Clinical trial results:
A Phase 2, Randomized, Placebo-Controlled, Parallel-Group, Double-Blinded, Proof-of-Concept Study to Evaluate the Safety and Efficacy of Intravenous Efgartigimod in Adult Participants With Primary Sjögren's Syndrome

Summary

EudraCT number	2021-005911-30
Trial protocol	NL BE HU
Global end of trial date	12 February 2024

Results information

Result version number	v1 (current)
This version publication date	26 February 2025
First version publication date	26 February 2025

Trial information

Trial identification

Sponsor protocol code	ARGX-113-2106

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05817669
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	argenx BV
Sponsor organisation address	Zwijnaarde 7, Zwijnaarde (Ghent), Belgium, 9052
Public contact	Regulatory, argenx, regulatory@argenx.com
Scientific contact	Regulatory, argenx, regulatory@argenx.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	08 January 2025
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	12 February 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the effect of efgartigimod intravenous (IV) compared to placebo on composite of relevant endpoints for Sjögren's syndrome (CRESS).

Protection of trial subjects:

This study was conducted in accordance with the protocol and consensus ethical principles derived from international guidelines, including the Declaration of Helsinki, applicable ICH GCP guidelines, and applicable laws and regulations. The participant's informed consent was documented by the dated signature of the participant (and assent, if applicable) and the dated signature of the investigator or investigator's delegate.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	08 May 2023
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Belgium: 8
Country: Number of subjects enrolled	Hungary: 5
Country: Number of subjects enrolled	Poland: 21
Worldwide total number of subjects	34
EEA total number of subjects	34

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)	26

From 65 to 84 years	8
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This Phase 2, double-blinded study was conducted in adult participants with primary Sjögren's disease (pSjD) at 15 investigational sites in 3 countries between 08-May-2023 and 12-Feb-2024.

Pre-assignment

Screening details:

A total of 34 participants were enrolled in study. Participants were randomized in a 2:1 ratio to either receive efgartigimod or placebo for 24 weeks. At end of treatment period, eligible participants rolled over to an open-label extension (OLE) study (ARGX-113-2211 [2023-503915-14]) or remained in the study for the post-treatment follow-up period.

Period 1

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

Arms

Are arms mutually exclusive?	Yes
Arm title	Efgartigimod

Arm description:

Participants received efgartigimod 10 milligram per kilogram (mg/kg) once weekly via IV infusion for up to 24 weeks.

Arm type	Experimental
Investigational medicinal product name	Efgartigimod
Investigational medicinal product code	ARGX-113
Other name	
Pharmaceutical forms	Concentrate for solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Participants received efgartigimod 10 mg/kg once weekly via IV infusion for up to 24 weeks.

Arm title	Placebo

Arm description:

Participants received placebo matched to efgartigimod once weekly via IV infusion for up to 24 weeks.

Arm type	Placebo
Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate for solution for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Participants received placebo matched to efgartigimod once weekly via IV infusion for up to 24 weeks.

Number of subjects in period 1	Efgartigimod	Placebo
Started	23	11
Completed	20	7
Not completed	3	4
Consent withdrawn by subject	1	2
Adverse event, non-fatal	1	-
Prohibited medication used	1	2

Baseline characteristics

Reporting groups	
Reporting group title	Efgartigimod
Reporting group description:	
Participants received efgartigimod 10 milligram per kilogram (mg/kg) once weekly via IV infusion for up to 24 weeks.	
Reporting group title	Placebo
Reporting group description:	
Participants received placebo matched to efgartigimod once weekly via IV infusion for up to 24 weeks.	

Reporting group values	Efgartigimod	Placebo	Total
Number of subjects	23	11	34
Age categorical Units: Subjects			
Age continuous Units: years			
arithmetic mean	50.1	54.7	
standard deviation	± 12.57	± 12.42	-
Gender categorical Units: Subjects			
Female	22	11	33
Male	1	0	1
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	0	0	0
Not Hispanic or Latino	22	11	33
Unknown or Not Reported	1	0	1
Race/Ethnicity, Customized Units: Subjects			
White	22	11	33
Unknown	1	0	1

End points

End points reporting groups

Reporting group title	Efgartigimod
Reporting group description:	
Participants received efgartigimod 10 milligram per kilogram (mg/kg) once weekly via IV infusion for up to 24 weeks.	
Reporting group title	Placebo
Reporting group description:	
Participants received placebo matched to efgartigimod once weekly via IV infusion for up to 24 weeks.	
Subject analysis set title	Efgartigimod (EAS)
Subject analysis set type	Per protocol
Subject analysis set description:	
Efficacy Analysis Set: Participants received efgartigimod 10 mg/kg once weekly via IV infusion for 24 weeks.	
Subject analysis set title	Placebo (EAS)
Subject analysis set type	Per protocol
Subject analysis set description:	
Efficacy Analysis Set: Participants received placebo matched to efgartigimod once weekly via IV infusion for 24 weeks.	
Subject analysis set title	Efgartigimod (SAF)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Safety Analysis Set: Participants received efgartigimod 10 mg/kg once weekly via IV infusion for 24 weeks.	
Subject analysis set title	Placebo (SAF)
Subject analysis set type	Safety analysis
Subject analysis set description:	
Safety Analysis Set: Participants received placebo matched to efgartigimod once weekly via IV infusion for 24 weeks.	
Subject analysis set title	Efgartigimod (PKAS)
Subject analysis set type	Per protocol
Subject analysis set description:	
Pharmacokinetic Analysis Set: Participants received efgartigimod 10 mg/kg once weekly via IV infusion for 24 weeks.	

Primary: Percentage of Participants Meeting Overall CRESS Response on ≥3 of 5 Items at Week 24

End point title	Percentage of Participants Meeting Overall CRESS Response on ≥3 of 5 Items at Week 24[1]
End point description:	
A Composite of Relevant Endpoints for Sjögren's Syndrome (CRESS) responder is defined as improvements in at least 3 of the 5 items of CRESS (systemic disease activity, patient-reported symptoms, tear gland function, salivary gland function and serology. The score ranges from 0 to 9 (higher score = worse symptoms).	
End point type	Primary
End point timeframe:	
Week 24	

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: As the endpoint was descriptive in nature, no statistical analysis was reported.

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	45.5 (26.92 to 65.34)	11.1 (1.99 to 43.50)		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants With TEAEs, AESI and SAEs

End point title	Number of Participants With TEAEs, AESI and SAEs

End point description:

A treatment-emergent adverse event (TEAE): adverse events reported from the first dose up to and including 60 days after the final dose were considered treatment-emergent. Serious adverse event (SAE): adverse event that resulted in death, was life threatening, required inpatient hospitalization or prolongation of existing hospitalization, resulted in persistent or significant disability/incapacity, was a congenital anomaly/birth defect or other situations. Adverse event of special interest (AESI): adverse event related to 'Infections and infestations'.

End point type	Secondary

End point timeframe:

Up to 32 weeks

End point values	Efgartigimod (SAF)	Placebo (SAF)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	23	11		
Units: Participants				
Any TEAE	20	7		
Any AESI	15	5		
Any SAE	1	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants With MCII in ESSDAI at Week 24

End point title	Percentage of Participants With MCII in ESSDAI at Week 24

End point description:

European Alliance of Associations for Rheumatology (EULAR) Sjögren’s Syndrome Disease Activity Index (ESSDAI) measures systemic disease activity in participants with pSjD and consists of 11 organ-specific domains and 1 biological domain that contribute to disease activity level scoring. Each domain is given a certain weight, which gives a score between 0 and 123 (higher score =worse symptoms). Minimally clinically important improvement (MCII) in ESSDAI was defined as improvement of at least 3 points in ESSDAI score at Week 24.

End point type	Secondary

End point timeframe:

Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	72.7 (51.85 to 86.85)	55.6 (26.67 to 81.12)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants With Low Disease Activity in ESSDAI at Week 24

End point title	Percentage of Participants With Low Disease Activity in ESSDAI at Week 24

End point description:

ESSDAI measures systemic disease activity in participants with pSjD and consists of 11 organ-specific domains and 1 biological domain that contribute to disease activity level scoring. Each domain is given a certain weight, which gives a score between 0 and 123 (higher score =worse symptoms). Low disease activity in ESSDAI was defined as ESSDAI score of less than 5 at Week 24.

End point type	Secondary

End point timeframe:

Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	59.1 (38.73 to 76.74)	22.2 (6.32 to 54.74)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants With MCII in clinESSDAI at Week 24

End point title	Percentage of Participants With MCII in clinESSDAI at Week 24

End point description:

Clinical (clin)ESSDAI includes the same 11 organ-specific domains as ESSDAI but with different domain weighting and without the biological domain. This way any change in clinESSDAI score would reflect disease specific features, irrespective of B-cell activity. The clinESSDAI score ranges between 0-135 (higher score = worse symptoms). Minimal clinically important improvement (MCII) in clinESSDAI was defined as improvement of at least 3 points in clinESSDAI score at Week 24.

End point type	Secondary

End point timeframe:

Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	77.3 (56.56 to 89.88)	77.8 (45.26 to 93.68)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants With Low Disease Activity in clinESSDAI at Week 24

End point title	Percentage of Participants With Low Disease Activity in clinESSDAI at Week 24

End point description:

clinESSDAI includes the same 11 organ-specific domains as ESSDAI but with different domain weighting and without the biological domain. This way any change in clinESSDAI score would reflect disease specific features, irrespective of B-cell activity. The clinESSDAI score ranges between 0-135 (higher score = worse symptoms). Low disease activity in clinESSDAI was defined as clinESSDAI score of less than 5 at Week 24.

End point type	Secondary

End point timeframe:

Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	59.1 (38.73 to 76.74)	33.3 (12.06 to 64.58)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants With MCII in ESSPRI at Week 24

End point title	Percentage of Participants With MCII in ESSPRI at Week 24

End point description:

Minimal clinically important improvement in ESSPRI was defined as decrease of 1 point or at least ≥15% at Week 24. ESSPRI is a questionnaire that has been developed to measure self-reported symptoms in participants with pSjD. The score ranges from 0 (no symptoms) to 10 (more symptoms).

End point type	Secondary

End point timeframe:

Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	31.8 (16.36 to 52.68)	33.3 (12.06 to 64.58)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in ESSDAI Score at Week 24

End point title	Change From Baseline in ESSDAI Score at Week 24

End point description:

ESSDAI measures systemic disease activity in participants with pSjD and consists of 11 organ-specific domains and 1 biological domain that contribute to disease activity level scoring. Each domain is given a certain weight, which gives a score between 0 and 123 (higher score = worse symptoms).

End point type	Secondary

End point timeframe:

Baseline (Day 1) and Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	19	7		
Units: score on a scale				
median (inter-quartile range (Q1-Q3))	-5.0 (-10.0 to -4.0)	-4.0 (-13.0 to -1.0)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in clinESSDAI Score at Week 24

End point title	Change From Baseline in clinESSDAI Score at Week 24

End point description:

clinESSDAI includes the same 11 organ-specific domains as ESSDAI but with different domain weighting and without the biological domain. This way any change in clinESSDAI score would reflect disease specific features, irrespective of B-cell activity. The clinESSDAI score ranges between 0-135 (higher score = worse symptoms).

End point type	Secondary

End point timeframe:

Baseline (Day 1) and Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	19	7		
Units: score on a scale				
median (inter-quartile range (Q1-Q3))	-7.0 (-12.0 to -3.0)	-4.0 (-17.0 to -3.0)		

Statistical analyses

No statistical analyses for this end point

Secondary: Change From Baseline in ESSPRI Score at Week 24

End point title	Change From Baseline in ESSPRI Score at Week 24

End point description:

ESSPRI is a questionnaire that has been developed to measure self-reported symptoms in participants with pSjD. The score ranges from 0 (no symptoms) to 10 (more symptoms).

End point type	Secondary

End point timeframe:

Baseline (Day 1) and Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	16	6		
Units: score on a scale				
median (inter-quartile range (Q1-Q3))	-0.500 (-1.333 to 0.333)	-0.833 (-1.333 to -0.333)		

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage of Participants With STAR Score of at Least 5 at Week 24

End point title	Percentage of Participants With STAR Score of at Least 5 at Week 24

End point description:

Sjögren's Tool for Assessing Response (STAR) is a composite endpoint assessing multiple clinically relevant disease features. A STAR responder is defined as a score of at least 5 points. Due to the weighting, participants must be a responder on either systemic disease activity (ESSDAI), patient-reported symptoms (ESSPRI), or both to be an overall STAR responder. The score ranges between 0 and 9 (higher score = worse outcome).

End point type	Secondary

End point timeframe:

Week 24

End point values	Efgartigimod (EAS)	Placebo (EAS)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	22	9		
Units: Percentage of participants				
number (confidence interval 95%)	54.5 (34.66 to 73.08)	33.3 (12.06 to 64.58)		

Statistical analyses

No statistical analyses for this end point

Secondary: Plasma Concentration of Efgartigimod

End point title	Plasma Concentration of Efgartigimod

End point description:

Serum samples were collected at indicated time points to assess the pharmacokinetic (PK) profile of efgartigimod. PK population included all randomized participants who received at least 1 dose of efgartigimod and had at least 1 measured concentration of efgartigimod at a scheduled PK time point. Here, n= number of participants with data collected for each specified category. "9999" indicates that mean and standard deviation could not be calculated as the values were below the lower limit of quantification (LLOQ). LLOQ was 200 ng/mL.

End point type	Secondary

End point timeframe:

Pre-dose at Day 1, Weeks 1, 2, 4, 8, 12, 16, and 20; 30 minutes post-dose at Day 1, Weeks 1, 2, 4, 8, 12, 16, 20 and 24

End point values	Efgartigimod (PKAS)			
Subject group type	Subject analysis set			
Number of subjects analysed	23			
Units: Nanograms per milliliter (ng/mL)				
arithmetic mean (standard deviation)				
Day 1: Pre-dose (n=23)	9999 (± 9999)			
Day 1: 30 minutes post-dose (n=23)	179391 (± 79751)			
Week 1: Pre-dose (n=22)	17238 (± 37356)			
Week 1: 30 minutes post-dose (n=23)	187300 (± 73091)			
Week 2: Pre-dose (n=22)	22470 (± 42838)			
Week 2: 30 minutes post-dose (n=21)	205148 (± 52448)			
Week 4: Pre-dose (n=16)	38595 (± 65900)			
Week 4: 30 minutes post-dose (n=16)	206414 (± 102089)			
Week 8: Pre-dose (n=19)	14193 (± 5753)			
Week 8: 30 minutes post-dose (n=19)	203505 (± 89762)			
Week 12: Pre-dose (n=18)	13096 (± 4523)			
Week 12: 30 minutes post-dose (n=18)	200856 (± 138766)			
Week 16: Pre-dose (n=19)	12676 (± 5835)			
Week 16: 30 minutes post-dose (n=19)	261647 (± 215940)			
Week 20: Pre-dose (n=16)	12942 (± 4472)			
Week 20: 30 minutes post-dose (n=16)	194944 (± 81722)			
Week 24: 30 minutes post-dose (n=15)	14284 (± 4987)			

Statistical analyses

No statistical analyses for this end point

Secondary: Percentage Change From Baseline in Total IgG Levels in Serum at Week 24

End point title	Percentage Change From Baseline in Total IgG Levels in Serum at Week 24

End point description:

Blood samples were collected at indicated timepoints to assess the total Immunoglobulin (Ig)G levels in serum. Only participants with data available at baseline and Week 24 are reported.

End point type	Secondary

End point timeframe:

Baseline (Day 1) and Week 24

End point values	Efgartigimod (SAF)	Placebo (SAF)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	20	7		
Units: Percentage change				
arithmetic mean (standard deviation)	-57.172 (± 17.7376)	0.279 (± 9.5193)		

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants With ADA Against Efgartigimod in Serum

End point title	Number of Participants With ADA Against Efgartigimod in Serum

End point description:

Blood samples were collected to assess anti-drug antibodies (ADAs) against efgartigimod. Treatment-boosted ADA was defined as participants who had a baseline positive sample and the titer value increased 4-fold or more compared to baseline. Treatment-induced ADA was defined as participants who had a baseline negative sample and at least 1 positive post-baseline samples. Treatment-unaffected ADA was defined as participants who had a baseline positive sample, but the titer value did not increase 4-fold or more compared to baseline.

End point type	Secondary

End point timeframe:

Up to Week 24

End point values	Efgartigimod (SAF)	Placebo (SAF)		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	23	11		
Units: Participants				
Treatment-boosted ADA	1	0		
Treatment-induced ADA	2	0		
Treatment-unaffected ADA	11	3		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Up to 32 weeks.	
Adverse event reporting additional description:	
The SAF included all participants exposed to the study treatment. Any abnormal laboratory results or changes in vital signs are captured in the adverse event listings.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	26.0

Reporting groups	
Reporting group title	Efgartigimod (SAF)
Reporting group description:	
Safety Analysis Set: Participants received efgartigimod 10 mg/kg once weekly via IV infusion for 24 weeks	
Reporting group title	Placebo (SAF)
Reporting group description:	
Safety Analysis Set: Participants received placebo matched to efgartigimod once weekly via IV infusion for 24 weeks.	

Serious adverse events	Efgartigimod (SAF)	Placebo (SAF)	
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 23 (4.35%)	0 / 11 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	
Vascular disorders			
Vasospasm			
subjects affected / exposed	1 / 23 (4.35%)	0 / 11 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	Efgartigimod (SAF)	Placebo (SAF)	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	20 / 23 (86.96%)	7 / 11 (63.64%)	
Injury, poisoning and procedural complications			

Arthropod sting			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Cardiac disorders			
Palpitations			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Nervous system disorders			
Dizziness			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	3	0	
Headache			
subjects affected / exposed	4 / 23 (17.39%)	1 / 11 (9.09%)	
occurrences (all)	6	1	
Syncope			
subjects affected / exposed	1 / 23 (4.35%)	1 / 11 (9.09%)	
occurrences (all)	1	1	
Blood and lymphatic system disorders			
Iron deficiency anaemia			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Malaise			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	3	0	
Ear and labyrinth disorders			
Motion sickness			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Gastrointestinal disorders			
Abdominal pain			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Abdominal pain lower			

subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Diarrhoea			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Peptic ulcer			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Vomiting			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Reproductive system and breast disorders			
Ovarian cyst			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	3	0	
Oropharyngeal pain			
subjects affected / exposed	2 / 23 (8.70%)	1 / 11 (9.09%)	
occurrences (all)	2	1	
Skin and subcutaneous tissue disorders			
Erythema			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Purpura			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Sjogren's syndrome			

subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Infections and infestations			
COVID-19			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Conjunctivitis			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Cystitis			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Gastroenteritis			
subjects affected / exposed	2 / 23 (8.70%)	0 / 11 (0.00%)	
occurrences (all)	2	0	
Gastrointestinal infection			
subjects affected / exposed	0 / 23 (0.00%)	1 / 11 (9.09%)	
occurrences (all)	0	1	
Influenza			
subjects affected / exposed	3 / 23 (13.04%)	0 / 11 (0.00%)	
occurrences (all)	3	0	
Nasopharyngitis			
subjects affected / exposed	4 / 23 (17.39%)	1 / 11 (9.09%)	
occurrences (all)	5	1	
Upper respiratory tract infection			
subjects affected / exposed	3 / 23 (13.04%)	2 / 11 (18.18%)	
occurrences (all)	3	2	
Urinary tract infection			
subjects affected / exposed	3 / 23 (13.04%)	1 / 11 (9.09%)	
occurrences (all)	3	1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
05 December 2022	The primary rationale for this amendment was to permit the use of historic biopsy where taken within 12 months of enrollment to prevent participants from needing to undergo unnecessary procedures, and to update the permitted/excluded concomitant medications to allow participants to receive required therapy. Inclusion and exclusion were updated. Other clarifications and corrections were made. Minor editorial changes, including the correction of typographical errors and formatting inconsistencies were done.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported