Clinical trial results:
A Long-term, Single-Arm, Open-label, Multicenter Phase 3 Study to Evaluate the Safety and Tolerability of Multiple Subcutaneous Injections of Efgartigimod PH20 SC in Patients With Generalized Myasthenia Gravis

Summary

EudraCT number	2020-004086-38
Trial protocol	DE HU CZ BE NL IT ES
Global end of trial date	31 December 2024

Results information

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

Trial information

Trial identification

Sponsor protocol code	ARGX-113-2002

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04818671
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	argenx BV
Sponsor organisation address	Industriepark Zwijnaarde 7, Zwijnaarde (Ghent), Belgium, 9052
Public contact	Regulatory, argenx BV, 0032 93103400, regulatory@argenx.com
Scientific contact	Regulatory, argenx BV, 0032 93103400, 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	05 June 2025
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	31 December 2024
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the long-term safety and tolerability of efgartigimod PH20 SC in participants with Generalized Myasthenia Gravis (gMG)

Protection of trial subjects:

The protocol, protocol amendments, ICFs and participant recruitment information were approved by the IRB, IEC and regulatory agency before participants were enrolled. 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. Participants or their legally authorized representative were required to sign a statement of informed consent that met the requirements of the local regulations, ICH guidelines, privacy and data protection requirements, where applicable, and the IRB/IEC or study site.

Background therapy:

Participants must have been receiving a stable dose of concomitant gMG therapy at study entry. During year 1 of the study, participants could reduce their dose of steroid, NSID, or AChE inhibitor after week 4 of a cycle and before the next cycle. Changes to concomitant gMG therapy were unrestricted from year 2 onward and based on the discretion of the investigator.
The medications and vaccinations allowed or prohibited during and/or after efgartigimod PH20 SC administration are described in the protocol.

Evidence for comparator:

Not applicable

Actual start date of recruitment	05 April 2021
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Georgia: 33
Country: Number of subjects enrolled	Japan: 16
Country: Number of subjects enrolled	Russian Federation: 7
Country: Number of subjects enrolled	United States: 33
Country: Number of subjects enrolled	Netherlands: 3
Country: Number of subjects enrolled	Poland: 60
Country: Number of subjects enrolled	Spain: 3
Country: Number of subjects enrolled	Belgium: 6
Country: Number of subjects enrolled	Czechia: 3
Country: Number of subjects enrolled	Germany: 4
Country: Number of subjects enrolled	Hungary: 4
Country: Number of subjects enrolled	Italy: 8

Worldwide total number of subjects	180
EEA total number of subjects	91

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)	139
From 65 to 84 years	41
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This study was conducted at 47 sites that enrolled participants in 12 countries. A total of 184 participants rolled over from ARGX-113-2001 and ARGX-113-1705, of whom 180 participants were exposed to efgartigimod PH20 SC treatment.

Pre-assignment

Screening details:

All participants were adults with gMG who participated in ARGX-113-2001 or ARGX-113-1705. No randomization or blinding was performed because this was an open-label study.

Period 1

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

Blinding implementation details:

This is an open-label study.

Arms

Arm title	Efgartigimod PH20 SC

Arm description:

All participants who were exposed to efgartigimod PH20 SC in this study.

Arm type	Experimental
Investigational medicinal product name	Efgartigimod PH20 SC
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Efgartigimod PH20 SC 1000 mg was administered in cycles. Each cycle comprising a treatment period (TP) and an intertreatment period (IP). 4 once-weekly injections of efgartigimod PH20 SC were administrated over 3 weeks during the TP. The duration of the IP was based on clinical evaluation with a minimum of 28 days in year 1 and 7 days in year 2.
Once considered capable for (self-)administration, participants and caregivers could perform SC injections at home except for the first administration in a cycle’s TP.

Number of subjects in period 1	Efgartigimod PH20 SC
Started	180
Completed	138
Not completed	42
Physician decision	4
Consent withdrawn by subject	16
Adverse event, non-fatal	5
Death	4
Requires prohibited medication	4

Sponsor request	1
Approved drug available for indication	1
Lost to follow-up	2
Lack of efficacy	5

Baseline characteristics

Reporting groups	
Reporting group title	Efgartigimod PH20 SC
Reporting group description:	
All participants who were exposed to efgartigimod PH20 SC in this study.	

Reporting group values	Efgartigimod PH20 SC	Total	
Number of subjects	180	180	
Age categorical Units: Subjects			
Adults (18-64 years)	139	139	
From 65-84 years	41	41	
Age continuous Units: years			
arithmetic mean	50.8		
standard deviation	± 15.50	-	
Gender categorical Units: Subjects			
Female	119	119	
Male	61	61	
Race Units: Subjects			
Asian (Japanese)	16	16	
Black or African American	2	2	
White	161	161	
Multiple	1	1	
Ethnicity Units: Subjects			
Hispanic or Latino	8	8	
Not Hispanic or Latino	172	172	

End points

End points reporting groups

Reporting group title	Efgartigimod PH20 SC

Reporting group description:

All participants who were exposed to efgartigimod PH20 SC in this study.

Primary: Number of AEs, SAEs and AESIs

End point title	Number of AEs, SAEs and AESIs[1]

End point description:

Adverse events, Serious Adverse event and Adverse events of special interest. Adverse events in the 'Infections and infestations' SOC were defined as AESIs because efgartigimod causes a transient reduction in total IgG levels.

End point type	Primary

End point timeframe:

Up to 3.5 years

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: Since this is an open-label extension study, inferential statistics are not applicable.

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	180			
Units: events				
Number of AEs	3325			
Number of SAEs	107			
Number of AESIs	420			

Statistical analyses

No statistical analyses for this end point

Secondary: MG-ADL total score changes from baseline

End point title	MG-ADL total score changes from baseline

End point description:

The Myasthenia Gravis Activities of Daily Living (MG-ADL) is an 8-item patient-reported scale that assesses MG symptoms and their effects on daily activities. It evaluates a participant’s capacity to perform different activities in their daily life. The total score ranges from 0 to 24 with higher scores indicating more impairment.

End point type	Secondary

End point timeframe:

Up to week 4 of the first cycle

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	180			
Units: score on scale				
arithmetic mean (standard error)				
Cycle 1, Week 1 (n=180)	-2.2 (± 0.20)			
Cycle 1, Week 2 (n=176)	-3.4 (± 0.21)			
Cycle 1, Week 3 (n=176)	-3.9 (± 0.22)			
Cycle 1, Week 4 (n=170)	-4.0 (± 0.23)			

Statistical analyses

No statistical analyses for this end point

Secondary: Percent Change in total IgG levels from baseline

End point title	Percent Change in total IgG levels from baseline
End point description: IgG: immunoglobulin G	
End point type	Secondary
End point timeframe: Up to week 4 of the first cycle	

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	164[2]			
Units: percent				
arithmetic mean (standard error)				
Cycle 1, Week 4	-62.6 (± 0.93)			

Notes:

[2] - Only participants with an assessment at baseline and week 4 of first cycle are reported here.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent Change in AChR-Ab From Baseline in AChR-Ab Seropositive Participants

End point title	Percent Change in AChR-Ab From Baseline in AChR-Ab Seropositive Participants
End point description: AchR-Ab: anti-acetylcholine receptor antibodies.	
End point type	Secondary
End point timeframe: Up to week 4 of the first cycle	

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	124[3]			
Units: percent change				
arithmetic mean (standard error)				
Cycle 1, Week 4	-57.6 (± 2.29)			

Notes:

[3] - Only AChR-Ab seropositive participants assessed at baseline and week 4 of first cycle are reported.

Statistical analyses

No statistical analyses for this end point

Secondary: Efgartigimod serum concentrations

End point title	Efgartigimod serum concentrations

End point description:

End point type	Secondary

End point timeframe:

Up to week 4 of the first cycle

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	141[4]			
Units: ng/mL				
arithmetic mean (standard deviation)				
Cycle 1, Week 4 (n=141)	21693 (± 8242)			

Notes:

[4] - Only participants with an assessment at week 4 of first cycle are reported here.

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of ADAs Against Efgartigimod Over Time

End point title	Incidence of ADAs Against Efgartigimod Over Time

End point description:

ADA: Anti-drug antibodies

End point type	Secondary

End point timeframe:

Up to 3.5 years

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	178[5]			
Units: Participants	35			

Notes:

[5] - The number analyzed is the total number of ADA evaluable participants.

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of NAbs Against Efgartigimod Over Time

End point title	Incidence of NAbs Against Efgartigimod Over Time

End point description:

NAbs: neutralizing antibodies

End point type	Secondary

End point timeframe:

Up to 3.5 years

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	178[6]			
Units: Participants	11			

Notes:

[6] - The number analyzed is the total number of NAb-evaluable participants.

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of Antibodies Against rHuPH20 Over Time

End point title	Incidence of Antibodies Against rHuPH20 Over Time

End point description:

End point type	Secondary

End point timeframe:

Up to 3.5 years

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	178[7]			
Units: Participants	49			

Notes:

[7] - The number analyzed is the total number of rHuPH20 Ab evaluable participants.

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of NAbs Against rHuPH20 Over Time

End point title	Incidence of NAbs Against rHuPH20 Over Time

End point description:

NAbs: neutralizing antibodies

End point type	Secondary

End point timeframe:

Up to 3.5 years

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	167[8]			
Units: Participants	1			

Notes:

[8] - The number analyzed is the total number of rHuPH20 NAb-evaluable participants.

Statistical analyses

No statistical analyses for this end point

Secondary: Changes in Total MG-QoL15r From Baseline

End point title	Changes in Total MG-QoL15r From Baseline

End point description:

Myasthenia Gravis Quality of Life 15 item scale revised (MG-QoL 15r) scores range from 0 to 30 with a higher score representing more severe symptoms.

End point type	Secondary

End point timeframe:

Up to week 4 of the first cycle

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	180			
Units: score				
arithmetic mean (standard error)				
Cycle 1, Week 1 (n=179)	-2.5 (± 0.29)			
Cycle 1, Week 2 (n=175)	-3.7 (± 0.34)			
Cycle 1, Week 3 (n=175)	-4.5 (± 0.38)			
Cycle 1, Week 4 (n=168)	-4.7 (± 0.39)			

Statistical analyses

No statistical analyses for this end point

Secondary: Changes in EQ-5D-5L VAS Score From Baseline

End point title	Changes in EQ-5D-5L VAS Score From Baseline

End point description:

EuroQoL 5 Dimensions 5-Level (EQ-5D-5L) visual analog scale (VAS) scores range from 0 to 100 with a higher score representing better health.

End point type	Secondary

End point timeframe:

Up to week 4 of the first cycle

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	169[9]			
Units: score on a scale				
arithmetic mean (standard error)				
Cycle 1, Week 4	13.9 (± 1.32)			

Notes:

[9] - Only participants with an assessment at baseline and week 4 of first cycle are reported here.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent of Participants or Caregivers by Number of Training Visits Needed to be Competent to Start Self- or Caregiver-Supported Administration

End point title	Percent of Participants or Caregivers by Number of Training Visits Needed to be Competent to Start Self- or Caregiver-Supported Administration

End point description:

End point type	Secondary

End point timeframe:

Up to 3.5 years

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	166[10]			
Units: percent				
number (not applicable)				
0 visits	4.8			
1 visit	45.8			
2 visits	13.9			
3 visits	11.4			
4 visits	12.7			
5 visits	1.8			
6 visits	5.4			
9 visits	1.2			
11 visits	0.6			
12 visits	0.6			
22 visits	0.6			
37 visits	0.6			
42 visits	0.6			

Notes:

[10] - Only participants/caregivers trained and capable of self- or caregiver-administrations are reported.

Statistical analyses

No statistical analyses for this end point

Secondary: Percent of Self- or Caregiver-supported Study Drug Administration Among All Study Treatment Visits at Home

End point title	Percent of Self- or Caregiver-supported Study Drug Administration Among All Study Treatment Visits at Home

End point description:

End point type	Secondary

End point timeframe:

Up to 3.5 years

End point values	Efgartigimod PH20 SC			
Subject group type	Reporting group			
Number of subjects analysed	180			
Units: percent				
number (not applicable)				
Participants at home	47.5			
Caregivers at home	1.7			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events:	
Up to 3.5 years	
Adverse event reporting additional description:	
Any clinically significant changes occurring during the study were reported as adverse events.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	24.1

Reporting groups	
Reporting group title	Total
Reporting group description:	
All participants who were exposed to efgartigimod PH20 SC in this study.	

Serious adverse events	Total		
Total subjects affected by serious adverse events			
subjects affected / exposed	55 / 180 (30.56%)		
number of deaths (all causes)	5		
number of deaths resulting from adverse events	5		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Bladder transitional cell carcinoma			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Metastases to liver			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Ovarian adenoma			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Rectal cancer stage IV			

subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Renal cancer metastatic				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Renal cell carcinoma				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 1			
Thymoma				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Uterine leiomyoma				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Vascular disorders				
Brachiocephalic vein stenosis				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Hypertension				
subjects affected / exposed	2 / 180 (1.11%)			
occurrences causally related to treatment / all	0 / 2			
deaths causally related to treatment / all	0 / 0			
Surgical and medical procedures				
Umbilical hernia repair				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Uterine polypectomy				

subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory, thoracic and mediastinal disorders			
Acute respiratory failure			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Chronic obstructive pulmonary disease			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Chronic respiratory failure			
subjects affected / exposed	2 / 180 (1.11%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Hydrothorax			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Interstitial lung disease			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pulmonary mass			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Respiratory failure			
subjects affected / exposed	4 / 180 (2.22%)		
occurrences causally related to treatment / all	0 / 5		
deaths causally related to treatment / all	0 / 3		
Injury, poisoning and procedural			

complications				
Fall				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Post procedural complication				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Rib fracture				
subjects affected / exposed	2 / 180 (1.11%)			
occurrences causally related to treatment / all	0 / 2			
deaths causally related to treatment / all	0 / 0			
Shunt occlusion				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Shunt stenosis				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Spinal fracture				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Tendon injury				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Cardiac disorders				
Acute myocardial infarction				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			

Angina unstable			
subjects affected / exposed	2 / 180 (1.11%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 0		
Cardiac arrest			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Cardiac failure congestive			
subjects affected / exposed	2 / 180 (1.11%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Coma			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Myasthenia gravis			
subjects affected / exposed	11 / 180 (6.11%)		
occurrences causally related to treatment / all	1 / 17		
deaths causally related to treatment / all	0 / 0		
Myasthenia gravis crisis			
subjects affected / exposed	4 / 180 (2.22%)		
occurrences causally related to treatment / all	0 / 5		
deaths causally related to treatment / all	0 / 0		
Syncope			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Eye disorders			
Diplopia			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Retinal detachment			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Vitreous haemorrhage			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Gastric ulcer haemorrhage			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal haemorrhage			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Inguinal hernia			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Intestinal perforation			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Terminal ileitis			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Renal and urinary disorders			
Renal failure			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Enthesopathy			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Muscular weakness			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Osteoarthritis			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Spinal osteoarthritis			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Spondyloarthropathy			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Anal abscess			

subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Bacteraemia				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Cellulitis				
subjects affected / exposed	2 / 180 (1.11%)			
occurrences causally related to treatment / all	0 / 2			
deaths causally related to treatment / all	0 / 0			
Cellulitis gangrenous				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
COVID-19				
subjects affected / exposed	4 / 180 (2.22%)			
occurrences causally related to treatment / all	0 / 4			
deaths causally related to treatment / all	0 / 1			
COVID-19 pneumonia				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Diarrhoea infectious				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Diverticulitis				
subjects affected / exposed	1 / 180 (0.56%)			
occurrences causally related to treatment / all	0 / 1			
deaths causally related to treatment / all	0 / 0			
Herpes zoster				

subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Orchitis			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Pneumonia			
subjects affected / exposed	7 / 180 (3.89%)		
occurrences causally related to treatment / all	1 / 7		
deaths causally related to treatment / all	0 / 0		
Rotavirus infection			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Sepsis			
subjects affected / exposed	2 / 180 (1.11%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Urinary tract infection			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Viral infection			
subjects affected / exposed	1 / 180 (0.56%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Total		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	167 / 180 (92.78%)		
Investigations			
Activated partial thromboplastin time prolonged			
subjects affected / exposed	12 / 180 (6.67%)		
occurrences (all)	15		
Injury, poisoning and procedural complications			
Contusion			
subjects affected / exposed	9 / 180 (5.00%)		
occurrences (all)	13		
Vascular disorders			
Hypertension			
subjects affected / exposed	14 / 180 (7.78%)		
occurrences (all)	17		
Nervous system disorders			
Dizziness			
subjects affected / exposed	13 / 180 (7.22%)		
occurrences (all)	18		
Headache			
subjects affected / exposed	48 / 180 (26.67%)		
occurrences (all)	205		
Myasthenia gravis			
subjects affected / exposed	18 / 180 (10.00%)		
occurrences (all)	23		
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	18 / 180 (10.00%)		
occurrences (all)	33		
Iron deficiency anaemia			
subjects affected / exposed	13 / 180 (7.22%)		
occurrences (all)	15		
General disorders and administration site conditions			
Fatigue			
subjects affected / exposed	10 / 180 (5.56%)		
occurrences (all)	20		
Influenza like illness			

subjects affected / exposed	14 / 180 (7.78%)		
occurrences (all)	20		
Injection site bruising			
subjects affected / exposed	24 / 180 (13.33%)		
occurrences (all)	80		
Injection site erythema			
subjects affected / exposed	55 / 180 (30.56%)		
occurrences (all)	727		
Injection site haematoma			
subjects affected / exposed	10 / 180 (5.56%)		
occurrences (all)	16		
Injection site pain			
subjects affected / exposed	22 / 180 (12.22%)		
occurrences (all)	47		
Injection site pruritus			
subjects affected / exposed	21 / 180 (11.67%)		
occurrences (all)	63		
Injection site rash			
subjects affected / exposed	15 / 180 (8.33%)		
occurrences (all)	32		
Injection site reaction			
subjects affected / exposed	10 / 180 (5.56%)		
occurrences (all)	47		
Injection site swelling			
subjects affected / exposed	11 / 180 (6.11%)		
occurrences (all)	100		
Pyrexia			
subjects affected / exposed	9 / 180 (5.00%)		
occurrences (all)	12		
Eye disorders			
Cataract			
subjects affected / exposed	9 / 180 (5.00%)		
occurrences (all)	13		
Gastrointestinal disorders			
Abdominal pain upper			

subjects affected / exposed	11 / 180 (6.11%)		
occurrences (all)	14		
Diarrhoea			
subjects affected / exposed	32 / 180 (17.78%)		
occurrences (all)	45		
Nausea			
subjects affected / exposed	16 / 180 (8.89%)		
occurrences (all)	25		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	12 / 180 (6.67%)		
occurrences (all)	14		
Musculoskeletal and connective tissue disorders			
Arthralgia			
subjects affected / exposed	17 / 180 (9.44%)		
occurrences (all)	22		
Back pain			
subjects affected / exposed	22 / 180 (12.22%)		
occurrences (all)	28		
Muscle spasms			
subjects affected / exposed	11 / 180 (6.11%)		
occurrences (all)	14		
Pain in extremity			
subjects affected / exposed	11 / 180 (6.11%)		
occurrences (all)	14		
Infections and infestations			
Bronchitis			
subjects affected / exposed	9 / 180 (5.00%)		
occurrences (all)	13		
COVID-19			
subjects affected / exposed	52 / 180 (28.89%)		
occurrences (all)	62		
Influenza			
subjects affected / exposed	9 / 180 (5.00%)		
occurrences (all)	9		
Nasopharyngitis			

subjects affected / exposed	36 / 180 (20.00%)		
occurrences (all)	55		
Upper respiratory tract infection			
subjects affected / exposed	32 / 180 (17.78%)		
occurrences (all)	75		
Urinary tract infection			
subjects affected / exposed	16 / 180 (8.89%)		
occurrences (all)	23		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
10 May 2021	Protocol v2.0 • Added optional PK sample collection visits with an associated objective and endpoint • Defined "caregiver" and provided information about the caregiver ICF • Added vaccination history collection for participants entering the study from ARGX-113-1705 • Added a retreatment condition to ensure participants can complete a full cycle before the end of the study • Required site staff to review the administration log and participant diary • Added specialty laboratory tests • Defined sexual abstinence as a contraceptive method
02 February 2023	Protocol v3.0 • Extended the study duration to collect data past 2 years of efgartigimod PH20 SC exposure. Participants can remain in the study until, at the latest, 31 Dec 2024 • Reduced the number of assessments from year 2 onward • Removed the 28-day restriction between TPs and the limit of 14 TPs from year 2 onward • Clarified inclusion criterion 2a so that participants who discontinued from an antecedent study for a non–life-threatening SAE are eligible • Removed the requirement that participants receive concurrent gMG therapy • Clarified in informed consent language that participants who are enrolled in another study with no therapeutic intervention are not to be excluded or discontinued • Added a requirement to permanently discontinue efgartigimod PH20 SC for participants diagnosed with a malignancy (except basal cell carcinoma of the skin) • Clarified the prohibited therapies requiring study discontinuation and prohibited activities during year 1 that do not require study discontinuation • Removed the requirement for male contraception

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported