Number of subjects enrolled	Georgia: 13
Country: Number of subjects enrolled	Germany: 7
Country: Number of subjects enrolled	Italy: 11
Country: Number of subjects enrolled	Japan: 12
Country: Number of subjects enrolled	Poland: 26
Country: Number of subjects enrolled	Russian Federation: 12
Country: Number of subjects enrolled	Serbia: 5
Country: Number of subjects enrolled	Taiwan: 3
Country: Number of subjects enrolled	United States: 32
Country: Number of subjects enrolled	Spain: 5
Worldwide total number of subjects	165
EEA total number of subjects	69

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)	123
From 65 to 84 years	40
85 years and over	2

Subject disposition

Recruitment

Recruitment details:

Study started to enroll participants in February 2021 and concluded in January 2024. Participant Flow refers to the Full Analysis Set (FAS).

Pre-assignment

Screening details:

Participants who completed MG0003 (NCT03971422) or required rescue therapy during observation period (OP) in MG0003 (except who received intravenous immunoglobulin/plasma exchange) or completed at least 6 treatment visits in MG0004 (NCT04124965) were enrolled in study.

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	Enrolled, but not Treated

Arm description:

Participants were enrolled but did not receive a treatment cycle of Rozanolixizumab (RLZ) in MG0007 per the Investigator’s discretion.

Arm type	No intervention

No investigational medicinal product assigned in this arm

Arm title	Rozanolixizumab ~7 mg/kg

Arm description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of subcutaneous (sc) dose of Rozanolixizumab (RLZ) equivalent to approximately 7 milligrams/kilogram (mg/kg) once weekly (QW), followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, premature end of treatment (PEOT) Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator’s discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator’s discretion.

Arm type	Experimental
Investigational medicinal product name	Rozanolixizumab
Investigational medicinal product code	UCB7665
Other name	RLZ
Pharmaceutical forms	Infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received rozanolixizumab at pre-specified time-points.

Arm title	Rozanolixizumab ~10 mg/kg

Arm description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of sc dose of RLZ equivalent to approximately 10 mg/kg QW, followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, PEOT Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator’s discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week

treatment cycle followed by OP, based on Investigator’s discretion.

Arm type	Experimental
Investigational medicinal product name	Rozanolixizumab
Investigational medicinal product code	UCB7665
Other name	RLZ
Pharmaceutical forms	Infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received rozanolixizumab at pre-specified time-points.

Number of subjects in period 1	Enrolled, but not Treated	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg
Started	8	80	77
Completed	3	15	16
Not completed	5	65	61
End Of Study	-	2	1
Enrollment Cup	-	-	1
Discontinued Due to Chronic Heart Failure	-	-	1
Withdraw Per Sponsor's Directive	-	1	-
Participant Withdraw After Drug Approved By FDA	-	-	1
Rescue Medication	-	-	1
Adverse event, non-fatal	1	12	14
Subject Performed PEOT, Not a Completed Subject	-	1	-
Last Treatment Per Sponsor	-	-	2
Last Treatment Per Sponsor Lplv Timelines- Japan	-	3	3
Withdrawal Due to Prohibited Treatment	-	-	1
Withdraw Based on Sponsor Decision	-	-	1
Participant Screened For MG0020	-	2	3
Use of Rescue Medication	-	-	1
Participant to Be Screened for MG0020 Study	-	4	5
Patient Withdrawn; Lack of IMP Shipments	-	2	1
Wish To Continue Study IMP in Patient Programme	-	1	-
Participant Received Rescue Medication	-	1	-
Participant Want to Start a Family	-	-	1
Consent withdrawn by subject	2	17	9
Last Treatment Per Lplv, Screened For MG0020	-	2	2
Last Treatment Per Sponsor Lplv Timelines	-	12	6

Pi Withdrew the Patient From the Study	-	-	1
Lost to follow-up	1	1	-
Patient Withdraw Due To Inappropriate Behavior	-	1	-
Lack of efficacy	1	3	6

Baseline characteristics

Reporting groups	
Reporting group title	Enrolled, but not Treated
Reporting group description:	
Participants were enrolled but did not receive a treatment cycle of Rozanolixizumab (RLZ) in MG0007 per the Investigator's discretion.	
Reporting group title	Rozanolixizumab ~7 mg/kg
Reporting group description:	
Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of subcutaneous (sc) dose of Rozanolixizumab (RLZ) equivalent to approximately 7 milligrams/kilogram (mg/kg) once weekly (QW), followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, premature end of treatment (PEOT) Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator's discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator's discretion.	
Reporting group title	Rozanolixizumab ~10 mg/kg
Reporting group description:	
Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of sc dose of RLZ equivalent to approximately 10 mg/kg QW, followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, PEOT Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator's discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator's discretion.	

Reporting group values	Enrolled, but not Treated	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg
Number of subjects	8	80	77
Age Categorical Units: participants			
18 to <65 years	5	61	57
65 to <85 years	2	19	19
>=85 years	1	0	1
Age Continuous Units: years			
arithmetic mean	59.6	52.5	52.2
standard deviation	± 17.6	± 15.7	± 17.0
Sex/Gender, Customized Units: participants			
Female	3	45	48
Male	5	35	29

Reporting group values	Total		
Number of subjects	165		
Age Categorical Units: participants			
18 to <65 years	123		
65 to <85 years	40		

>=85 years	2		

Age Continuous Units: years arithmetic mean standard deviation	- -		
Sex/Gender, Customized Units: participants			
Female	96		
Male	69		

End points

End points reporting groups

Reporting group title	Enrolled, but not Treated

Reporting group description:

Participants were enrolled but did not receive a treatment cycle of Rozanolixizumab (RLZ) in MG0007 per the Investigator's discretion.

Reporting group title	Rozanolixizumab ~7 mg/kg

Reporting group description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of subcutaneous (sc) dose of Rozanolixizumab (RLZ) equivalent to approximately 7 milligrams/kilogram (mg/kg) once weekly (QW), followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, premature end of treatment (PEOT) Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator's discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator's discretion.

Reporting group title	Rozanolixizumab ~10 mg/kg

Reporting group description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of sc dose of RLZ equivalent to approximately 10 mg/kg QW, followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, PEOT Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator's discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator's discretion.

Subject analysis set title	Rozanolixizumab ~7 mg/kg
Subject analysis set type	Safety analysis

Subject analysis set description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of subcutaneous (sc) dose of Rozanolixizumab (RLZ) equivalent to approximately 7 milligrams/kilogram (mg/kg) once weekly (QW), followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, premature end of treatment (PEOT) Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator's discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator's discretion.

Subject analysis set title	Rozanolixizumab ~10 mg/kg
Subject analysis set type	Safety analysis

Subject analysis set description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of sc dose of RLZ equivalent to approximately 10 mg/kg QW, followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, PEOT Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator's discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator's discretion.

Primary: Percentage of participants with treatment-emergent adverse events (TEAEs)

End point title	Percentage of participants with treatment-emergent adverse events (TEAEs)[1]

End point description:

An adverse event (AE) was any untoward medical occurrence in a patient or clinical investigation participant administered a pharmaceutical product, which did not necessarily had a causal relationship with this treatment. An AE could therefore be any unfavorable and unintended sign, symptom, or disease temporally associated with the use of a medicinal (investigational) product, whether or not related to the medicinal (investigational) product. TEAE was defined as any event that was not present prior to first dose of investigational medicinal product (IMP), or any unresolved event already present that worsened in intensity following treatment, up to 8 weeks after end of Treatment Period or after last dose of IMP in participants who discontinued study or IMP. Safety Set (SS): All study participants in the FAS who received at least one dose of IMP. Participants who switched doses were counted in both Rozanolixizumab (RLZ) doses.

End point type	Primary

End point timeframe:

From Baseline (Day 1) to End of Study (up to 34 months)

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: No formal statistical hypothesis testing was planned for this study. Results were summarized as descriptive statistics only.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	102	102		
Units: percentage of participants				
number (not applicable)	78.4	94.1		

Statistical analyses

No statistical analyses for this end point

Primary: Percentage of participants with TEAEs leading to withdrawal of investigational medicinal product (IMP)

End point title	Percentage of participants with TEAEs leading to withdrawal of investigational medicinal product (IMP)[2]

End point description:

An adverse event (AE) was any untoward medical occurrence in a patient or clinical investigation participant administered a pharmaceutical product, which does not necessarily have a causal relationship with this treatment. AEs leading to permanent withdrawal of study medication. Safety Set (SS): All study participants in the FAS who received at least one dose of IMP. Participants who switched doses were counted in both RLZ doses.

End point type	Primary

End point timeframe:

From Baseline (Day 1) to End of Study (up to 34 months)

Notes:

[2] - 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: No formal statistical hypothesis testing was planned for this study. Results were summarized as descriptive statistics only.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	102	102		
Units: percentage of participants				
number (not applicable)	9.8	16.7		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline (Day 1) to Day 43 in Myasthenia Gravis-Activities of Daily Living (MG-ADL) score within one treatment cycle (Cycle 1, 2, and 3)

End point title	Change from Baseline (Day 1) to Day 43 in Myasthenia Gravis-Activities of Daily Living (MG-ADL) score within one treatment cycle (Cycle 1, 2, and 3)[3]

End point description:

The MG-ADL is an 8-item PRO instrument developed on basis of QMG. The MG-ADL targeted symptoms and disability across ocular, bulbar, respiratory, and axial symptoms. The total MG-ADL score was obtained by summing responses to each individual item (8 items; Grades: 0, 1, 2, 3), where 0= no symptoms or impaired performance and 3= most severe symptoms or impaired performance. The total score ranges from 0 to 24, with higher score indicating more disability. A positive change in score indicates worsening and negative change indicates improvement. For analyses done by study cycle, Baseline values were last available values prior to or on the same date of first administration of IMP at each cycle (Baseline [Day 1]) value for that cycle. Safety Set (SS): All study participants in FAS who received at least one dose of IMP. Number of Participants Analyzed included those participants who were evaluable for assessment. 'n' included those participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[3] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74	66		
Units: score on a scale				
arithmetic mean (standard deviation)				
Cycle 1: Treatment (Day 43) (n=74,66)	-3.7 (± 3.5)	-3.1 (± 2.9)		
Cycle 2: Treatment (Day 43) (n=56,65)	-2.9 (± 3.1)	-3.8 (± 3.9)		
Cycle 3: Treatment (Day 43) (n=41,58)	-3.3 (± 2.7)	-3.2 (± 3.3)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline (Day 1) to Day 43 in Quantitative Myasthenia

Gravis (QMG) score within one treatment cycle (Cycle 1, 2, and 3)

End point title	Change from Baseline (Day 1) to Day 43 in Quantitative Myasthenia Gravis (QMG) score within one treatment cycle (Cycle 1, 2, and 3)[4]

End point description:

The QMG is a validated assessment and the scale tested 13 items, including ocular and facial involvement, swallowing, speech, limb strength, and forced vital capacity. The total QMG score was obtained by summing the responses to each individual item (13 items; Responses: None=0, Mild=1, Moderate=2, Severe=3) and the score ranges from 0 to 39, with lower scores indicating lower disease activity. A positive change in the score indicates worsening and a negative change indicates improvement. Safety Set (SS): All study participants in the FAS who received at least one dose of IMP. Number of Participants Analyzed included those participants who were evaluable for the assessment. 'n' included those participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[4] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	73	64		
Units: score on a scale				
arithmetic mean (standard deviation)				
Cycle 1: Treatment (Day 43) (n=73,64)	-4.5 (± 5.0)	-4.1 (± 4.2)		
Cycle 2: Treatment (Day 43) (n=55,64)	-3.9 (± 4.1)	-4.9 (± 5.5)		
Cycle 3: Treatment (Day 43) (n=41,57)	-5.1 (± 4.8)	-4.2 (± 4.4)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline (Day 1) to Day 43 in Myasthenia Gravis-Composite (MG-C) score within one treatment cycle (Cycle 1, 2, and 3)

End point title	Change from Baseline (Day 1) to Day 43 in Myasthenia Gravis-Composite (MG-C) score within one treatment cycle (Cycle 1, 2, and 3)[5]

End point description:

MG-C scale consists of 10 items: ptosis (0 to 3), double vision on lateral gaze left/right/both (0 to 4), eye closure (0 to 2), talking (0 to 6), chewing (0 to 6), swallowing (0 to 6), breathing (0 to 9), neck flexion or extension (0 to 4), shoulder abduction (0 to 5) and hip flexion (0 to 5), lower scores indicates lower disease activity. Total MG-C score obtained by summing responses to each individual item and score ranges from 0 - 50, (lower scores= lower disease activity). A positive change indicates worsening and a negative change improvement. Safety Set (SS): All study participants in FAS who received at least 1 dose of IMP. Number of Participants Analyzed included participants who were evaluable for the assessment. 'n' included participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[5] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	73	66		
Units: score on a scale				
arithmetic mean (standard deviation)				
Cycle 1: Treatment (Day 43) (n=73,66)	-7.6 (± 7.3)	-4.7 (± 5.7)		
Cycle 2: Treatment (Day 43) (n=56,65)	-5.7 (± 5.4)	-7.5 (± 7.0)		
Cycle 3: Treatment (Day 43) (n=41,56)	-6.8 (± 5.9)	-6.1 (± 7.2)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline (Day 1) to Day 43 in Myasthenia Gravis (MG) Symptoms Patient Reported Outcome (PRO) 'Muscle Weakness Fatigability' score within one treatment cycle (Cycle 1, 2, and 3)

End point title	Change from Baseline (Day 1) to Day 43 in Myasthenia Gravis (MG) Symptoms Patient Reported Outcome (PRO) 'Muscle Weakness Fatigability' score within one treatment cycle (Cycle 1, 2, and 3)[6]

End point description:

The MG symptoms PRO instrument consisted of 42 items across 5 scales: ocular symptoms (1-5); bulbar muscle weakness (6-15); respiratory muscle weakness (16-18); physical fatigue (19-33) and muscle weakness fatigability (34-42). Study participants had to choose response option that best described severity of ocular, bulbar, and respiratory symptoms over past 7 days using a 4-point Likert scale ("none" to "severe") and frequency of experiencing physical fatigue and muscle weakness fatigability over past 7 days using a 5-point Likert scale (1="none of the time" – 5= "all of the time"), for each item. Sum of each item score is linearly transformed to have all domain scores ranging from 0 to 100, higher scores indicates more severe symptoms. Safety Set (SS): All study participants in FAS who received at least 1 dose of IMP. Number of Participants Analyzed: participants who were evaluable for assessment. 'n' included participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[6] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	75	66		
Units: score on a scale				
arithmetic mean (standard deviation)				
Cycle 1: Treatment (Day 43) (n=75,66)	-17.6 (± 21.0)	-14.6 (± 17.2)		
Cycle 2: Treatment (Day 43) (n=56,65)	-13.2 (± 19.2)	-19.2 (± 21.6)		

Cycle 3: Treatment (Day 43) (n=41,58)	-12.8 (± 14.4)	-15.4 (± 17.7)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline (Day 1) to Day 43 in MG Symptoms PRO 'Physical Fatigue' score within one treatment cycle (Cycle 1, 2, and 3)

End point title	Change from Baseline (Day 1) to Day 43 in MG Symptoms PRO 'Physical Fatigue' score within one treatment cycle (Cycle 1, 2, and 3)[7]

End point description:

The MG symptoms PRO instrument consisted of 42 items across 5 scales: ocular muscle weakness (1-5); bulbar muscle weakness (6-15); respiratory muscle weakness (16-18); physical fatigue (19-33) and muscle weakness fatigability (34-42). Study participants had to choose response option based on frequency of experiencing physical fatigue (19-33) over past 7 days using a 5-point Likert scale (1="none of time" - 5="all of time") for each item. Sum of each item score is linearly transformed to have all domain scores and total score ranging from 0 to 100, higher scores indicated severe symptoms. Safety Set (SS): All study participants in FAS who received at least 1 dose of IMP. Number of Participants Analyzed: participants who were evaluable for the assessment. 'n' included participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[7] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	75	66		
Units: score on a scale				
arithmetic mean (standard deviation)				
Cycle 1: Treatment (Day 43) (n=75,66)	-16.5 (± 18.6)	-14.8 (± 18.5)		
Cycle 2: Treatment (Day 43) (n=56,65)	-13.6 (± 21.6)	-16.0 (± 18.2)		
Cycle 3: Treatment (Day 43) (n=41,58)	-15.3 (± 16.9)	-15.0 (± 18.4)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline (Day 1) to Day 43 in MG Symptoms PRO 'Bulbar Muscle Weakness' score within one treatment cycle (Cycle 1, 2, and 3)

End point title	Change from Baseline (Day 1) to Day 43 in MG Symptoms PRO 'Bulbar Muscle Weakness' score within one treatment cycle (Cycle 1, 2, and 3)[8]

End point description:

The MG symptoms PRO instrument consisted of 42 items across 5 scales: ocular muscle weakness (1-5); bulbar muscle weakness (6-15); respiratory muscle weakness (16-18); physical fatigue (19-33) and muscle weakness fatigability (34-42). Bulbar symptoms are now recognised as bulbar muscle weakness. Study participants were asked to choose response option that best described severity of bulbar muscle weakness (6-15) symptoms over past 7 days using a 4-point Likert scale (1="none" to 4="severe") for each item. Sum of each item score is linearly transformed to have all domain scores and total score ranging from 0 to 100, higher scores indicated severe symptoms. Safety Set (SS): All study participants in the FAS who received at least one dose of IMP. Number of Participants Analyzed: participants who were evaluable for the assessment. 'n' included those participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[8] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	75	66		
Units: score on a scale				
arithmetic mean (standard deviation)				
Cycle 1: Treatment (Day 43) (n=75,66)	-13.4 (± 19.8)	-11.6 (± 16.2)		
Cycle 2: Treatment (Day 43) (n=56,65)	-10.4 (± 16.2)	-15.5 (± 18.1)		
Cycle 3: Treatment (Day 43) (n=41,58)	-14.0 (± 16.8)	-13.0 (± 17.9)		

Statistical analyses

No statistical analyses for this end point

Secondary: MG-ADL responder rate (>=2.0-point improvement from Baseline [Day 1]) within one treatment cycle (Cycle 1, 2, and 3 [Day 43])

End point title	MG-ADL responder rate (>=2.0-point improvement from Baseline [Day 1]) within one treatment cycle (Cycle 1, 2, and 3 [Day 43])[9]

End point description:

The MG-ADL is an 8-item PRO instrument developed on the basis of the Quantitative Myasthenia Gravis (QMG). The MG-ADL targeted symptoms and disability across ocular, bulbar, respiratory, and axial symptoms. The total MG-ADL score was obtained by summing the responses to each individual item (8 items; Grades: 0, 1, 2, 3), where 0 represents no symptoms or impaired performance and 3 represents the most severe symptoms or impaired performance. The total score ranges from 0 to 24, with a higher score indicating more disability. A MG-ADL responder was defined as achieving at least 2.0-point improvement (decrease) in the MG-ADL score from Baseline. Safety Set (SS): All study participants in the FAS who received at least one dose of IMP. Number of Participants Analyzed: participants who were evaluable for the assessment. 'n' included those participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

Day 43 of Cycle 1, 2, and 3

Notes:

[9] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	74	66		
Units: percentage of participants				
number (not applicable)				
Cycle 1: Treatment (Day 43) (n=74,66)	74.3	63.6		
Cycle 2: Treatment (Day 43) (n=56,65)	62.5	67.7		
Cycle 3: Treatment (Day 43) (n=41,58)	73.2	67.2		

Statistical analyses

No statistical analyses for this end point

Secondary: Time between consecutive treatment cycles

End point title	Time between consecutive treatment cycles[10]

End point description:

Time between consecutive treatment cycles: Study participants were assessed for MG worsening prior to repeated cycles. In case of symptom worsening (eg, an increase of 2.0 points on MG-ADL or 3.0 points on QMG scale) between assessments, resulted additional treatment, study participants undergone another 6-week treatment cycle followed by an Observation Period, based on Investigator's discretion. Time between treatment cycles was calculated as: date of first sc infusion in consecutive cycle - date of last sc infusion before new cycle + 1 (or date of censoring - date of last sc infusion before potential new cycle + 1). Safety Set (SS): All study participants in FAS who received at least 1 dose of IMP. 'n' included participants who were evaluable for specified category. 99999 indicates median and full range were not estimated as no participants were analysed at specific timepoint.

End point type	Secondary

End point timeframe:

From end of the 6-week treatment cycle (Day 43) to the next 6-week treatment cycle (Day 1), assessed up to 2.5 years

Notes:

[10] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	80	77		
Units: days				
median (full range (min-max))				
Between Cycle 1 and Cycle 2 (n=80,77)	64.0 (9.0 to 883)	51.0 (2.0 to 856)		
Between Cycle 2 and Cycle 3 (n=59,67)	58.0 (1.0 to 631)	50.0 (15.0 to 575)		
Between Cycle 3 and Cycle 4 (n=44,61)	42.5 (27 to 399)	43.0 (5 to 194)		
Between Cycle 4 and Cycle 5 (n=40,55)	43.0 (9 to 181)	43.0 (17 to 242)		
Between Cycle 5 and Cycle 6 (n=36,51)	44.0 (22 to 192)	43.0 (15 to 175)		
Between Cycle 6 and Cycle 7 (n=33,49)	36.0 (8 to 348)	43.0 (7 to 106)		
Between Cycle 7 and Cycle 8 (n=31,40)	37.0 (22 to 87)	37.0 (6 to 84)		

Between Cycle 8 and Cycle 9 (n=27,36)	36.0 (8 to 120)	36.0 (7 to 134)		
Between Cycle 9 and Cycle 10 (n=21,31)	29.0 (7 to 97)	29.0 (8 to 72)		
Between Cycle 10 and Cycle 11 (n=15,26)	29.0 (8 to 64)	29.0 (8 to 99)		
Between Cycle 11 and Cycle 12 (n=11,19)	23.0 (8 to 52)	22.0 (7 to 37)		
Between Cycle 12 and Cycle 13 (n=9,13)	22.0 (6 to 36)	22.0 (7 to 38)		
Between Cycle 13 and Cycle 14 (n=5,9)	29.0 (22 to 35)	16.0 (8 to 65)		
Between Cycle 14 and Cycle 15 (n=4,5)	29.0 (29 to 64)	9.0 (8 to 16)		
Between Cycle 15 and Cycle 16 (n=3,2)	23.0 (8 to 29)	26.5 (9 to 44)		
Between Cycle 16 and Cycle 17 (n=1,0)	29.0 (29 to 29)	99999 (99999 to 99999)		
Between Cycle 17 and Cycle 18 (n=1,0)	8.0 (8 to 8)	99999 (99999 to 99999)		

Statistical analyses

No statistical analyses for this end point

Secondary: Time to MG-ADL response (>=2.0-point improvement from Baseline [Day 1]) within one treatment cycle (Cycle 1, 2, and 3)

End point title	Time to MG-ADL response (>=2.0-point improvement from Baseline [Day 1]) within one treatment cycle (Cycle 1, 2, and 3)[11]

End point description:

Time to achieve MG-ADL response, defined as at least 2.0-point improvement from Baseline. Time to first MG-ADL response (in days) by study cycle was defined as date of first MG-ADL Response within study cycle − date of MG-ADL Baseline within study cycle + 1. Safety Set (SS): All study participants in the FAS who received at least one dose of IMP. Number Analyzed included those participants who were evaluable at specified timepoint.

End point type	Secondary

End point timeframe:

From Baseline (Day 1) to Day 43 of Cycle 1, 2, and 3

Notes:

[11] - 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: The endpoint was assessed only for participants who received treatment and the arm 'Enrolled, but not treated' did not receive any treatment during this study.

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	80	77		
Units: days				
median (confidence interval 95%)				
Cycle 1 (n=80,77)	9.00 (8.00 to 15.00)	22.00 (15.00 to 36.00)		
Cycle 2 (n=59,67)	15.00 (10.00 to 17.00)	15.00 (9.00 to 22.00)		
Cycle 3 (n=44,61)	15.00 (9.00 to 15.00)	15.00 (9.00 to 17.00)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Enrolled, but not treated: From Screening until End of Study (up to 34 months); Randomized participants: From Baseline (Day 1) until End of Study (up to 34 months)

Adverse event reporting additional description:

TEAEs were reported for all study participants in the FAS who received at least 1 dose of IMP. Eligible participants who did not undergo any treatment of RLZ but experienced an AE, are presented in a separate group "Enrolled but not treated". Participants who switched doses were counted in both RLZ doses.

Assessment type	Non-systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	24.0

Reporting groups

Reporting group title	Rozanolixizumab ~7 mg/kg

Reporting group description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of subcutaneous (sc) dose of Rozanolixizumab (RLZ) equivalent to approximately 7 milligrams/kilogram (mg/kg) once weekly (QW), followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, premature end of treatment (PEOT) Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator’s discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator’s discretion.

Reporting group title	Enrolled, but not Treated

Reporting group description:

Participants were enrolled but did not receive a treatment cycle of Rozanolixizumab (RLZ) in MG0007 per the Investigator’s discretion.

Reporting group title	Rozanolixizumab ~10 mg/kg

Reporting group description:

Eligible participants from MG0003 were randomized to receive an initial fixed 6-week treatment cycle of sc dose of RLZ equivalent to approximately 10 mg/kg QW, followed by OP that began after last dose of that treatment cycle. Eligible participants from MG0004 who completed at least 6 scheduled visits for RLZ treatment, PEOT Visit and who were in OP could complete End of Study Visit could move directly into OP in MG0007. These participants underwent their first MG0007 treatment with RLZ upon worsening of MG symptoms. Participants continued their last treatment dose from MG0004. Dose adjustments could be applied in further cycles as per Investigator’s discretion. If symptom worsens between assessments, resulting in need for additional treatment, participants underwent another 6-week treatment cycle followed by OP, based on Investigator’s discretion.

Serious adverse events	Rozanolixizumab ~7 mg/kg	Enrolled, but not Treated	Rozanolixizumab ~10 mg/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	16 / 102 (15.69%)	3 / 8 (37.50%)	31 / 102 (30.39%)
number of deaths (all causes)	1	1	3
number of deaths resulting from adverse events	0	0	0
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			

Retroperitoneal neoplasm			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Invasive ductal breast carcinoma			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Prostate cancer stage II			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Thymoma			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Intestinal adenocarcinoma			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Vascular disorders			
Shock haemorrhagic			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hypertension			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Surgical and medical procedures			
Thymectomy			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General physical health deterioration			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pyrexia			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Haemothorax			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Chronic obstructive pulmonary disease			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Bronchitis chronic			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory failure			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Arteriovenous fistula thrombosis			

subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			
Angina unstable			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac failure			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 1
Atrial fibrillation			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Myocardial infarction			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 8	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 0
Nervous system disorders			
Sciatica			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Transient global amnesia			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Myasthenia gravis crisis			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	4 / 102 (3.92%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 4
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Myasthenia gravis			

subjects affected / exposed	5 / 102 (4.90%)	1 / 8 (12.50%)	11 / 102 (10.78%)
occurrences causally related to treatment / all	0 / 8	0 / 1	0 / 17
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Iron deficiency anaemia			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Eye disorders			
Macular hole			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Duodenal ulcer haemorrhage			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Large intestine polyp			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Oesophageal food impaction			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Skin and subcutaneous tissue disorders			
Subacute cutaneous lupus erythematosus			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Nephrolithiasis			

subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Dysuria			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Endocrine disorders			
Adrenal disorder			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Musculoskeletal and connective tissue disorders			
Osteoarthritis			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Diverticulitis			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Liver abscess			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
COVID-19 pneumonia			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 1
COVID-19			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	2 / 102 (1.96%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 1

Meningitis aseptic			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Endocarditis			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia bacterial			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sinusitis aspergillus			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	0 / 102 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Peritonitis			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Complicated appendicitis			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Appendicitis			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Urosepsis			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Septic shock			

subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	1 / 102 (0.98%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 0

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

Non-serious adverse events	Rozanolixizumab ~7 mg/kg	Enrolled, but not Treated	Rozanolixizumab ~10 mg/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	67 / 102 (65.69%)	5 / 8 (62.50%)	78 / 102 (76.47%)
Investigations			
Lymphocyte count decreased			
subjects affected / exposed	3 / 102 (2.94%)	0 / 8 (0.00%)	7 / 102 (6.86%)
occurrences (all)	8	0	8
Blood immunoglobulin G decreased			
subjects affected / exposed	6 / 102 (5.88%)	0 / 8 (0.00%)	15 / 102 (14.71%)
occurrences (all)	13	0	27
Blood cholesterol increased			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	7 / 102 (6.86%)
occurrences (all)	0	0	10
Blood triglycerides increased			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	6 / 102 (5.88%)
occurrences (all)	0	0	10
Low density lipoprotein increased			
subjects affected / exposed	0 / 102 (0.00%)	0 / 8 (0.00%)	8 / 102 (7.84%)
occurrences (all)	0	0	9
Interferon gamma release assay positive			
subjects affected / exposed	1 / 102 (0.98%)	1 / 8 (12.50%)	2 / 102 (1.96%)
occurrences (all)	1	1	2
Injury, poisoning and procedural complications			

Fall			
subjects affected / exposed	3 / 102 (2.94%)	1 / 8 (12.50%)	5 / 102 (4.90%)
occurrences (all)	3	1	6
Vascular disorders			
Hypertension			
subjects affected / exposed	3 / 102 (2.94%)	0 / 8 (0.00%)	8 / 102 (7.84%)
occurrences (all)	4	0	11
Nervous system disorders			
Headache			
subjects affected / exposed	36 / 102 (35.29%)	0 / 8 (0.00%)	46 / 102 (45.10%)
occurrences (all)	99	0	218
Dizziness			
subjects affected / exposed	2 / 102 (1.96%)	0 / 8 (0.00%)	10 / 102 (9.80%)
occurrences (all)	2	0	11
Myasthenia gravis			
subjects affected / exposed	2 / 102 (1.96%)	0 / 8 (0.00%)	6 / 102 (5.88%)
occurrences (all)	2	0	6
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	9 / 102 (8.82%)	0 / 8 (0.00%)	19 / 102 (18.63%)
occurrences (all)	20	0	34
Influenza like illness			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	7 / 102 (6.86%)
occurrences (all)	1	0	8
Oedema peripheral			
subjects affected / exposed	7 / 102 (6.86%)	0 / 8 (0.00%)	8 / 102 (7.84%)
occurrences (all)	8	0	9
Hepatic pain			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	0 / 102 (0.00%)
occurrences (all)	0	1	0
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	20 / 102 (19.61%)	0 / 8 (0.00%)	28 / 102 (27.45%)
occurrences (all)	49	0	55
Abdominal pain			

subjects affected / exposed	10 / 102 (9.80%)	0 / 8 (0.00%)	7 / 102 (6.86%)
occurrences (all)	11	0	11
Abdominal pain upper			
subjects affected / exposed	2 / 102 (1.96%)	0 / 8 (0.00%)	6 / 102 (5.88%)
occurrences (all)	2	0	8
Nausea			
subjects affected / exposed	9 / 102 (8.82%)	0 / 8 (0.00%)	18 / 102 (17.65%)
occurrences (all)	17	0	26
Vomiting			
subjects affected / exposed	2 / 102 (1.96%)	0 / 8 (0.00%)	8 / 102 (7.84%)
occurrences (all)	3	0	10
Respiratory, thoracic and mediastinal disorders			
Oropharyngeal pain			
subjects affected / exposed	5 / 102 (4.90%)	0 / 8 (0.00%)	9 / 102 (8.82%)
occurrences (all)	5	0	13
Cough			
subjects affected / exposed	3 / 102 (2.94%)	0 / 8 (0.00%)	6 / 102 (5.88%)
occurrences (all)	5	0	7
Dyspnoea			
subjects affected / exposed	3 / 102 (2.94%)	1 / 8 (12.50%)	6 / 102 (5.88%)
occurrences (all)	4	1	6
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	5 / 102 (4.90%)	0 / 8 (0.00%)	8 / 102 (7.84%)
occurrences (all)	7	0	8
Myalgia			
subjects affected / exposed	1 / 102 (0.98%)	0 / 8 (0.00%)	6 / 102 (5.88%)
occurrences (all)	2	0	9
Arthralgia			
subjects affected / exposed	5 / 102 (4.90%)	0 / 8 (0.00%)	12 / 102 (11.76%)
occurrences (all)	6	0	13
Infections and infestations			
Urinary tract infection			
subjects affected / exposed	6 / 102 (5.88%)	1 / 8 (12.50%)	2 / 102 (1.96%)
occurrences (all)	12	1	3
COVID-19			

subjects affected / exposed	15 / 102 (14.71%)	1 / 8 (12.50%)	24 / 102 (23.53%)
occurrences (all)	15	1	26
Upper respiratory tract infection			
subjects affected / exposed	7 / 102 (6.86%)	0 / 8 (0.00%)	11 / 102 (10.78%)
occurrences (all)	13	0	22
Nasopharyngitis			
subjects affected / exposed	10 / 102 (9.80%)	1 / 8 (12.50%)	11 / 102 (10.78%)
occurrences (all)	16	1	14
Streptococcal urinary tract infection			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	0 / 102 (0.00%)
occurrences (all)	0	1	0
Urinary tract infection bacterial			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	0 / 102 (0.00%)
occurrences (all)	0	1	0
Metabolism and nutrition disorders			
Hypomagnesaemia			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	1 / 102 (0.98%)
occurrences (all)	0	1	1
Fluid overload			
subjects affected / exposed	0 / 102 (0.00%)	1 / 8 (12.50%)	0 / 102 (0.00%)
occurrences (all)	0	1	0

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
03 March 2021	Protocol Amendment 1 (dated 03 Mar 2021) was primarily implemented to incorporate an additional study objective and endpoint on post vaccination biomarkers in study participants who had received a coronavirus disease 2019 (COVID-19) vaccine. Other required changes included aligning the protocol with the updates for the rozanolixizumab generalized myasthenia gravis (gMG) clinical program, and to incorporate specific local ethics committees and/or agency requirements into the global protocol.
30 June 2022	Protocol Amendment 2 (dated 30 Jun 2022) was primarily implemented to incorporate the option to administer investigational medicinal product (IMP) via manual push instead of using a syringe driver, implement changes specific to the Schedule of Activities, update the temporary investigational medicinal product (IMP) discontinuation criteria, and update the study medication permanent discontinuation criteria, as well as provide additional information on the benefit-risk for study participants who had received a full COVID-19 vaccination. Protocol Amendment 2 was approved and submitted to Food and Drug Administration (FDA) but was not implemented at the study sites.
03 October 2022	Protocol Amendment 3 (dated 03 Oct 2022) was primarily implemented to provide an update on the safety information in line with the updated Investigator's Brochure dated Sep 2022 and to update the list of adverse events of special monitoring (adverse events of special monitoring [AESM]; remove severe gastrointestinal [GI] disorders [ie, abdominal pain, diarrhea, vomiting] and opportunistic infections and include serious headache and suspected aseptic meningitis). The possibility for participants receiving rescue therapy during the Observation Period to continue in the study was also added. The criteria for study medication discontinuation and the requirements for male contraception were also updated. Additional study assessments were specified in case of AESM, and a full neurological examination was added as part of the physical examination.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported