Clinical trial results:
A Phase 2, Randomized, Double-Blind, Placebo-Controlled Multicenter Study to Evaluate the Efficacy and Safety of ALXN2050 in Adult Participants with Generalized Myasthenia Gravis

Summary

EudraCT number	2021-001229-26
Trial protocol	IT ES DE
Global end of trial date	03 April 2024

Results information

Result version number	v1 (current)
This version publication date	28 February 2025
First version publication date	28 February 2025

Trial information

Trial identification

Sponsor protocol code	ALXN2050-MG-201

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05218096
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Alexion Pharmaceuticals, Inc.
Sponsor organisation address	121 Seaport Boulevard, Boston, MA, United States, 02210
Public contact	European Clinical Trial Information, Alexion Pharmaceuticals, Inc., +35 3874162507, clinicaltrials.eu@alexion.com
Scientific contact	European Clinical Trial Information, Alexion Pharmaceuticals, Inc., +35 3874162507, clinicaltrials.eu@alexion.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	Interim
Date of interim/final analysis	30 November 2023
Is this the analysis of the primary completion data?	Yes
Primary completion date	30 November 2023
Global end of trial reached?	Yes
Global end of trial date	03 April 2024
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

This study will evaluate the efficacy and safety of ALXN2050 (120 milligrams [mg], 180 mg) in participants with generalized myasthenia gravis (gMG). Safety will be monitored throughout the study.

Protection of trial subjects:

This study was conducted in accordance with the protocol and with the following: Consensus ethical principles derived from international guidelines including the Declaration of Helsinki and Council for International Organizations of Medical Sciences (CIOMS) International Ethical Guidelines; Applicable International Council for Harmonisation of Technical Requirements for Pharmaceuticals for Human Use (ICH) Good Clinical Practice (GCP) guidelines; Applicable laws and regulations.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	27 April 2022
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?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Canada: 4
Country: Number of subjects enrolled	Germany: 4
Country: Number of subjects enrolled	Italy: 20
Country: Number of subjects enrolled	Korea, Republic of: 2
Country: Number of subjects enrolled	Serbia: 9
Country: Number of subjects enrolled	Spain: 9
Country: Number of subjects enrolled	Taiwan: 4
Country: Number of subjects enrolled	United States: 18
Worldwide total number of subjects	70
EEA total number of subjects	33

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37	0

wk	
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	0
Adolescents (12-17 years)	0
Adults (18-64 years)	50
From 65 to 84 years	20
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This study did not meet its primary efficacy end point and was early terminated by the Sponsor.

Pre-assignment

Screening details:

All study drugs (ALXN2050, placebo) were administered twice daily (BID).

Period 1

Period 1 title	Primary Evaluation
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Investigator, Carer, Subject

Blinding implementation details:

Masking of treatment allocation will be observed until at least Week 34.

Arms

Are arms mutually exclusive?	Yes
Arm title	Group 1: ALXN2050 180 mg BID

Arm description:

Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).

Arm type	Experimental
Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 2: ALXN2050 120 mg BID

Arm description:

Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).

Arm type	Experimental
Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 3: Placebo

Arm description:

Participants received placebo BID during the primary evaluation period.

Arm type	Placebo

Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received placebo orally.

Number of subjects in period 1	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3: Placebo
Started	28	14	28
Received at Least 1 Dose of Study Drug	28	14	28
Entered Extended Treatment Period	27	14	26
Re-randomized to ALXN2050 120 mg BID	0 [1]	0 [2]	13 [3]
Re-randomized to ALXN2050 180 mg BID	0 [4]	0 [5]	13 [6]
Completed	27	14	26
Not completed	1	0	2
Physician decision	1	-	-
Consent withdrawn by subject	-	-	2

Notes:

[1] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone applies only to participants in Group 3: Placebo.

[2] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone applies only to participants in Group 3: Placebo.

[3] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: Participants in this arm who completed the Primary Evaluation period were re-randomized to Group 3A: Placebo/ALXN2050 180 mg BID.

[4] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone applies only to participants in Group 3: Placebo.

[5] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: This milestone applies only to participants in Group 3: Placebo.

[6] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: Participants in this arm who completed the Primary Evaluation period were re-randomized to Group 3A: Placebo/ALXN2050 120 mg BID.

Period 2

Period 2 title	Extended Treatment (Ext Treat)
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Carer

Blinding implementation details:

Masking of treatment allocation will be observed until at least Week 34.

Arms

Are arms mutually exclusive?	Yes
Arm title	Group 1: ALXN2050 180 mg BID

Arm description:

Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).

Arm type	Experimental
Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 2: ALXN2050 120 mg BID

Arm description:

Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).

Arm type	Experimental
Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 3a: Placebo/ALXN2050 180 mg BID

Arm description:

Participants received placebo BID during the primary evaluation period, followed by ALXN2050 180 mg BID during the extended treatment period and the open-label extension period.

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

Dosage and administration details:

Participants received placebo orally.

Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 3b: Placebo/ALXN2050 120 mg BID

Arm description:

Participants received placebo BID during the primary evaluation period, followed by ALXN2050 120 mg BID during the extended treatment period and the open-label extension period.

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

Dosage and administration details:

Participants received placebo orally.

Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Number of subjects in period 2	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3a: Placebo/ALXN2050 180 mg BID
Started	27	14	13
Received at Least 1 Dose of Study Drug	27	14	13
Entered Open-label Extension Period	16	6	8
Completed	16	6	8
Not completed	11	8	5
Clinical Deterioration	1	1	-
Consent withdrawn by subject	1	3	2
Physician decision	-	1	-
Study Terminated by Sponsor	6	3	3
Lack of efficacy	3	-	-

Number of subjects in period 2	Group 3b: Placebo/ALXN2050 120 mg BID
Started	13
Received at Least 1 Dose of Study Drug	13
Entered Open-label Extension Period	9
Completed	9
Not completed	4

Clinical Deterioration	-
Consent withdrawn by subject	2
Physician decision	-
Study Terminated by Sponsor	2
Lack of efficacy	-

Period 3

Period 3 title	Open-label Extension (OLE)
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Carer

Blinding implementation details:

Masking of treatment allocation will be observed until at least Week 34.

Arms

Are arms mutually exclusive?	Yes
Arm title	Group 1: ALXN2050 180 mg BID

Arm description:

Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).

Arm type	Experimental
Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 2: ALXN2050 120 mg BID

Arm description:

Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).

Arm type	Experimental
Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 3a: Placebo/ALXN2050 180 mg BID

Arm description:

Participants received placebo BID during the primary evaluation period, followed by ALXN2050 180 mg BID during the extended treatment period and the open-label extension period.

Arm type	Experimental

Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received placebo orally.

Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Arm title	Group 3b: Placebo/ALXN2050 120 mg BID

Arm description:

Participants received placebo BID during the primary evaluation period, followed by ALXN2050 120 mg BID during the extended treatment period and the open-label extension period.

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

Dosage and administration details:

Participants received placebo orally.

Investigational medicinal product name	ALXN2050
Investigational medicinal product code	
Other name	ACH-0145228 (formerly)
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Participants received ALXN2050 orally.

Number of subjects in period 3	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3a: Placebo/ALXN2050 180 mg BID
Started	16	6	8
Received at Least 1 Dose of Study Drug	16	6	8
Completed	0	0	0
Not completed	16	6	8
Adverse event, serious fatal	-	-	1
Adverse event, non-fatal	1	-	-
Pregnancy	-	-	-
Study Terminated by Sponsor	13	4	7
Lost to follow-up	1	-	-
Lack of efficacy	1	2	-

Number of subjects in period 3	Group 3b: Placebo/ALXN2050 120 mg BID
Started	9
Received at Least 1 Dose of Study Drug	9
Completed	0
Not completed	9
Adverse event, serious fatal	-
Adverse event, non-fatal	1
Pregnancy	1
Study Terminated by Sponsor	7
Lost to follow-up	-
Lack of efficacy	-

Baseline characteristics

Reporting groups	
Reporting group title	Group 1: ALXN2050 180 mg BID
Reporting group description:	
Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 2: ALXN2050 120 mg BID
Reporting group description:	
Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 3: Placebo
Reporting group description:	
Participants received placebo BID during the primary evaluation period.	

Reporting group values	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3: Placebo
Number of subjects	28	14	28
Age categorical Units: Subjects			
In utero Preterm newborn infants (gestational age < 37 wks) Newborns (0-27 days) Infants and toddlers (28 days-23 months) Children (2-11 years) Adolescents (12-17 years) Adults (18-64 years) From 65-84 years 85 years and over			
Age Continuous Units: years			
arithmetic mean	49.0	55.9	58.2
standard deviation	± 15.55	± 13.02	± 16.45
Sex: Female, Male Units:			
Female	18	10	10
Male	10	4	18
Ethnicity (NIH/OMB)			
National Institutes of Health/Office of Management & Budget (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	4	1	1
Not Hispanic or Latino	24	13	26
Unknown or Not Reported	0	0	1
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	0	0	0
Asian	2	4	1
Native Hawaiian or Other Pacific Islander	0	0	0

Black or African American	0	0	0
White	26	10	27
More than one race	0	0	0
Unknown or Not Reported	0	0	0
Baseline MG-ADL Total Score			
Myasthenia Gravis Activities of Daily Living (MG-ADL)			
Units: units on a scale			
arithmetic mean	9.4	9.0	8.8
standard deviation	± 2.23	± 2.96	± 1.75

Reporting group values	Total		
Number of subjects	70		
Age categorical			
Units: Subjects			
In utero	0		
Preterm newborn infants (gestational age < 37 wks)	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)	0		
From 65-84 years	0		
85 years and over	0		
Age Continuous			
Units: years			
arithmetic mean			
standard deviation	-		
Sex: Female, Male			
Units:			
Female	38		
Male	32		
Ethnicity (NIH/OMB)			
National Institutes of Health/Office of Management & Budget (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	6		
Not Hispanic or Latino	63		
Unknown or Not Reported	1		
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native	0		
Asian	7		
Native Hawaiian or Other Pacific Islander	0		
Black or African American	0		
White	63		
More than one race	0		
Unknown or Not Reported	0		
Baseline MG-ADL Total Score			
Myasthenia Gravis Activities of Daily Living (MG-ADL)			
Units: units on a scale			

arithmetic mean standard deviation	-		

End points

End points reporting groups

Reporting group title	Group 1: ALXN2050 180 mg BID
Reporting group description:	
Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 2: ALXN2050 120 mg BID
Reporting group description:	
Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 3: Placebo
Reporting group description:	
Participants received placebo BID during the primary evaluation period.	
Reporting group title	Group 1: ALXN2050 180 mg BID
Reporting group description:	
Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 2: ALXN2050 120 mg BID
Reporting group description:	
Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 3a: Placebo/ALXN2050 180 mg BID
Reporting group description:	
Participants received placebo BID during the primary evaluation period, followed by ALXN2050 180 mg BID during the extended treatment period and the open-label extension period.	
Reporting group title	Group 3b: Placebo/ALXN2050 120 mg BID
Reporting group description:	
Participants received placebo BID during the primary evaluation period, followed by ALXN2050 120 mg BID during the extended treatment period and the open-label extension period.	
Reporting group title	Group 1: ALXN2050 180 mg BID
Reporting group description:	
Participants received 180 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 2: ALXN2050 120 mg BID
Reporting group description:	
Participants received 120 mg ALXN2050 BID during all 3 study periods (primary evaluation, extended treatment, open-label extension).	
Reporting group title	Group 3a: Placebo/ALXN2050 180 mg BID
Reporting group description:	
Participants received placebo BID during the primary evaluation period, followed by ALXN2050 180 mg BID during the extended treatment period and the open-label extension period.	
Reporting group title	Group 3b: Placebo/ALXN2050 120 mg BID
Reporting group description:	
Participants received placebo BID during the primary evaluation period, followed by ALXN2050 120 mg BID during the extended treatment period and the open-label extension period.	

Primary: Percentage Of Participants With a Myasthenia Gravis Activities of Daily Living (MG-ADL) Total Score Reduction Of ≥ 2 Points In Any 4 Consecutive Weeks During The First 8 Weeks And Who Did Not Receive Rescue Therapy

End point title	Percentage Of Participants With a Myasthenia Gravis Activities of Daily Living (MG-ADL) Total Score Reduction Of ≥ 2 Points In Any 4 Consecutive Weeks During The First 8 Weeks And

	Who Did Not Receive Rescue Therapy

End point description:

The MG-ADL profile is an 8-item participant-reported scale that focuses on relevant symptoms and functional performance of ADL in participants with MG. The 8 items of the MG-ADL questionnaire were derived from symptom-based components of the original 13-item QMG scale to assess disability secondary to ocular (2 items), bulbar (3 items), respiratory (1 item), and gross motor or limb (2 items) impairment related to effects of MG. Each response is graded 0 (normal) to 3 (most severe). The MG-ADL total score was calculated as the sum of the scores of the 8 items and ranges from 0 to 24, with higher scores indicating worse function.

End point type	Primary

End point timeframe:

Baseline through Week 8

End point values	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3: Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	16	8	18	
Units: percentage of participants				
number (confidence interval 90%)	57.1 (40.0 to 73.1)	57.1 (32.5 to 79.4)	64.3 (47.0 to 79.2)	

Statistical analyses

Statistical analysis title	Group 2: ALXN2050 120 mg BID, Group 3: Placebo
Comparison groups	Group 2: ALXN2050 120 mg BID v Group 3: Placebo
Number of subjects included in analysis	26
Analysis specification	Pre-specified
Analysis type	
P-value	= 0.7341
Method	Barnard's Unconditional Exact Test
Parameter estimate	Difference
Point estimate	-7.1
Confidence interval	
level	90 %
sides	2-sided
lower limit	-34.8
upper limit	19.3

Statistical analysis title	Group 1: ALXN2050 180 mg BID, Group 3: Placebo
Comparison groups	Group 1: ALXN2050 180 mg BID v Group 3: Placebo

Number of subjects included in analysis	34
Analysis specification	Pre-specified
Analysis type	
P-value	= 0.6797
Method	Barnard's Unconditional Exact Test
Parameter estimate	Difference
Point estimate	-7.1
Confidence interval	
level	90 %
sides	2-sided
lower limit	-28.8
upper limit	15

Secondary: Change From Baseline In Quantitative Myasthenia Gravis (QMG) Total Score At Week 8

End point title	Change From Baseline In Quantitative Myasthenia Gravis (QMG) Total Score At Week 8

End point description:

The QMG Score for Disease Severity is an objective evaluation of therapy for MG and is based on quantitative testing of sentinel muscle groups. The QMG instrument consists of 13 items: ocular (2 items), facial (1 item), bulbar (2 items), gross motor (6 items), axial (1 item), and respiratory (1 item); each graded 0 to 3, with 3 being the most severe. The QMG total score was calculated as the sum of the scores of the 13 items and ranges from 0 to 39, with higher scores indicating more severe disease. Baseline score at each timepoint as the response variable, treatment group, study visit, and treatment-by-study visit interaction as fixed categorical effects, and baseline QMG total score as a covariate were used to calculate the least square (LS) mean and the standard error.

End point type	Secondary

End point timeframe:

Baseline, Week 8

End point values	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3: Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	28	13	27	
Units: units on a scale				
least squares mean (standard error)	-1.1 (± 0.67)	-3.0 (± 0.98)	-1.4 (± 0.68)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline In MG-ADL Total Score At Week 8

End point title	Change From Baseline In MG-ADL Total Score At Week 8

End point description:

The MG-ADL profile is an 8-item participant-reported scale that focuses on relevant symptoms and functional performance of ADL in participants with MG. The 8 items of the MG-ADL questionnaire were

derived from symptom-based components of the original 13-item QMG scale to assess disability secondary to ocular (2 items), bulbar (3 items), respiratory (1 item), and gross motor or limb (2 items) impairment related to effects of MG. Each response is graded 0 (normal) to 3 (most severe). The MG-ADL total score was calculated as the sum of the scores of the 8 items and ranges from 0 to 24, with higher scores indicating worse function. Baseline score at each timepoint as the response variable, treatment group, study visit, and treatment-by-study visit interaction as fixed categorical effects, and baseline MG-ADL total score as a covariate were used to calculate the LS mean and the standard error.

End point type	Secondary

End point timeframe:

Baseline, Week 8

End point values	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3: Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	27	14	26	
Units: units on a scale				
least squares mean (standard error)	-2.5 (± 0.50)	-3.7 (± 0.70)	-3.2 (± 0.51)	

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline In Quality Of Life In Neurological Disorders (Neuro-QoL) Fatigue Score At Week 8

End point title	Change From Baseline In Quality Of Life In Neurological Disorders (Neuro-QoL) Fatigue Score At Week 8

End point description:

The Neuro-QoL Fatigue questionnaire is a reliable and validated brief 19-item survey of fatigue, completed by the participant. Each item is scored on a scale of 1–5, with 1 indicating "never" and 5 indicating "sometimes". The Neuro-QoL Fatigue score was calculated as the sum of the scores of the 19 items and ranges from 19–95, with higher scores indicating greater fatigue and greater impact of MG on activities. Baseline score at each timepoint as the response variable, treatment group, study visit, and treatment-by-study visit interaction as fixed categorical effects, and baseline Neuro-QoL Fatigue score as a covariate were used to calculate the LS mean and the standard error.

End point type	Secondary

End point timeframe:

Baseline, Week 8

End point values	Group 1: ALXN2050 180 mg BID	Group 2: ALXN2050 120 mg BID	Group 3: Placebo	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	28	13	27	
Units: units on a scale				
least squares mean (standard error)	-8.7 (± 2.65)	-10.1 (± 3.77)	-8.3 (± 2.73)	

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Baseline (Day 1) up to 30 days after last dose of study intervention (approximately 2 years).	
Adverse event reporting additional description:	
Safety Set: all randomized participants who received at least 1 dose of study intervention.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	26.1

Reporting groups	
Reporting group title	Group 1: ALXN2050 180 mg BID (Primary Evaluation)
Reporting group description:	
Participants received 180 mg ALXN2050 BID during the primary evaluation period.	
Reporting group title	Group 3: Placebo (Primary Evaluation)
Reporting group description:	
Participants received placebo BID during the primary evaluation period.	
Reporting group title	Group 3b: Placebo/ALXN2050 120 mg BID (Ext Treat & OLE)
Reporting group description:	
Participants received placebo BID during the primary evaluation period, followed by ALXN2050 120 mg BID during the extended treatment period and the open-label extension period.	
Reporting group title	Group 2: ALXN2050 120 mg BID (Ext Treat & OLE)
Reporting group description:	
Participants received 120 mg ALXN2050 BID during the extended treatment period and open-label extension period.	
Reporting group title	Group 3a: Placebo/ALXN2050 180 mg BID (Ext Treat & OLE)
Reporting group description:	
Participants received placebo BID during the primary evaluation period, followed by ALXN2050 180 mg BID during the extended treatment period and the open-label extension period.	
Reporting group title	Group 2: ALXN2050 120 mg BID (Primary Evaluation)
Reporting group description:	
Participants received 120 mg ALXN2050 BID during the primary evaluation period.	
Reporting group title	Group 1: ALXN2050 180 mg BID (Ext Treat & OLE)
Reporting group description:	
Participants received 180 mg ALXN2050 BID during the extended treatment period and open-label extension period.	

Serious adverse events	Group 1: ALXN2050 180 mg BID (Primary Evaluation)	Group 3: Placebo (Primary Evaluation)	Group 3b: Placebo/ALXN2050 120 mg BID (Ext Treat & OLE)
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 28 (3.57%)	3 / 28 (10.71%)	2 / 13 (15.38%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0
Vascular disorders			
Hypertension			

subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	0 / 13 (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
Reproductive system and breast disorders			
Cervical dysplasia	Additional description: Adverse event only affected female participants.		
subjects affected / exposed[1]	0 / 18 (0.00%)	0 / 10 (0.00%)	1 / 4 (25.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Respiratory failure			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Acute respiratory failure			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Psychiatric disorders			
Substance-induced psychotic disorder			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Investigations			
Hepatic enzyme increased			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cardiac disorders			

Myocardial infarction			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sinus arrest			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Myasthenia gravis			
subjects affected / exposed	1 / 28 (3.57%)	1 / 28 (3.57%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Diverticulum intestinal haemorrhagic			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Hepatic failure			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	0 / 13 (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
Infections and infestations			
COVID-19			

subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Herpes simplex meningitis			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	0 / 13 (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
Pneumonia aspiration			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Salmonella sepsis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Group 2: ALXN2050 120 mg BID (Ext Treat & OLE)	Group 3a: Placebo/ALXN2050 180 mg BID (Ext Treat & OLE)	Group 2: ALXN2050 120 mg BID (Primary Evaluation)
Total subjects affected by serious adverse events			
subjects affected / exposed	4 / 14 (28.57%)	3 / 13 (23.08%)	1 / 14 (7.14%)
number of deaths (all causes)	0	1	0
number of deaths resulting from adverse events	0	1	0
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Reproductive system and breast disorders			
Cervical dysplasia	Additional description: Adverse event only affected female participants.		
subjects affected / exposed[1]	0 / 10 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory, thoracic and mediastinal disorders			
Respiratory failure			

subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (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
Acute respiratory failure			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Psychiatric disorders			
Substance-induced psychotic disorder			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (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
Investigations			
Hepatic enzyme increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (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
Cardiac disorders			
Myocardial infarction			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sinus arrest			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (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
Nervous system disorders			
Myasthenia gravis			

subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (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
Gastrointestinal disorders			
Diverticulum intestinal haemorrhagic			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (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
Hepatobiliary disorders			
Hepatic failure			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	1 / 1	0 / 0
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
COVID-19			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (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
Herpes simplex meningitis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia aspiration			

subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Salmonella sepsis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (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

Serious adverse events	Group 1: ALXN2050 180 mg BID (Ext Treat & OLE)		
Total subjects affected by serious adverse events			
subjects affected / exposed	4 / 27 (14.81%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Reproductive system and breast disorders			
Cervical dysplasia	Additional description: Adverse event only affected female participants.		
subjects affected / exposed[1]	0 / 17 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Respiratory, thoracic and mediastinal disorders			
Respiratory failure			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Acute respiratory failure			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Psychiatric disorders			
Substance-induced psychotic disorder			

subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Investigations			
Hepatic enzyme increased			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Femur fracture			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
Myocardial infarction			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Sinus arrest			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Myasthenia gravis			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Diverticulum intestinal haemorrhagic			

subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Hepatic failure			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Acute kidney injury			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
COVID-19			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Herpes simplex meningitis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pneumonia aspiration			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Salmonella sepsis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		

Notes:

[1] - The number of subjects exposed to this adverse event is less than the total number of subjects exposed to this adverse event. These numbers are expected to be equal.
Justification: Adverse events affected only female participants.

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

Non-serious adverse events	Group 1: ALXN2050 180 mg BID (Primary Evaluation)	Group 3: Placebo (Primary Evaluation)	Group 3b: Placebo/ALXN2050 120 mg BID (Ext Treat & OLE)
Total subjects affected by non-serious adverse events			
subjects affected / exposed	17 / 28 (60.71%)	15 / 28 (53.57%)	10 / 13 (76.92%)
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	0 / 13 (0.00%)
occurrences (all)	0	1	0
Haematoma			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Spider vein			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Hypotension			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
General disorders and administration site conditions			
Chest pain			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Chest discomfort			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Facial discomfort			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Influenza like illness			
subjects affected / exposed	0 / 28 (0.00%)	2 / 28 (7.14%)	1 / 13 (7.69%)
occurrences (all)	0	2	1
Oedema peripheral			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	1	0	0
Vaccination site erythema			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0

Swelling face			
subjects affected / exposed	0 / 28 (0.00%)	2 / 28 (7.14%)	0 / 13 (0.00%)
occurrences (all)	0	2	0
Pyrexia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Peripheral swelling			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Immune system disorders			
Drug hypersensitivity			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Reproductive system and breast disorders			
Cervical dysplasia	Additional description: Adverse events affected only female participants.		
subjects affected / exposed[2]	0 / 18 (0.00%)	0 / 10 (0.00%)	1 / 4 (25.00%)
occurrences (all)	0	0	1
Benign prostatic hyperplasia	Additional description: Adverse events affected only male participants.		
subjects affected / exposed[3]	0 / 10 (0.00%)	0 / 18 (0.00%)	0 / 9 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			
Dyspnoea			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	1	0	0
Cough			
subjects affected / exposed	0 / 28 (0.00%)	2 / 28 (7.14%)	0 / 13 (0.00%)
occurrences (all)	0	2	0
Psychiatric disorders			
Insomnia			
subjects affected / exposed	2 / 28 (7.14%)	1 / 28 (3.57%)	0 / 13 (0.00%)
occurrences (all)	2	1	0
Investigations			
C-reactive protein increased			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Aspartate aminotransferase increased			

subjects affected / exposed	2 / 28 (7.14%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	2	0	0
Alanine aminotransferase increased			
subjects affected / exposed	2 / 28 (7.14%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	2	0	0
Injury, poisoning and procedural complications			
Ligament sprain			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Fall			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	1	0	0
Contusion			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Wound			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Cardiac disorders			
Extrasystoles			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Sinus node dysfunction			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Supraventricular tachycardia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Arteriosclerosis coronary artery			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Demyelination			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Dizziness			

subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Headache			
subjects affected / exposed	8 / 28 (28.57%)	5 / 28 (17.86%)	1 / 13 (7.69%)
occurrences (all)	12	6	1
Sensory disturbance			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Tremor			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Syncope			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Blood and lymphatic system disorders			
Macrocytosis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Iron deficiency anaemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Leukocytosis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Spontaneous haematoma			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Neutrophilia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Microcytic anaemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Conjunctivitis allergic			

subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Vision blurred			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Gastrointestinal disorders			
Abdominal pain upper			
subjects affected / exposed	2 / 28 (7.14%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	3	0	0
Dry mouth			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Diarrhoea			
subjects affected / exposed	5 / 28 (17.86%)	1 / 28 (3.57%)	1 / 13 (7.69%)
occurrences (all)	5	1	1
Constipation			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Dyspepsia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Vomiting			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	3 / 28 (10.71%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	3	0	1
Hypoaesthesia oral			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Gastrooesophageal reflux disease			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	1	0	1
Faeces discoloured			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1

Hepatobiliary disorders			
Cholelithiasis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Skin and subcutaneous tissue disorders			
Skin ulcer			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Pruritus			
subjects affected / exposed	0 / 28 (0.00%)	2 / 28 (7.14%)	0 / 13 (0.00%)
occurrences (all)	0	2	0
Hyperhidrosis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Renal and urinary disorders			
Renal impairment			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Renal colic			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Ketonuria			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Cystitis haemorrhagic			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Endocrine disorders			
Thyroid cyst			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Spinal stenosis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Pain in jaw			

subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Pain in extremity			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Osteoarthritis			
subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	1 / 13 (7.69%)
occurrences (all)	0	1	1
Musculoskeletal chest pain			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Muscle spasms			
subjects affected / exposed	1 / 28 (3.57%)	1 / 28 (3.57%)	0 / 13 (0.00%)
occurrences (all)	1	1	0
Limb discomfort			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Back pain			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	1	0	0
Arthralgia			
subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	0 / 13 (0.00%)
occurrences (all)	0	1	0
Infections and infestations			
Bronchitis			
subjects affected / exposed	1 / 28 (3.57%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	1	0	1
COVID-19			
subjects affected / exposed	2 / 28 (7.14%)	1 / 28 (3.57%)	2 / 13 (15.38%)
occurrences (all)	2	1	2
Conjunctivitis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	1
Cystitis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0

Gastroenteritis viral			
subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	1 / 13 (7.69%)
occurrences (all)	0	1	1
Influenza			
subjects affected / exposed	2 / 28 (7.14%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	2	0	0
Nasopharyngitis			
subjects affected / exposed	0 / 28 (0.00%)	3 / 28 (10.71%)	0 / 13 (0.00%)
occurrences (all)	0	3	0
Oral candidiasis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Respiratory tract infection			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Upper respiratory tract infection			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	2
Urinary tract infection			
subjects affected / exposed	0 / 28 (0.00%)	1 / 28 (3.57%)	1 / 13 (7.69%)
occurrences (all)	0	1	1
Viral infection			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	1 / 13 (7.69%)
occurrences (all)	0	0	2
Viral upper respiratory tract infection			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Metabolism and nutrition disorders			
Hypertriglyceridaemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Hypernatraemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Vitamin B12 deficiency			

subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0
Type 2 diabetes mellitus			
subjects affected / exposed	0 / 28 (0.00%)	0 / 28 (0.00%)	0 / 13 (0.00%)
occurrences (all)	0	0	0

Non-serious adverse events	Group 2: ALXN2050 120 mg BID (Ext Treat & OLE)	Group 3a: Placebo/ALXN2050 180 mg BID (Ext Treat & OLE)	Group 2: ALXN2050 120 mg BID (Primary Evaluation)
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 14 (50.00%)	9 / 13 (69.23%)	7 / 14 (50.00%)
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 14 (7.14%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	1	1	0
Haematoma			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Spider vein			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Hypotension			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Chest pain			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Chest discomfort			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Facial discomfort			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Influenza like illness			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0

Oedema peripheral			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Vaccination site erythema			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Swelling face			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Pyrexia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Peripheral swelling			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Immune system disorders			
Drug hypersensitivity			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Reproductive system and breast disorders			
Cervical dysplasia	Additional description: Adverse events affected only female participants.		
subjects affected / exposed[2]	0 / 10 (0.00%)	0 / 5 (0.00%)	0 / 10 (0.00%)
occurrences (all)	0	0	0
Benign prostatic hyperplasia	Additional description: Adverse events affected only male participants.		
subjects affected / exposed[3]	0 / 4 (0.00%)	0 / 8 (0.00%)	1 / 4 (25.00%)
occurrences (all)	0	0	1
Respiratory, thoracic and mediastinal disorders			
Dyspnoea			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Cough			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Psychiatric disorders			
Insomnia			

subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Investigations			
C-reactive protein increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Aspartate aminotransferase increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Alanine aminotransferase increased			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Ligament sprain			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Fall			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Contusion			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Wound			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Cardiac disorders			
Extrasystoles			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Sinus node dysfunction			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Supraventricular tachycardia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Arteriosclerosis coronary artery			

subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Nervous system disorders			
Demyelination			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Dizziness			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Headache			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Sensory disturbance			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	3	0	1
Tremor			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Syncope			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Macrocytosis			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Iron deficiency anaemia			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Leukocytosis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Spontaneous haematoma			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Neutrophilia			

subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Microcytic anaemia			
subjects affected / exposed	1 / 14 (7.14%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	1	1	0
Eye disorders			
Conjunctivitis allergic			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Vision blurred			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Gastrointestinal disorders			
Abdominal pain upper			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Dry mouth			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	2	0	1
Constipation			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Dyspepsia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Vomiting			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Hypoaesthesia oral			

subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Gastrooesophageal reflux disease			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Faeces discoloured			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Hepatobiliary disorders			
Cholelithiasis			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Skin and subcutaneous tissue disorders			
Skin ulcer			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Pruritus			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Hyperhidrosis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Renal and urinary disorders			
Renal impairment			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Renal colic			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Ketonuria			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	2
Cystitis haemorrhagic			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Endocrine disorders			

Thyroid cyst			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Musculoskeletal and connective tissue disorders			
Spinal stenosis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Pain in jaw			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Pain in extremity			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	2
Osteoarthritis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Musculoskeletal chest pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Muscle spasms			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Limb discomfort			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Back pain			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Arthralgia			
subjects affected / exposed	2 / 14 (14.29%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	2	0	0
Infections and infestations			
Bronchitis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
COVID-19			

subjects affected / exposed	1 / 14 (7.14%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	1	1	0
Conjunctivitis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Cystitis			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	1	0	0
Gastroenteritis viral			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Influenza			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Nasopharyngitis			
subjects affected / exposed	0 / 14 (0.00%)	2 / 13 (15.38%)	0 / 14 (0.00%)
occurrences (all)	0	3	0
Oral candidiasis			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Respiratory tract infection			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Upper respiratory tract infection			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Urinary tract infection			
subjects affected / exposed	1 / 14 (7.14%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	2	0	0
Viral infection			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	0 / 14 (0.00%)
occurrences (all)	0	0	0
Viral upper respiratory tract infection			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Metabolism and nutrition disorders			

Hypertriglyceridaemia			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Hypernatraemia			
subjects affected / exposed	0 / 14 (0.00%)	0 / 13 (0.00%)	1 / 14 (7.14%)
occurrences (all)	0	0	1
Vitamin B12 deficiency			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0
Type 2 diabetes mellitus			
subjects affected / exposed	0 / 14 (0.00%)	1 / 13 (7.69%)	0 / 14 (0.00%)
occurrences (all)	0	1	0

Non-serious adverse events	Group 1: ALXN2050 180 mg BID (Ext Treat & OLE)		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	17 / 27 (62.96%)		
Vascular disorders			
Hypertension			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Haematoma			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Spider vein			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Hypotension			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
General disorders and administration site conditions			
Chest pain			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Chest discomfort			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		

Facial discomfort			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Influenza like illness			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Oedema peripheral			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Vaccination site erythema			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Swelling face			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Pyrexia			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Peripheral swelling			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Immune system disorders			
Drug hypersensitivity			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Reproductive system and breast disorders			
Cervical dysplasia	Additional description: Adverse events affected only female participants.		
subjects affected / exposed[2]	0 / 17 (0.00%)		
occurrences (all)	0		
Benign prostatic hyperplasia	Additional description: Adverse events affected only male participants.		
subjects affected / exposed[3]	0 / 10 (0.00%)		
occurrences (all)	0		
Respiratory, thoracic and mediastinal disorders			
Dyspnoea			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		

Cough			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Psychiatric disorders			
Insomnia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Investigations			
C-reactive protein increased			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Aspartate aminotransferase increased			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Alanine aminotransferase increased			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Injury, poisoning and procedural complications			
Ligament sprain			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Fall			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Contusion			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Wound			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Cardiac disorders			
Extrasystoles			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Sinus node dysfunction			

subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Supraventricular tachycardia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Arteriosclerosis coronary artery			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Nervous system disorders			
Demyelination			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Dizziness			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Headache			
subjects affected / exposed	4 / 27 (14.81%)		
occurrences (all)	5		
Sensory disturbance			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Tremor			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Syncope			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Blood and lymphatic system disorders			
Macrocytosis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Iron deficiency anaemia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Leukocytosis			

subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Spontaneous haematoma			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Neutrophilia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Microcytic anaemia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Eye disorders			
Conjunctivitis allergic			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Vision blurred			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Gastrointestinal disorders			
Abdominal pain upper			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Dry mouth			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Diarrhoea			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Constipation			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Dyspepsia			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Vomiting			

subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Nausea			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Hypoaesthesia oral			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Gastrooesophageal reflux disease			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Faeces discoloured			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Hepatobiliary disorders			
Cholelithiasis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Skin and subcutaneous tissue disorders			
Skin ulcer			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Pruritus			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Hyperhidrosis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Renal and urinary disorders			
Renal impairment			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Renal colic			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Ketonuria			

subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Cystitis haemorrhagic			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Endocrine disorders			
Thyroid cyst			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Musculoskeletal and connective tissue disorders			
Spinal stenosis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Pain in jaw			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Pain in extremity			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Osteoarthritis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Musculoskeletal chest pain			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Muscle spasms			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Limb discomfort			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Back pain			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Arthralgia			

subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Infections and infestations			
Bronchitis			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	2		
COVID-19			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Conjunctivitis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Cystitis			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Gastroenteritis viral			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Influenza			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		
Nasopharyngitis			
subjects affected / exposed	0 / 27 (0.00%)		
occurrences (all)	0		
Oral candidiasis			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Respiratory tract infection			
subjects affected / exposed	2 / 27 (7.41%)		
occurrences (all)	2		
Upper respiratory tract infection			
subjects affected / exposed	5 / 27 (18.52%)		
occurrences (all)	6		
Urinary tract infection			
subjects affected / exposed	1 / 27 (3.70%)		
occurrences (all)	1		

Viral infection subjects affected / exposed occurrences (all) Viral upper respiratory tract infection subjects affected / exposed occurrences (all)	0 / 27 (0.00%) 0 2 / 27 (7.41%) 2		
Metabolism and nutrition disorders Hypertriglyceridaemia subjects affected / exposed occurrences (all) Hypernatraemia subjects affected / exposed occurrences (all) Vitamin B12 deficiency subjects affected / exposed occurrences (all) Type 2 diabetes mellitus subjects affected / exposed occurrences (all)	 1 / 27 (3.70%) 1 0 / 27 (0.00%) 0 0 / 27 (0.00%) 0 0 / 27 (0.00%) 0		

Notes:

[2] - The number of subjects exposed to this adverse event is less than the total number of subjects exposed for the reporting group. These numbers are expected to be equal.
Justification: Adverse events affected only female participants.

[3] - The number of subjects exposed to this adverse event is less than the total number of subjects exposed for the reporting group. These numbers are expected to be equal.
Justification: Adverse events affected only male participants.

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
22 July 2021	Updated the list of exclusion criteria with a renal impairment criterion and indicated that medications known to significantly prolong the corrected QT interval were not allowed during the study.
09 March 2023	Addressed the requirements for conducting a clinical study under the EU Clinical Trials Register (EU CTR).
15 November 2023	Addressed the requirements for transitioning a clinical study under the EU CTR.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

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

This study did not meet its primary efficacy end point and was early terminated by the Sponsor.

Notes: