Clinical trial results:
A Randomized, Open-Label Extension Study to Investigate the Long-Term Safety, Tolerability, and Efficacy of Rozanolixizumab in Adult Patients With Generalized Myasthenia Gravis

Summary

EudraCT number	2019-000969-21
Trial protocol	HU GB DK BE DE ES CZ PL IT
Global end of trial date	01 September 2021

Results information

Result version number	v1 (current)
This version publication date	14 September 2022
First version publication date	14 September 2022

Trial information

Trial identification

Sponsor protocol code	MG0004

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04124965
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	01 October 2021
Is this the analysis of the primary completion data?	Yes
Primary completion date	01 September 2021
Global end of trial reached?	Yes
Global end of trial date	01 September 2021
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Evaluate the long-term safety and tolerability of rozanolixizumab in study participants with generalized myasthenia gravis (MG)

Protection of trial subjects:

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

Background therapy:

Not applicable

Evidence for comparator:

Not applicable

Actual start date of recruitment	29 October 2019
Long term follow-up planned	Yes
Long term follow-up rationale	Efficacy, Safety
Long term follow-up duration	2 Years
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Canada: 10
Country: Number of subjects enrolled	Czechia: 2
Country: Number of subjects enrolled	Denmark: 1
Country: Number of subjects enrolled	France: 12
Country: Number of subjects enrolled	Germany: 3
Country: Number of subjects enrolled	Italy: 2
Country: Number of subjects enrolled	Japan: 6
Country: Number of subjects enrolled	Poland: 7
Country: Number of subjects enrolled	Russian Federation: 6
Country: Number of subjects enrolled	Spain: 3
Country: Number of subjects enrolled	Taiwan: 2
Country: Number of subjects enrolled	United States: 17
Worldwide total number of subjects	71
EEA total number of subjects	30

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)	55
From 65 to 84 years	14
85 years and over	2

Subject disposition

Recruitment

Recruitment details:

The study started to enroll study participants in Oct 2019 and concluded in Sep 2021.

Pre-assignment

Screening details:

Participant Flow refers to the Randomized Set which consisted of all study participants who were randomized, using the treatment assigned instead of the actual treatment received.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Rozanolixizumab ~7 mg/kg

Arm description:

Participants received rozanolixizumab equivalent to approximately 7 milligrams/kilogram (mg/kg), subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60).

Arm type	Experimental
Investigational medicinal product name	Rozanolixizumab
Investigational medicinal product code	UCB7665
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received rozanolixizumab equivalent to 7 mg/kg subcutaneously on a weekly basis over a 52-week Treatment Period.

Arm title	Rozanolixizumab ~10 mg/kg

Arm description:

Participants received rozanolixizumab equivalent to approximately 10 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60).

Arm type	Experimental
Investigational medicinal product name	Rozanolixizumab
Investigational medicinal product code	UCB7665
Other name	
Pharmaceutical forms	Solution for infusion
Routes of administration	Subcutaneous use

Dosage and administration details:

Participants received rozanolixizumab equivalent to 10 mg/kg subcutaneously on a weekly basis over a 52-week Treatment Period.

Number of subjects in period 1	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg
Started	35	36
Safety Set	35	35
Completed	5	3
Not completed	30	33
Personal surgery	-	1
Consent withdrawn by subject	1	1
Physician decision	-	1
Adverse event, non-fatal	3	1
Pregnancy	1	-
Rolled Over To MG0007 Study	25	28
Sponsor and participant decision	-	1

Baseline characteristics

Reporting groups	
Reporting group title	Rozanolixizumab ~7 mg/kg
Reporting group description:	
Participants received rozanolixizumab equivalent to approximately 7 milligrams/kilogram (mg/kg), subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60).	
Reporting group title	Rozanolixizumab ~10 mg/kg
Reporting group description:	
Participants received rozanolixizumab equivalent to approximately 10 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60).	

Reporting group values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg	Total
Number of subjects	35	36	71
Age Categorical Units: participants			
<=18 years	0	0	0
Between 18 and 65 years	29	26	55
>=65 years	6	10	16
Age Continuous Units: years			
arithmetic mean	50.6	53.7	
standard deviation	± 14.2	± 17.2	-
Sex: Female, Male Units: participants			
Female	19	19	38
Male	16	17	33

End points

End points reporting groups

Reporting group title	Rozanolixizumab ~7 mg/kg

Reporting group description:

Participants received rozanolixizumab equivalent to approximately 7 milligrams/kilogram (mg/kg), subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60).

Reporting group title	Rozanolixizumab ~10 mg/kg

Reporting group description:

Participants received rozanolixizumab equivalent to approximately 10 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60).

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

Subject analysis set description:

Participants received rozanolixizumab equivalent to approximately 7 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60). This set included participants that switched to Rozanolixizumab equivalent to approximately 10 mg/kg at least once during the study.

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

Subject analysis set description:

Participants received rozanolixizumab equivalent to approximately 10 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60). This set included participants that switched to Rozanolixizumab equivalent to approximately 7 mg/kg at least once during the study.

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:

A TEAE is defined as an AE starting on or after the time of first administration of investigational medicinal product (IMP) or any unresolved event already present before the first administration of IMP that worsened in intensity following exposure to IMP, up to 8 weeks after the last dose of IMP in study participants who discontinued the study or IMP. The Safety Set (SS) consisted of all randomized study participants who received at least 1 dose of IMP in this study. This endpoint was planned to be analyzed using the SS by most recent dose received i.e. the most recent dose received at or before the AE onset. Participants who switched doses were counted in both rozanolixizumab (RLZ) doses.

End point type	Primary

End point timeframe:

From Baseline until End of Study (up to Week 60)

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 in tables 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	50	42		
Units: percentage of participants				
number (not applicable)	76.0	78.6		

Statistical analyses

No statistical analyses for this end point

Primary: Percentage of participants with treatment-emergent adverse events (TEAEs) leading to permanent withdrawal of study medication

End point title	Percentage of participants with treatment-emergent adverse events (TEAEs) leading to permanent withdrawal of study medication[2]

End point description:

A TEAE is 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 worsened in intensity following exposure to IMP, up to 8 weeks after the last dose of IMP in study participants who discontinued the study or IMP. The Safety Set consisted of all randomized study participants who received at least 1 dose of IMP in this study. This endpoint was planned to be analyzed using the SS by most recent dose received i.e. the most recent dose received at or before the AE onset. Participants who switched doses were counted in both rozanolixizumab doses.

End point type	Primary

End point timeframe:

From Baseline until End of Study (up to Week 60)

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 in tables 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	50	42		
Units: percentage of participants				
number (not applicable)	6.0	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score at each scheduled assessment during Treatment and Observation Periods

End point title	Change from Baseline in Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score at each scheduled assessment during Treatment and Observation Periods

End point description:

MG-ADL is an 8-item patient-reported outcome (PRO) instrument developed on the basis of the 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 positive change indicates worsening and a negative change indicates improvement. The Safety Set consisted of all randomized study participants who received at least 1 dose of IMP in this study. Here, number analyzed (n) signifies those who were evaluable at specified time points. 99999 signifies that as pre-specified in the Statistical Analysis Plan, Standard Deviation was only calculated if there were a minimum of 4 participants.

End point type	Secondary

End point timeframe:

Baseline, Weeks 5, 7, 9, 13, 17, 21, 25, 29, 33, 37, 41, 45, 49, 52 and 60

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	35	35		
Units: units on a scale				
arithmetic mean (standard deviation)				
Week 5 (n=34, 34)	-2.7 (± 3.3)	-3.2 (± 3.8)		
Week 7 (n=35, 32)	-2.7 (± 3.8)	-3.7 (± 3.4)		
Week 9 (n=29, 31)	-2.8 (± 3.4)	-3.4 (± 3.7)		
Week 13 (n=30, 30)	-3.1 (± 3.4)	-3.9 (± 4.0)		
Week 17 (n=25, 29)	-2.8 (± 3.3)	-4.0 (± 3.9)		
Week 21 (n=20, 24)	-3.0 (± 3.4)	-4.1 (± 4.3)		
Week 25 (n=18, 17)	-2.7 (± 3.0)	-3.7 (± 4.7)		
Week 29 (n=13, 14)	-2.8 (± 2.1)	-3.6 (± 4.3)		
Week 33 (n=10, 12)	-3.0 (± 2.8)	-3.5 (± 4.5)		
Week 37 (n=7, 10)	-3.9 (± 2.5)	-3.6 (± 3.6)		
Week 41 (n=6, 6)	-2.8 (± 2.1)	-1.8 (± 1.0)		
Week 45 (n=4, 7)	-3.8 (± 2.4)	-2.1 (± 1.1)		
Week 49 (n=5, 6)	-2.4 (± 1.1)	-0.5 (± 3.7)		
Week 52 (n=5, 3)	-2.6 (± 1.3)	-2.0 (± 99999)		
Week 60 (n=7, 7)	-0.3 (± 2.1)	-1.3 (± 3.9)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Myasthenia Gravis-Composite (MG-C) total score at each scheduled assessment during Treatment and Observation Periods

End point title	Change from Baseline in Myasthenia Gravis-Composite (MG-C) total score at each scheduled assessment during Treatment and Observation Periods

End point description:

MG-C scale is a validated assessment and scale tests 10 items with individual items being weighted differently. Items: ptosis/upward gaze (range: 0 [>45 second] -3 [Immediate]), double vision on lateral gaze (0 [>45 second] -4 [Immediate]), eye closure (0 [Normal] -2 [severe weakness]), talking (0 [Normal] -6 [difficult to understand speech]), chewing & swallowing (0 [Normal] -6 [gastric tube]), breathing (0 [Normal] -9 [ventilator dependence]), neck flexion (0 [Normal] -4 [severe weakness]), shoulder abduction & hip felxion (0 [Normal] -5 [severe weakness]), lower scores= lower disease activity. Total MG-C score was obtained by summing responses to each individual item and score ranges from 0 to 50,lower scores=lower disease activity.A positive change=worsening and a negative change=improvement.Analysis population was SS.n= participants evaluable at specified time points.

99999=mean and S.D. was calculated if there were a minimum of 3 participants and 4 participants respectively.

End point type	Secondary

End point timeframe:

Baseline, Weeks 5, 7, 9, 13, 17, 21, 25, 29, 33, 37, 41, 45, 49, 52 and 60

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	35	35		
Units: units on a scale				
arithmetic mean (standard deviation)				
Week 5 (n=34, 35)	-4.7 (± 5.5)	-5.5 (± 7.3)		
Week 7 (n=35, 33)	-5.0 (± 5.7)	-7.1 (± 7.4)		
Week 9 (n=29, 30)	-5.0 (± 5.3)	-6.0 (± 7.4)		
Week 13 (n=30, 30)	-4.8 (± 5.5)	-7.0 (± 7.8)		
Week 17 (n=25, 29)	-4.4 (± 5.7)	-5.5 (± 8.6)		
Week 21 (n=20, 24)	-5.3 (± 6.9)	-7.3 (± 8.1)		
Week 25 (n=18, 17)	-6.1 (± 5.8)	-8.8 (± 7.7)		
Week 29 (n=13, 14)	-5.1 (± 5.5)	-9.1 (± 9.2)		
Week 33 (n=9, 12)	-4.6 (± 5.1)	-8.4 (± 8.6)		
Week 37 (n=7, 10)	-6.3 (± 6.8)	-8.6 (± 6.9)		
Week 41 (n=6, 6)	-6.0 (± 7.1)	-6.5 (± 5.8)		
Week 45 (n=4, 7)	-4.0 (± 6.2)	-5.3 (± 4.9)		
Week 49 (n=5, 6)	-1.4 (± 3.8)	-0.8 (± 9.2)		
Week 52 (n=5, 2)	-3.8 (± 3.1)	99999 (± 99999)		
Week 60 (n=7, 7)	1.7 (± 3.7)	-2.3 (± 8.5)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in Quantitative Myasthenia Gravis (QMG) total score at each scheduled assessment during Treatment and Observation Periods

End point title	Change from Baseline in Quantitative Myasthenia Gravis (QMG) total score at each scheduled assessment during Treatment and Observation Periods

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 indicates worsening and a negative change indicates improvement. The Safety Set consisted of all randomized study participants who received at least 1 dose of IMP in this study. Here, number analyzed signifies those participants who were evaluable at specified time points. 99999 signifies that as pre-specified in the Statistical Analysis Plan, mean was only calculated if there were a minimum of 3 participants and standard deviation was only calculated if there were a minimum of 4 participants.

End point type	Secondary

End point timeframe:

Baseline, Weeks 5, 7, 9, 13, 17, 21, 25, 29, 33, 37, 41, 45, 49, 52 and 60

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	35	35		
Units: units on a scale				
arithmetic mean (standard deviation)				
Week 5 (n=34, 34)	-2.9 (± 4.7)	-4.5 (± 4.4)		
Week 7 (n=35, 32)	-3.3 (± 4.4)	-5.2 (± 4.3)		
Week 9 (n=28, 30)	-3.3 (± 3.8)	-4.7 (± 4.3)		
Week 13 (n=30, 29)	-2.6 (± 4.2)	-5.5 (± 4.4)		
Week 17 (n=25, 28)	-3.1 (± 4.9)	-4.2 (± 4.4)		
Week 21 (n=20, 23)	-4.0 (± 4.7)	-5.1 (± 4.9)		
Week 25 (n=18, 16)	-5.1 (± 4.6)	-5.7 (± 5.5)		
Week 29 (n=13, 13)	-5.4 (± 3.6)	-5.5 (± 6.3)		
Week 33 (n=10, 11)	-4.9 (± 4.8)	-6.2 (± 5.7)		
Week 37 (n=7, 9)	-5.3 (± 3.9)	-6.8 (± 5.9)		
Week 41 (n=6, 5)	-5.7 (± 4.3)	-4.0 (± 3.1)		
Week 45 (n=4, 6)	-5.3 (± 2.8)	-4.0 (± 3.5)		
Week 49 (n=5, 5)	-3.8 (± 0.8)	-1.8 (± 1.8)		
Week 52 (n=5, 2)	-4.6 (± 2.9)	99999 (± 99999)		
Week 60 (n=7, 6)	-0.9 (± 2.4)	-1.8 (± 5.1)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of participants using rescue medication (intravenous infusion of immunoglobulin G (IVIg) or plasma exchange (PEX))

End point title	Percentage of participants using rescue medication (intravenous infusion of immunoglobulin G (IVIg) or plasma exchange (PEX))

End point description:

Rescue therapy consisted of IVIg or PEX. Study participants who experienced disease worsening (eg, an increase of 2 points on the MG-ADL or 3 points on the QMG scale between 2 consecutive visits) may be considered for rescue therapy at the discretion of the Investigator. The Safety Set consisted of all randomized study participants who received at least 1 dose of IMP in this study.

End point type	Secondary

End point timeframe:

From Baseline until End of Study (up to Week 60)

End point values	Rozanolixizumab ~7 mg/kg	Rozanolixizumab ~10 mg/kg		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	35	35		
Units: percentage of participants				
number (not applicable)	11.4	0		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

From Baseline until End of Study (up to Week 60)

Adverse event reporting additional description:

TEAEs are reported in the safety section. TEAEs were planned to be analyzed using SS by most recent dose received i.e. the most recent dose received at or before the AE onset. 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 ~10 mg/kg

Reporting group description:

Participants received rozanolixizumab equivalent to approximately 10 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60). This set included participants that switched to Rozanolixizumab equivalent to approximately 7 mg/kg at least once during the study.

Reporting group title	Rozanolixizumab ~7 mg/kg

Reporting group description:

Participants received rozanolixizumab equivalent to approximately 7 mg/kg, subcutaneously on a weekly basis over a 52-week Treatment Period. After 52-week Treatment Period, participants were followed up for 8 weeks (up to Week 60). This set included participants that switched to Rozanolixizumab equivalent to approximately 10 mg/kg at least once during the study.

Serious adverse events	Rozanolixizumab ~10 mg/kg	Rozanolixizumab ~7 mg/kg	
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 42 (4.76%)	7 / 50 (14.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Investigations			
Biopsy kidney abnormal			
subjects affected / exposed	0 / 42 (0.00%)	1 / 50 (2.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Cardiac disorders			
Cardiac failure congestive			
subjects affected / exposed	0 / 42 (0.00%)	1 / 50 (2.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pericarditis			

subjects affected / exposed	1 / 42 (2.38%)	0 / 50 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Myasthenia gravis			
subjects affected / exposed	1 / 42 (2.38%)	3 / 50 (6.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 4	
deaths causally related to treatment / all	0 / 0	0 / 0	
Eye disorders			
Retinal detachment			
subjects affected / exposed	0 / 42 (0.00%)	1 / 50 (2.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Musculoskeletal and connective tissue disorders			
Muscular weakness			
subjects affected / exposed	0 / 42 (0.00%)	1 / 50 (2.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	Rozanolixizumab ~10 mg/kg	Rozanolixizumab ~7 mg/kg	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	25 / 42 (59.52%)	27 / 50 (54.00%)	
Investigations			
Blood immunoglobulin G decreased			
subjects affected / exposed	5 / 42 (11.90%)	6 / 50 (12.00%)	
occurrences (all)	6	12	
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 42 (2.38%)	3 / 50 (6.00%)	
occurrences (all)	1	3	
Nervous system disorders			
Headache			

subjects affected / exposed	12 / 42 (28.57%)	15 / 50 (30.00%)	
occurrences (all)	40	55	
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	3 / 42 (7.14%)	4 / 50 (8.00%)	
occurrences (all)	4	5	
Immune system disorders			
Hypogammaglobulinaemia			
subjects affected / exposed	2 / 42 (4.76%)	2 / 50 (4.00%)	
occurrences (all)	2	4	
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	7 / 42 (16.67%)	6 / 50 (12.00%)	
occurrences (all)	10	11	
Abdominal pain			
subjects affected / exposed	2 / 42 (4.76%)	2 / 50 (4.00%)	
occurrences (all)	2	2	
Nausea			
subjects affected / exposed	5 / 42 (11.90%)	4 / 50 (8.00%)	
occurrences (all)	8	4	
Vomiting			
subjects affected / exposed	4 / 42 (9.52%)	0 / 50 (0.00%)	
occurrences (all)	4	0	
Skin and subcutaneous tissue disorders			
Rash			
subjects affected / exposed	4 / 42 (9.52%)	1 / 50 (2.00%)	
occurrences (all)	7	1	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	3 / 42 (7.14%)	2 / 50 (4.00%)	
occurrences (all)	3	2	
Infections and infestations			
Nasopharyngitis			
subjects affected / exposed	4 / 42 (9.52%)	2 / 50 (4.00%)	
occurrences (all)	4	2	
Urinary tract infection			

subjects affected / exposed occurrences (all)	2 / 42 (4.76%) 2	5 / 50 (10.00%) 5	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
01 November 2019	Protocol Amendment 1 (dated 01 Nov 2019) was a substantial amendment implemented to reference to another lead-in study, MGC003, throughout the protocol; however, MGC003 was not conducted. Other changes included additional wording to allow study participants to enroll into a substudy at selected sites (however, no study participants were included in the substudy), addition of 2 exploratory objective and endpoints (1 to assess the effect of rozanolixizumab on tetanus IgG antibodies, and 1 to capture the reduction steroid use in study participants receiving rozanolixizumab) and changes throughout the Schedule of Assessments.
30 July 2020	Protocol Amendment 2 (dated 30 Jul 2020) was a substantial amendment implemented to introduce the transition of study participants to MG0007 and closure of MG0004, once MG0007 was available as the open-label study to MG0003. Other changes included updates to decrease the complexity of assessments to be performed; to clarify some operational aspects of the study; to incorporate the harmonization of inclusion criteria with studies performed across the rozanolixizumab clinical development program; and to include the management of study participant treatment during the coronavirus disease 2019 (COVID-19) pandemic including contingency measures.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported