



Clinical trial results:

An Open-Label, Multicenter, Follow-up Trial of ARGX-113-1904 to Evaluate the Safety, Tolerability, and Efficacy of Efgartigimod PH20 SC in Patients with Pemphigus (ADDRESS+)

Summary

EudraCT number	2020-002917-16
Trial protocol	DE HU ES GR BG FR IT
Global end of trial date	25 March 2024

Results information

Result version number	v1 (current)
This version publication date	04 January 2025
First version publication date	04 January 2025

Trial information

Trial identification

Sponsor protocol code	ARGX-113-1905
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Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT04598477
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	31 October 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	25 March 2024
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

To assess the long-term safety of treatment, tolerability, and efficacy of efgartigimod PH20 SC in participants with pemphigus vulgaris (PV) or pemphigus foliaceus (PF) who participated in ARGX-113-1904

Protection of trial subjects:

The protocol, protocol amendments, ICFs, Investigator Brochure, and participant recruitment information were approved by the IEC/IRB 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 were required to sign a statement of informed consent that met the requirements of the local regulations, ICH guidelines, Health Insurance Portability and Accountability Act requirements, where applicable, and the IRB/IEC or study center.

Background therapy:

Similar to the antecedent study ARGX-113-1904, participants could also receive concomitant prednisone. Investigators could increase or decrease the dose based on protocol-specified criteria.

Evidence for comparator:

Not applicable

Actual start date of recruitment	25 October 2020
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	Australia: 4
Country: Number of subjects enrolled	China: 25
Country: Number of subjects enrolled	Georgia: 6
Country: Number of subjects enrolled	India: 11
Country: Number of subjects enrolled	Japan: 8
Country: Number of subjects enrolled	Serbia: 1
Country: Number of subjects enrolled	Türkiye: 3
Country: Number of subjects enrolled	United Kingdom: 2
Country: Number of subjects enrolled	Ukraine: 12
Country: Number of subjects enrolled	United States: 13
Country: Number of subjects enrolled	Russian Federation: 15
Country: Number of subjects enrolled	Poland: 17
Country: Number of subjects enrolled	Romania: 2
Country: Number of subjects enrolled	Spain: 4

Country: Number of subjects enrolled	Bulgaria: 13
Country: Number of subjects enrolled	France: 8
Country: Number of subjects enrolled	Germany: 13
Country: Number of subjects enrolled	Greece: 9
Country: Number of subjects enrolled	Hungary: 6
Country: Number of subjects enrolled	Italy: 11
Worldwide total number of subjects	183
EEA total number of subjects	83

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)	159
From 65 to 84 years	24
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

This study was conducted at 76 sites that enrolled participants in 20 countries.

Pre-assignment

Screening details:

A total of 183 participants rolled over from ARGX-113-1904. Of these, 57 participants had a CRmin status (complete remission on minimal prednisone therapy) at rollover of which 34 participants did not receive efgartigimod PH20 SC in this ARGX-113-1905 study.

Period 1

Period 1 title	Overall 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

Are arms mutually exclusive?	Yes
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Arm title	Efgartigimod-efgartigimod PH20 SC
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Arm description:

Participants who received efgartigimod PH20 SC in antecedent study ARGX-113-1904.

Arm type	Experimental
Investigational medicinal product name	Efgartigimod PH20 SC
Investigational medicinal product code	ARGX-113 PH20 SC
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

At baseline, eligible participants received efgartigimod PH20 SC according to their clinical status at the rollover visit and was administered until participants achieved CRmin. Participants could continue to receive a prednisone (or equivalent) dose according to their clinical status at the rollover visit, and the dose was tapered or escalated based on clinical status at the investigator's discretion following protocol-specified instructions.

Arm title	Placebo-efgartigimod PH20 SC
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Arm description:

Participants who received placebo PH20 SC in antecedent study ARGX-113-1904.

Arm type	Experimental
Investigational medicinal product name	Efgartigimod PH20 SC
Investigational medicinal product code	ARGX-113 PH20 SC
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

At baseline, eligible participants received efgartigimod PH20 SC according to their clinical status at the rollover visit and was administered until participants achieved CRmin. Participants could continue to receive a prednisone (or equivalent) dose according to their clinical status at the rollover visit, and the dose was tapered or escalated based on clinical status at the investigator's discretion following protocol-specified instructions.

Number of subjects in period 1	Efgartigimod- efgartigimod PH20 SC	Placebo-efgartigimod PH20 SC
Started	123	60
Completed	64	23
Not completed	59	37
Adverse event, serious fatal	-	1
Required prohibited medication	-	1
Consent withdrawn by subject	16	12
Physician decision	7	2
Adverse event, non-fatal	2	-
Not specified	15	8
Pregnancy	-	1
Study terminated by sponsor	18	12
Lost to follow-up	1	-

Baseline characteristics

Reporting groups

Reporting group title	Efgartigimod-efgartigimod PH20 SC
Reporting group description: Participants who received efgartigimod PH20 SC in antecedent study ARGX-113-1904.	
Reporting group title	Placebo-efgartigimod PH20 SC
Reporting group description: Participants who received placebo PH20 SC in antecedent study ARGX-113-1904.	

Reporting group values	Efgartigimod-efgartigimod PH20 SC	Placebo-efgartigimod PH20 SC	Total
Number of subjects	123	60	183
Age categorical Units: Subjects			
Adults (18-64 years)	108	51	159
From 65-84 years	15	9	24
Age continuous Units: years			
arithmetic mean	50.1	52.4	
standard deviation	± 11.40	± 13.02	-
Gender categorical Units: Subjects			
Female	61	32	93
Male	62	28	90

End points

End points reporting groups

Reporting group title	Efgartigimod-efgartigimod PH20 SC
Reporting group description: Participants who received efgartigimod PH20 SC in antecedent study ARGX-113-1904.	
Reporting group title	Placebo-efgartigimod PH20 SC
Reporting group description: Participants who received placebo PH20 SC in antecedent study ARGX-113-1904.	
Subject analysis set title	Rollover Analysis Set
Subject analysis set type	Full analysis
Subject analysis set description: The rollover analysis set included all participants who rolled over from study ARGX-113-1904, regardless of whether or not they received efgartigimod PH20 SC treatment as part of this study. Additional restrictions to the analysis set might apply in the different outcome measures. These are described as notes to the number of subjects analyzed.	
Subject analysis set title	Safety Analysis Set
Subject analysis set type	Safety analysis
Subject analysis set description: The safety analysis set included participants who received at least 1 dose of efgartigimod PH20 SC during this study. Additional restrictions to the analysis set might apply in the different outcome measures. These are described in the number of subjects analyzed.	

Primary: Incidence of Treatment-Emergent Adverse Events (TEAE), Adverse Events of Special Interest (AESI), and Serious Adverse Events (SAE)

End point title	Incidence of Treatment-Emergent Adverse Events (TEAE), Adverse Events of Special Interest (AESI), and Serious Adverse Events (SAE) ^[1]
End point description: Incidence rates were calculated as $100 \times n / \text{PYFU}$. PYFU=participant-years of follow-up. The safety data sets includes participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF).	
End point type	Primary
End point timeframe: Up to Week 60	
Notes: [1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point. Justification: No statistical analysis was applied to this end point	

End point values	Efgartigimod-efgartigimod PH20 SC	Placebo-efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	101 ^[2]	48 ^[3]		
Units: number				
number (not applicable)				
TEAE	116.4	113.0		
AESI	72.0	69.3		
SAE	24.5	14.6		

Notes:

[2] - Safety set

[3] - Safety set

Statistical analyses

No statistical analyses for this end point

Secondary: Proportion of participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF) who achieve CRmin

End point title	Proportion of participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF) who achieve CRmin
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End point description:

CRmin defined as the absence of new lesions and complete healing of established lesions while the participant was receiving prednisone at ≤ 10 mg/day for at least 8 weeks.

End point type	Secondary
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End point timeframe:

Up to 60 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	101 ^[4]	48 ^[5]		
Units: participants, n	55	26		

Notes:

[4] - Safety set

[5] - Safety set

Statistical analyses

No statistical analyses for this end point

Secondary: Proportion of participants with pemphigus vulgaris (PV) participants who achieve CRmin

End point title	Proportion of participants with pemphigus vulgaris (PV) participants who achieve CRmin
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End point description:

CRmin (complete clinical remission on minimal prednisone therapy) defined as the absence of new lesions and complete healing of established lesions while the participant was receiving prednisone at ≤ 10 mg/day for at least 8 weeks.

End point type	Secondary
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End point timeframe:

Up to 60 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	86 ^[6]	41 ^[7]		
Units: participants, n	47	22		

Notes:

[6] - Safety set - Participants with PV only

[7] - Safety set - Participants with PV only

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Disease Control (DC) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)

End point title	Time to Disease Control (DC) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)
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End point description:

Disease Control (DC) defined as absence of new lesions and the start of healing of established lesions.

End point type	Secondary
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End point timeframe:

Up to 52 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	26 ^[8]	17 ^[9]		
Units: days				
median (confidence interval 95%)	8.5 (8.0 to 15.0)	15.0 (8.0 to 22.0)		

Notes:

[8] - Safety set – Participants with status DC at the roll-over visit were not included in the analysis

[9] - Safety set – Participants with status DC at the roll-over visit were not included in the analysis

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Complete clinical remission (CR) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)

End point title	Time to Complete clinical remission (CR) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)
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End point description:

Measure Description CR (Complete clinical remission) defined as the absence of new lesions and complete healing of established lesions.

End point type	Secondary
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End point timeframe:

Up to 52 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	61 ^[10]	34 ^[11]		
Units: days				
median (confidence interval 95%)	66.0 (43.0 to 182.0)	71.0 (41.0 to 100.0)		

Notes:

[10] - Safety set – Participants with status CR at the roll-over visit were not included in this analysis

[11] - Safety set – Participants with status CR at the roll-over visit were not included in this analysis

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Complete remission on minimal prednisone therapy (CRmin) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)

End point title	Time to Complete remission on minimal prednisone therapy (CRmin) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)
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End point description:

CRmin defined as the absence of new lesions and complete healing of established lesions while the participant was receiving prednisone at ≤ 10 mg/day for at least 8 weeks.

* The value '9999' is a dummy number to indicate the number was not calculable.

End point type	Secondary
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End point timeframe:

Up to 52 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	83 ^[12]	43 ^[13]		
Units: days				
median (confidence interval 95%)	229.0 (161.0 to 9999)	169.0 (141.0 to 322.0)		

Notes:

[12] - Safety set – Participants with status CRmin at the rollover visit were not included in this analysis

[13] - Safety set – Participants with status CRmin at the rollover visit were not included in this analysis

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Complete remission off therapy (CROff) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)

End point title	Time to Complete remission off therapy (CROff) in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)
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End point description:

Complete remission off therapy (CROff) is defined as the absence of new and established lesions completely healed while the patient is receiving no prednisone therapy for at least 8 weeks.

* The value '9999' is a dummy number to indicate the number was not calculable.

End point type	Secondary
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End point timeframe:
up to 52 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	83 ^[14]	43 ^[15]		
Units: days				
median (confidence interval 95%)	9999 (9999 to 9999)	9999 (9999 to 9999)		

Notes:

[14] - Safety set – Participants with status CROff at the rollover visit were not included in this analysis

[15] - Safety set – Participants with status CROff at the rollover visit were not included in this analysis

Statistical analyses

No statistical analyses for this end point

Secondary: Time to flare after CRmin in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)

End point title	Time to flare after CRmin in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)
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End point description:

CRmin defined as defined as the absence of new lesions and complete healing of established lesions while the participant was receiving prednisone at ≤ 10 mg/day for at least 8 weeks.

End point type	Secondary
End point timeframe:	
Up to 52 weeks	

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	42 ^[16]	23 ^[17]		
Units: days				
median (confidence interval 95%)	339.0 (223.0 to 9999)	168.0 (64.0 to 9999)		

Notes:

[16] - Safety set - Only participants who achieved CRmin were considered for the analysis

[17] - Safety set - Only participants who achieved CRmin were considered for the analysis

Statistical analyses

No statistical analyses for this end point

Secondary: Rate of treatment failure in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)

End point title	Rate of treatment failure in participants with pemphigus vulgaris (PV) and pemphigus foliaceus (PF)
End point description: The absence of DC with oral prednisone 1.5 mg/kg/day for a minimum of 3 weeks, or absence of DC due to prednisone-related SAE, or flare before CRmin resulting in withdrawal of the participant.	
End point type	Secondary
End point timeframe: Up to 52 weeks	

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	101 ^[18]	48 ^[19]		
Units: number	3	1		

Notes:

[18] - Safety set

[19] - Safety set

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Flares in participants with PV and PF

End point title	Number of Flares in participants with PV and PF
End point description: A flare is defined as the appearance of 3 or more new lesions in a 4-week period that do not heal spontaneously within 1 week or the extension, of established lesions in a participant who had achieved DC.	
End point type	Secondary
End point timeframe: Up to 60 weeks	

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	122 ^[20]	59 ^[21]		
Units: number				
arithmetic mean (standard deviation)	0.8 (± 1.03)	0.7 (± 0.79)		

Notes:

[20] - Roll-over set - Only participants who achieved DC were considered for the analysis

[21] - Roll-over set - Only participants who achieved DC were considered for the analysis

Statistical analyses

No statistical analyses for this end point

Secondary: Normalized Cumulative Prednisone Dose in participants with PV and PF

End point title	Normalized Cumulative Prednisone Dose in participants with PV and PF
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End point description:

Normalized Cumulative prednisone dose (NCPD, mg/kg/day) is the average daily intake of all weight-adjusted prednisone doses received during the study, taking into account the number of days in study

End point type	Secondary
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End point timeframe:

up to 60 weeks

End point values	Efgartigimod- efgartigimod PH20 SC	Placebo- efgartigimod PH20 SC		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	123 ^[22]	60 ^[23]		
Units: mg/kg/day				
arithmetic mean (standard error)	0.212 (± 0.2018)	0.241 (± 0.2401)		

Notes:

[22] - For participants that do not achieve CRmin/CROff, NCPD until CRmin/CROff is not calculated

[23] - For participants that do not achieve CRmin/CROff, NCPD until CRmin/CROff is not calculated

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

Up to 60 weeks

Adverse event reporting additional description:

Participants who rolled over from the antecedent study ARGX-113-1904, and received at least 1 dose of efgartigimod PH20 SC during this ARGX-113-1905 study (Safety set). Laboratory abnormalities were reported as AEs.

Assessment type	Systematic
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Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.0
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Reporting groups

Reporting group title	Efgartigimodefgartigimod PH20 SC
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Reporting group description:

Participants who received efgartigimod PH20 SC in antecedent study ARGX-113-1904.

Reporting group title	Placebo-efgartigimod PH20 SC
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Reporting group description:

Participants who received placebo PH20 SC in antecedent study ARGX-113-1904.

Serious adverse events	Efgartigimodefgartigimod PH20 SC	Placebo-efgartigimod PH20 SC	
Total subjects affected by serious adverse events			
subjects affected / exposed	16 / 101 (15.84%)	4 / 48 (8.33%)	
number of deaths (all causes)	0	1	
number of deaths resulting from adverse events	0	1	
Investigations			
Blood immunoglobulin G decreased			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood pressure increased			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Injury, poisoning and procedural complications			
Patella fracture			

subjects affected / exposed	0 / 101 (0.00%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Tibia fracture			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Wound			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Vascular disorders			
Deep vein thrombosis			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hypertension			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Shock haemorrhagic			
subjects affected / exposed	0 / 101 (0.00%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
General disorders and administration site conditions			
Disease progression			
subjects affected / exposed	2 / 101 (1.98%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Anal fistula			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Gastritis erosive			
subjects affected / exposed	2 / 101 (1.98%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Intestinal obstruction			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Reflux gastritis			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory, thoracic and mediastinal disorders			
Respiratory failure			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Skin and subcutaneous tissue disorders			
Pemphigus			
subjects affected / exposed	3 / 101 (2.97%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	1 / 3	1 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Musculoskeletal and connective tissue disorders			
Musculoskeletal pain			
subjects affected / exposed	0 / 101 (0.00%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Osteonecrosis			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Anal abscess			

subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
COVID-19			
subjects affected / exposed	2 / 101 (1.98%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Epiglottitis			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Lung abscess			
subjects affected / exposed	0 / 101 (0.00%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pneumonia			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Sepsis			
subjects affected / exposed	0 / 101 (0.00%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 1	
Septic shock			
subjects affected / exposed	1 / 101 (0.99%)	0 / 48 (0.00%)	
occurrences causally related to treatment / all	1 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Skin infection			
subjects affected / exposed	0 / 101 (0.00%)	1 / 48 (2.08%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	

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

Non-serious adverse events	Efgartigimod PH20 SC	Placebo-efgartigimod PH20 SC	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	46 / 101 (45.54%)	19 / 48 (39.58%)	
Investigations			
Blood lactate dehydrogenase increased			
subjects affected / exposed	7 / 101 (6.93%)	1 / 48 (2.08%)	
occurrences (all)	8	1	
Blood uric acid increased			
subjects affected / exposed	5 / 101 (4.95%)	0 / 48 (0.00%)	
occurrences (all)	5	0	
Glycosylated haemoglobin increased			
subjects affected / exposed	6 / 101 (5.94%)	0 / 48 (0.00%)	
occurrences (all)	6	0	
Low density lipoprotein increased			
subjects affected / exposed	5 / 101 (4.95%)	0 / 48 (0.00%)	
occurrences (all)	8	0	
Cardiac disorders			
Tachycardia			
subjects affected / exposed	1 / 101 (0.99%)	3 / 48 (6.25%)	
occurrences (all)	1	3	
Nervous system disorders			
Headache			
subjects affected / exposed	3 / 101 (2.97%)	4 / 48 (8.33%)	
occurrences (all)	11	4	
General disorders and administration site conditions			
Injection site erythema			
subjects affected / exposed	4 / 101 (3.96%)	3 / 48 (6.25%)	
occurrences (all)	30	5	
Blood and lymphatic system disorders			
Increased tendency to bruise			
subjects affected / exposed	0 / 101 (0.00%)	3 / 48 (6.25%)	
occurrences (all)	0	3	
Skin and subcutaneous tissue disorders			

Pruritus subjects affected / exposed occurrences (all)	2 / 101 (1.98%) 2	3 / 48 (6.25%) 5	
Renal and urinary disorders Haematuria subjects affected / exposed occurrences (all)	2 / 101 (1.98%) 2	3 / 48 (6.25%) 4	
Musculoskeletal and connective tissue disorders Myopathy subjects affected / exposed occurrences (all)	2 / 101 (1.98%) 2	3 / 48 (6.25%) 3	
Infections and infestations COVID-19 subjects affected / exposed occurrences (all) Folliculitis subjects affected / exposed occurrences (all) Nasopharyngitis subjects affected / exposed occurrences (all) Oral candidiasis subjects affected / exposed occurrences (all) Urinary tract infection subjects affected / exposed occurrences (all)	12 / 101 (11.88%) 12 5 / 101 (4.95%) 6 5 / 101 (4.95%) 7 6 / 101 (5.94%) 8 5 / 101 (4.95%) 5	5 / 48 (10.42%) 5 1 / 48 (2.08%) 1 1 / 48 (2.08%) 4 0 / 48 (0.00%) 0 2 / 48 (4.17%) 2	
Metabolism and nutrition disorders Hypercholesterolaemia subjects affected / exposed occurrences (all)	5 / 101 (4.95%) 5	0 / 48 (0.00%) 0	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
05 February 2021	Protocol, Version 2.0 • A secondary objective and endpoints were added to explore the feasibility of efgartigimod PH20 SC self-administration or caregiver-supported administration. Instructions were added and the schedule of activities updated to include self-administration or caregiver-supported administration and training. • A secondary objective and endpoint were added to measure the health impact of glucocorticoid use in participants with pemphigus. • Evaluating antibodies against rHuPH20 was changed from a secondary objective and endpoint to an exploratory objective and endpoint due to safety concerns. • Lymphocyte dynamic changes was added as an endpoint to the exploratory objective to evaluate the disease-specific genetic background and effects of efgartigimod PH20 SC on the serological and immunological profiles. • Assessments of vaccine-induced immunity in the context of efgartigimod PH20 SC administration were added. • Changes to the contraceptive requirements based on new data about efgartigimod PH20 SC were implemented. • A transition in efgartigimod concentration from 165 mg/mL to 180 mg/mL was implemented to reduce the volume for each 1000-mg SC injection. • A new criterion was added allowing the withdrawal from the study of a participant for whom a severe AE, SAE, or clinically significant change in a laboratory test parameter was reported.
05 September 2022	Protocol, Version 3.0 • The upper limit of the number of participants who could enter the study (originally up to 150) was removed after the sample size for ARGX-113-1904 was increased. • The timing of samples collected for lymphocyte populations was updated to allow for long-term immunological profiling. • Based on nonclinical teratogenicity and reproductive toxicity data, the inclusion and exclusion criteria were updated: (1) Female participants could stop their contraception method after the last IMP dose, (2) female participants and the female partners of male participants could become pregnant immediately after the study, and (3) male participants could donate sperm. • Information on injection-site reactions and instructions on monitoring and reporting injection-site reactions were added.

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.

Study was terminated prematurely by the Sponsor.

Notes: