



Clinical trial results:

A Randomized, Double blind, Placebo Controlled Phase II Study to Evaluate the Safety, Efficacy, and Pharmacokinetics of ARGX 113 in Patients with Myasthenia Gravis who have Generalized Muscle Weakness

Summary

| | |
|--------------------------|-----------------|
| EudraCT number | 2016-002938-73 |
| Trial protocol | BE SE ES NL IT |
| Global end of trial date | 20 October 2017 |

Results information

| | |
|--------------------------------|------------------|
| Result version number | v1 (current) |
| This version publication date | 03 November 2018 |
| First version publication date | 03 November 2018 |

Trial information

Trial identification

| | |
|-----------------------|---------------|
| Sponsor protocol code | ARGX-113-1602 |
|-----------------------|---------------|

Additional study identifiers

| | |
|------------------------------------|-------------|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | NCT02965573 |
| WHO universal trial number (UTN) | - |

Notes:

Sponsors

| | |
|------------------------------|---------------------------------------------------------------|
| Sponsor organisation name | argenx |
| Sponsor organisation address | Industriepark 7, Zwijnaarde, Belgium, 9052 |
| Public contact | Regulatory, argenx BVBA, +32 9310 3400, regulatory@argenx.com |
| Scientific contact | Regulatory, argenx BVBA, +32 9310 3400, 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 | 20 October 2017 |
| Is this the analysis of the primary completion data? | No |
| Global end of trial reached? | Yes |
| Global end of trial date | 20 October 2017 |
| Was the trial ended prematurely? | No |

Notes:

General information about the trial

Main objective of the trial:

To evaluate the safety and tolerability of ARGX 113.

Protection of trial subjects:

Safety assessments consisted of monitoring and recording all AEs, including SAEs, and pregnancies; suicidality assessment; safety laboratory testing, measurement of vital signs, ECGs, physical examinations; and other tests that were deemed critical to the safety evaluation of the study in all subjects who received at least 1 dose of the IMP.

Background therapy:

In this study, standard of care (SoC) for a patient was the stable dose and administration of their MG treatment prior to enrollment. Permitted SoC for MG treatment under this protocol included azathioprine (AZA), other non steroidal immunosuppressant drugs (NSIDs: e.g., methotrexate, cyclosporine, tacrolimus, mycophenolate mofetil, and cyclophosphamide), steroids, as well as cholinesterase inhibitors. Patients had to be on a stable dose and frequency of SoC prior to enrollment as detailed in the protocol and for the duration of the study.

Evidence for comparator: -

| | |
|-----------------------------------------------------------|------------------|
| Actual start date of recruitment | 30 December 2016 |
| 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 | Canada: 1 |
| Country: Number of subjects enrolled | United States: 4 |
| Country: Number of subjects enrolled | Netherlands: 6 |
| Country: Number of subjects enrolled | Poland: 6 |
| Country: Number of subjects enrolled | Spain: 1 |
| Country: Number of subjects enrolled | Sweden: 1 |
| Country: Number of subjects enrolled | Belgium: 1 |
| Country: Number of subjects enrolled | Italy: 4 |
| Worldwide total number of subjects | 24 |
| EEA total number of subjects | 19 |

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) | 18 |
| From 65 to 84 years | 6 |
| 85 years and over | 0 |

Subject disposition

Recruitment

Recruitment details:

This study was conducted by 15 Investigators at 15 study centers (i.e., study centers that consented at least 1 patient) in 8 countries (Belgium, Canada, Italy, the Netherlands, Poland, Spain, Sweden, and United States). A total of 24 subjects were randomized in the study.

Pre-assignment

Screening details:

The study included a maximum Screening period of 15 days to evaluate patients' eligibility. Evaluations at screening and confirmation at visit 1 were used to determine the eligibility of each subject for randomization in the study. Subjects who failed to meet the eligibility criteria were considered screen failures.

Period 1

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

Blinding implementation details:

The IMPs (ARGX-113 and placebo) were identical in physical appearance.

Arms

| | |
|------------------------------|-----|
| Are arms mutually exclusive? | Yes |
|------------------------------|-----|

| | |
|------------------|----------|
| Arm title | ARGX-113 |
|------------------|----------|

Arm description: -

| | |
|----------------------------------------|---------------------------------------|
| 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:

ARGX-113 was administered weekly for 4 weeks by intravenous infusion.

| | |
|------------------|---------|
| Arm title | Placebo |
|------------------|---------|

Arm description: -

| | |
|----------------------------------------|---------------------------------------|
| 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:

Placebo was administered weekly for 4 weeks by intravenous use.

| Number of subjects in period 1 | ARGX-113 | Placebo |
|---------------------------------------|----------|---------|
| Started | 12 | 12 |
| Completed | 11 | 12 |
| Not completed | 1 | 0 |
| Lack of efficacy | 1 | - |

Baseline characteristics

Reporting groups

| | |
|--------------------------------|----------|
| Reporting group title | ARGX-113 |
| Reporting group description: - | |
| Reporting group title | Placebo |
| Reporting group description: - | |

| Reporting group values | ARGX-113 | Placebo | Total |
|-------------------------------------------------------|----------|---------|-------|
| Number of subjects | 12 | 12 | 24 |
| Age categorical Units: Subjects | | | |
| In utero | 0 | 0 | 0 |
| Preterm newborn infants (gestational age < 37 wks) | 0 | 0 | 0 |
| Newborns (0-27 days) | 0 | 0 | 0 |
| Infants and toddlers (28 days-23 months) | 0 | 0 | 0 |
| Children (2-11 years) | 0 | 0 | 0 |
| Adolescents (12-17 years) | 0 | 0 | 0 |
| Adults (18-64 years) | 8 | 10 | 18 |
| From 65-84 years | 4 | 2 | 6 |
| 85 years and over | 0 | 0 | 0 |
| Gender categorical Units: Subjects | | | |
| Female | 7 | 8 | 15 |
| Male | 5 | 4 | 9 |

End points

End points reporting groups

| | |
|--------------------------------|----------|
| Reporting group title | ARGX-113 |
| Reporting group description: - | |
| Reporting group title | Placebo |
| Reporting group description: - | |

Primary: 1. Adverse events

| | |
|------------------------|----------------------------------|
| End point title | 1. Adverse events ^[1] |
| End point description: | |

| | |
|-----------------------------------|---------|
| End point type | Primary |
| End point timeframe: | |
| The entire duration of the study. | |

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: The primary endpoint is analysed by means of descriptive statistics.

| End point values | ARGX-113 | Placebo | | |
|-------------------------------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Number of events/ Number of patients | | | | |
| Number of events | 60 | 44 | | |
| Patients with at least 1 TEAE | 10 | 10 | | |
| Patients with at least 1 nonTEAE | 2 | 4 | | |
| Patients with at least 1 serious TEAE | 0 | 0 | | |
| Patients withdrawn with at least 1 serious TEAE | 0 | 0 | | |
| Patients with at least 1 related serious TEAE | 0 | 0 | | |
| Patients who discontinued due to a TEAE | 0 | 0 | | |
| Patients with at least 1 TEAE CTCAE ≥3 | 0 | 0 | | |
| Patients with at least 1 related TEAE | 8 | 3 | | |
| Number of deaths | 0 | 0 | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 2. MG-ADL Score Change from Baseline

| | |
|------------------------|--------------------------------------|
| End point title | 2. MG-ADL Score Change from Baseline |
| End point description: | |

| | |
|-------------------------------------|-----------|
| End point type | Secondary |
| End point timeframe: | |
| From Day 1 (Baseline) until Day 78. | |

| End point values | ARGX-113 | Placebo | | |
|-------------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Score change | | | | |
| median (full range (min-max)) | | | | |
| Day 1 (Baseline) | 7.5 (5 to 15) | 8.0 (5 to 13) | | |
| Day 8 | -1.0 (-10 to 0) | -0.5 (-4 to 2) | | |
| Day 15 | -2.0 (-9 to 0) | -2.0 (-7 to 0) | | |
| Day 22 | -3.5 (-8 to 0) | -2.0 (-7 to 1) | | |
| Day 29 | -4.0 (-8 to 0) | -1.0 (-7 to 0) | | |
| Day 36 | -3.5 (-10 to 1) | -1.5 (-6 to 1) | | |
| Day 43 | -4.0 (-8 to 1) | -1.0 (-8 to 1) | | |
| Day 50 | -3 (-11 to -1) | -1.0 (-9 to 0) | | |
| Day 64 | -2.5 (-9 to 1) | -1.0 (-8 to 5) | | |
| Day 78 | -3.0 (-10 to 1) | -1.0 (-9 to 4) | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 3. QMG score change from baseline

| | |
|-------------------------------------|-----------------------------------|
| End point title | 3. QMG score change from baseline |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| From Day 1 (Baseline) until Day 78. | |

| End point values | ARGX-113 | Placebo | | |
|-----------------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Score change from baseline | | | | |
| median (full range (min-max)) | | | | |
| Day 1 (Baseline) | 14.0 (6 to 30) | 12.5 (3 to 24) | | |
| Day 8 | -2.0 (-10 to 0) | 0 (-3 to 4) | | |
| Day 15 | -3.0 (-12 to 1) | -1.5 (-8 to 2) | | |
| Day 22 | -3.0 (-14 to 3) | -2.0 (-8 to 4) | | |
| Day 29 | -4.5 (-16 to 1) | -1.0 (-8 to 4) | | |
| Day 36 | -3.5 (-16 to 2) | -0.5 (-10 to 1) | | |

| | | | | |
|--------|-----------------|-----------------|--|--|
| Day 43 | -3.5 (-15 to 3) | -2.0 (-10 to 6) | | |
| Day 50 | -5.0 (-16 to 2) | -1.0 (-10 to 3) | | |
| Day 64 | -2.0 (-15 to 2) | -1.0 (-13 to 3) | | |
| Day 78 | -2.0 (-18 to 3) | -1.5 (-11 to 4) | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 4. MGC score change from baseline

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|-----------------|-----------------------------------|
| End point title | 4. MGC score change from baseline |
|-----------------|-----------------------------------|

End point description:

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 (Baseline) until Day 78.

| End point values | ARGX-113 | Placebo | | |
|-----------------------------------|------------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Score change from baseline | | | | |
| median (full range (min-max)) | | | | |
| Day 1 (baseline) | 14.0 (6 to 37) | 14.0 (8 to 25) | | |
| Day 8 | -4.0 (-17 to 4) | -0.5 (-6 to 3) | | |
| Day 15 | -5.5 (-16 to 4) | -3.5 (-14 to 1) | | |
| Day 22 | -5.5 (-19 to 2) | -3.5 (-11 to 0) | | |
| Day 29 | -8.0 (-19 to 5) | -4.0 (-12 to 4) | | |
| Day 36 | -7.5 (-21 to 7) | -4.0 (-12 to 4) | | |
| Day 43 | -10.5 (-20 to 7) | -5.0 (-12 to 8) | | |
| Day 50 | -5.0 (-23 to 1) | -2.5 (-13 to 4) | | |
| Day 64 | -6.0 (-21 to 4) | -3.0 (-15 to 7) | | |
| Day 78 | -8.0 (-23 to 6) | -3.5 (-14 to 5) | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 5. MGQoL15r score change from baseline

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|-----------------|----------------------------------------|
| End point title | 5. MGQoL15r score change from baseline |
|-----------------|----------------------------------------|

End point description:

Myasthenia Gravis Quality of Life-15 (revised version)

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|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 (Baseline) until Day 78.

| End point values | ARGX-113 | Placebo | | |
|-----------------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Score change from baseline | | | | |
| median (full range (min-max)) | | | | |
| Day 1 (Baseline) | 21.0 (10 to 28) | 12.5 (5 to 25) | | |
| Day 8 | 0 (-20 to 2) | 0 (-5 to 2) | | |
| Day 15 | -2.5 (-16 to 0) | 0 (-6 to 3) | | |
| Day 22 | -3.0 (-17 to 0) | 0 (-8 to 2) | | |
| Day 29 | -3.0 (-18 to 2) | 0 (-8 to 2) | | |
| Day 36 | -6.0 (-19 to 2) | -1.0 (-11 to 3) | | |
| Day 43 | -4.5 (-19 to 2) | 0 (-10 to 3) | | |
| Day 50 | -4.0 (-12 to 2) | -1.0 (-10 to 3) | | |
| Day 64 | -3.0 (-15 to 2) | 0 (-10 to 3) | | |
| Day 78 | -3.0 (-15 to 5) | -0.5 (-10 to 3) | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 6. Maximum reduction from Baseline across visit days for the various scores

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|-----------------|-----------------------------------------------------------------------------|
| End point title | 6. Maximum reduction from Baseline across visit days for the various scores |
|-----------------|-----------------------------------------------------------------------------|

End point description:

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 (Baseline) until Day 78.

| End point values | ARGX-113 | Placebo | | |
|----------------------------------------------|-------------------|------------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Maximum reduction | | | | |
| median (full range (min-max)) | | | | |
| Myasthenia Gravis Activities of Daily Living | -4.5 (-11 to 0) | -2.0 (-9 to 0) | | |
| Quantitative Myasthenia Gravis | -4.5 (-18 to -2) | -4.0 (-13 to 1) | | |
| Myasthenia Gravis Composite | -10.5 (-23 to -1) | -6.5 (-15 to -2) | | |

| | | | | |
|------------------------------------------------------|------------------|-----------------|--|--|
| Myasthenia Gravis Quality of Life-15 (revised v.) | -6.0 (-20 to -1) | -3.0 (-11 to 0) | | |
|------------------------------------------------------|------------------|-----------------|--|--|

Statistical analyses

No statistical analyses for this end point

Secondary: 7. Pharmacokinetics: Cmax

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|-----------------|---------------------------|
| End point title | 7. Pharmacokinetics: Cmax |
|-----------------|---------------------------|

End point description:

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 (Baseline) until Day 22.

| End point values | ARGX-113 | Placebo | | |
|-------------------------------|--------------------|------------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 ^[2] | 0 ^[3] | | |
| Units: µg/mL | | | | |
| median (full range (min-max)) | | | | |
| Day 1 | 173.5 (114 to 276) | (to) | | |
| Day 8 | 173.0 (117 to 219) | (to) | | |
| Day 15 | 156.0 (110 to 209) | (to) | | |
| Day 22 | 156.0 (113 to 253) | (to) | | |

Notes:

[2] - As of Day 8, values for n=11

[3] - No pharmacokinetic analysis was performed for placebo-treated subjects.

Statistical analyses

No statistical analyses for this end point

Secondary: 8. Pharmacokinetics: Tmax

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|-----------------|---------------------------|
| End point title | 8. Pharmacokinetics: Tmax |
|-----------------|---------------------------|

End point description:

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 (Baseline) until Day 22.

| End point values | ARGX-113 | Placebo | | |
|-------------------------------|---------------------|------------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 0 ^[4] | | |
| Units: Hours | | | | |
| median (full range (min-max)) | | | | |
| Day 1 | 2.44 (2.08 to 2.58) | (to) | | |
| Day 8 | 2.50 (2.08 to 2.50) | (to) | | |
| Day 15 | 2.50 (2.07 to 2.50) | (to) | | |
| Day 22 | 2.46 (2.08 to 2.67) | (to) | | |

Notes:

[4] - No pharmacokinetic analysis was performed for placebo-treated subjects.

Statistical analyses

No statistical analyses for this end point

Secondary: 9. Evaluation of total IgG

| | |
|-------------------------------------|----------------------------|
| End point title | 9. Evaluation of total IgG |
| End point description: | |
| End point type | Secondary |
| End point timeframe: | |
| From Day 1 (Baseline) until Day 78. | |

| End point values | ARGX-113 | Placebo | | |
|-------------------------------|----------------------|----------------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: µg/mL | | | | |
| median (full range (min-max)) | | | | |
| Day 1 (Baseline) | 8825 (4340 to 19800) | 6855 (4670 to 14700) | | |
| Day 8 | 5400 (2610 to 13800) | 7220 (4050 to 13900) | | |
| Day 15 | 3825 (1620 to 9120) | 7840 (4480 to 14300) | | |
| Day 22 | 2475 (1680 to 4790) | 6800 (4800 to 12900) | | |
| Day 29 | 2540 (1370 to 7600) | 6460 (4330 to 13200) | | |
| Day 36 | 4590 (1410 to 8430) | 6745 (5180 to 9940) | | |
| Day 43 | 3870 (2240 to 12300) | 7210 (3880 to 10600) | | |
| Day 50 | 5310 (2410 to 14300) | 7435 (4530 to 11100) | | |
| Day 57 | 6070 (3080 to 16400) | 7610 (4890 to 13300) | | |

| | | | | |
|--------|----------------------|----------------------|--|--|
| Day 64 | 6220 (3940 to 13100) | 7225 (4480 to 14400) | | |
| Day 71 | 6080 (2750 to 21700) | 7585 (4180 to 14700) | | |
| Day 78 | 7095 (1890 to 24200) | 7420 (5000 to 14200) | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 10. Evaluation of anti-AChR antibodies

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|-----------------|----------------------------------------|
| End point title | 10. Evaluation of anti-AChR antibodies |
|-----------------|----------------------------------------|

End point description:

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 until Day 78.

| End point values | ARGX-113 | Placebo | | |
|-------------------------------|--------------------------|---------------------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: nmol/L | | | | |
| median (full range (min-max)) | | | | |
| Day 1 | 7.030 (0.265 to 106.000) | 11.115 (0.398 to 186.000) | | |
| Day 8 | 5.035 (0.265 to 51.700) | 10.715 (0.401 to 223.000) | | |
| Day 15 | 4.185 (0.265 to 38.100) | 11.845 (0.379 to 179.000) | | |
| Day 22 | 2.160 (0.265 to 34.900) | 11.545 (0.366 to 169.000) | | |
| Day 29 | 3.755 (0.265 to 32.100) | 6.185 (0.265 to 173.000) | | |
| Day 36 | 4.280 (0.265 to 40.100) | 12.200 (0.380 to 188.000) | | |
| Day 43 | 5.590 (0.265 to 46.300) | 3.310 (0.361 to 168.000) | | |
| Day 50 | 5.390 (0.265 to 52.400) | 6.805 (0.379 to 162.000) | | |
| Day 57 | 6.330 (0.265 to 73.600) | 7.390 (0.370 to 213.000) | | |
| Day 64 | 6.470 (0.265 to 73.700) | 7.375 (0.366 to 169.000) | | |
| Day 71 | 7.790 (0.265 to 73.100) | 13.050 (0.367 to 181.000) | | |
| Day 78 | 7.165 (0.265 to 88.900) | 7.425 (0.356 to 165.000) | | |

Statistical analyses

No statistical analyses for this end point

Secondary: 11. Evaluation of the incidence of anti-drug antibodies

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|-----------------|---------------------------------------------------------|
| End point title | 11. Evaluation of the incidence of anti-drug antibodies |
|-----------------|---------------------------------------------------------|

End point description:

| | |
|----------------|-----------|
| End point type | Secondary |
|----------------|-----------|

End point timeframe:

From Day 1 until Day 78.

| End point values | ARGX-113 | Placebo | | |
|------------------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Subjects | | | | |
| Subjects with pre-dose ADA titers | 4 | 2 | | |
| Subjects with post-dose ADA titers | 4 | 3 | | |

Statistical analyses

No statistical analyses for this end point

Post-hoc: 12. Percentage of patients with sustained clinically relevant improvement (drop in MG-ADL score ≥ 2)

| | |
|-----------------|------------------------------------------------------------------------------------------------------------|
| End point title | 12. Percentage of patients with sustained clinically relevant improvement (drop in MG-ADL score ≥ 2) |
|-----------------|------------------------------------------------------------------------------------------------------------|

End point description:

Sustained clinically relevant improvement is defined in this case as starting the latest 1 week after last infusion of IMP and lasting for ≥ 4 consecutive weeks.

| | |
|----------------|----------|
| End point type | Post-hoc |
|----------------|----------|

End point timeframe:

For the duration of the study

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Percentage | | | | |
| number (not applicable) | 75.0 | 33.3 | | |

Statistical analyses

No statistical analyses for this end point

Post-hoc: 13. Percentage of patients with sustained clinically relevant improvement (drop in MG-ADL \geq 2)

| | |
|-----------------|-----------------------------------------------------------------------------------------------------|
| End point title | 13. Percentage of patients with sustained clinically relevant improvement (drop in MG-ADL \geq 2) |
|-----------------|-----------------------------------------------------------------------------------------------------|

End point description:

Sustained clinically relevant improvement is defined as starting at the latest 1 week after last infusion of IMP and lasting for \geq 6 weeks.

| | |
|----------------|----------|
| End point type | Post-hoc |
|----------------|----------|

End point timeframe:

For the duration of the study.

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Percentage | | | | |
| number (not applicable) | 75 | 25 | | |

Statistical analyses

No statistical analyses for this end point

Post-hoc: 14. Percentage of patients showing at least x points reduction in total MG-ADL score at Day 29

| | |
|-----------------|------------------------------------------------------------------------------------------------|
| End point title | 14. Percentage of patients showing at least x points reduction in total MG-ADL score at Day 29 |
|-----------------|------------------------------------------------------------------------------------------------|

End point description:

| | |
|----------------|----------|
| End point type | Post-hoc |
|----------------|----------|

End point timeframe:

At Day 29

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Percentage | | | | |
| number (not applicable) | | | | |
| Change from baseline: -2 | 83 | 42 | | |
| Change from baseline: -3 | 75 | 33 | | |
| Change from baseline: -4 | 58 | 33 | | |
| Change from baseline: -5 | 42 | 25 | | |
| Change from baseline: -6 | 25 | 17 | | |
| Change from baseline: -7 | 25 | 8 | | |
| Change from baseline: -8 | 17 | 0 | | |
| Change from baseline: -9 | 0 | 0 | | |
| Change from baseline: -10 | 0 | 0 | | |

Statistical analyses

No statistical analyses for this end point

Post-hoc: 15. Percentage of patients showing at least x points reduction in total MG-ADL score at Day 36

| | |
|-----------------|------------------------------------------------------------------------------------------------|
| End point title | 15. Percentage of patients showing at least x points reduction in total MG-ADL score at Day 36 |
|-----------------|------------------------------------------------------------------------------------------------|

End point description:

| | |
|----------------|----------|
| End point type | Post-hoc |
|----------------|----------|

End point timeframe:

Day 36

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Percentage | | | | |
| number (not applicable) | | | | |
| Change from baseline: -2 | 75 | 50 | | |
| Change from baseline: -3 | 67 | 33 | | |
| Change from baseline: -4 | 50 | 25 | | |
| Change from baseline: -5 | 42 | 25 | | |
| Change from baseline: -6 | 33 | 17 | | |
| Change from baseline: -7 | 33 | 0 | | |
| Change from baseline: -8 | 17 | 0 | | |
| Change from baseline: -9 | 8 | 0 | | |
| Change from baseline: -10 | 8 | 0 | | |

Statistical analyses

No statistical analyses for this end point

Post-hoc: 16. Percentage of patients with sustained clinically relevant improvement (drop in QMG score ≥ 3)

| | |
|-----------------------------------------------------------------------------------------------------------------------------------------------------------------|---------------------------------------------------------------------------------------------------------|
| End point title | 16. Percentage of patients with sustained clinically relevant improvement (drop in QMG score ≥ 3) |
| End point description: Sustained response is defined as starting at the latest 1 week after last infusion of IMP and lasting for ≥ 4 consecutive weeks. | |
| End point type | Post-hoc |
| End point timeframe: For the duration of the study. | |

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Percentage | | | | |
| number (not applicable) | 58.3 | 16.7 | | |

Statistical analyses

No statistical analyses for this end point

Post-hoc: 17. Percentage of patients showing at least x points reduction in total QMG score at Day 29

| | |
|--------------------------------|---------------------------------------------------------------------------------------------|
| End point title | 17. Percentage of patients showing at least x points reduction in total QMG score at Day 29 |
| End point description: | |
| End point type | Post-hoc |
| End point timeframe: Day 29 | |

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-------------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 11 ^[5] | | |
| Units: Percentage | | | | |
| number (not applicable) | | | | |
| Change from baseline: -3 | 58 | 27 | | |
| Change from baseline: -4 | 58 | 27 | | |
| Change from baseline: -5 | 50 | 18 | | |
| Change from baseline: -6 | 42 | 18 | | |

| | | | | |
|---------------------------|----|----|--|--|
| Change from baseline: -7 | 25 | 18 | | |
| Change from baseline: -8 | 25 | 9 | | |
| Change from baseline: -9 | 25 | 0 | | |
| Change from baseline: -10 | 17 | 0 | | |
| Change from baseline: -11 | 8 | 0 | | |
| Change from baseline: -12 | 8 | 0 | | |

Notes:

[5] - missing value in 1 patient

Statistical analyses

No statistical analyses for this end point

Post-hoc: 18. Percentage of patients showing at least x points reduction in total QMG score at Day 36

| | |
|-----------------|---------------------------------------------------------------------------------------------|
| End point title | 18. Percentage of patients showing at least x points reduction in total QMG score at Day 36 |
|-----------------|---------------------------------------------------------------------------------------------|

End point description:

| | |
|----------------|----------|
| End point type | Post-hoc |
|----------------|----------|

End point timeframe:

Day 36

| End point values | ARGX-113 | Placebo | | |
|-----------------------------|-----------------|-----------------|--|--|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 12 | 12 | | |
| Units: Percentage | | | | |
| number (not applicable) | | | | |
| Change from baseline: -3 | 58 | 33 | | |
| Change from baseline: -4 | 50 | 25 | | |
| Change from baseline: -5 | 42 | 17 | | |
| Change from baseline: -6 | 33 | 17 | | |
| Change from baseline: -7 | 33 | 8 | | |
| Change from baseline: -8 | 33 | 8 | | |
| Change from baseline: -9 | 25 | 8 | | |
| Change from baseline: -10 | 25 | 8 | | |
| Change from baseline: -11 | 25 | 0 | | |
| Change from baseline: -12 | 25 | 0 | | |

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

For each subject, adverse events were recorded from the time of signing the informed consent form until last visit.

| | |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

Dictionary used

| | |
|--------------------|--------|
| Dictionary name | MedDRA |
| Dictionary version | 19.1 |

Reporting groups

| | |
|-----------------------|----------|
| Reporting group title | ARGX-113 |
|-----------------------|----------|

Reporting group description: -

| | |
|-----------------------|---------|
| Reporting group title | Placebo |
|-----------------------|---------|

Reporting group description: -

| Serious adverse events | ARGX-113 | Placebo | |
|---------------------------------------------------|----------------|----------------|--|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 0 / 12 (0.00%) | 0 / 12 (0.00%) | |
| number of deaths (all causes) | 0 | 0 | |
| number of deaths resulting from adverse events | 0 | 0 | |

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

| Non-serious adverse events | ARGX-113 | Placebo | |
|-------------------------------------------------------|------------------|------------------|--|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 10 / 12 (83.33%) | 10 / 12 (83.33%) | |
| Investigations | | | |
| B-lymphocyte count decreased | | | |
| subjects affected / exposed | 2 / 12 (16.67%) | 0 / 12 (0.00%) | |
| occurrences (all) | 3 | 0 | |
| Lymphocyte count decreased | | | |
| subjects affected / exposed | 2 / 12 (16.67%) | 0 / 12 (0.00%) | |
| occurrences (all) | 4 | 0 | |
| Monocyte count decreased | | | |
| subjects affected / exposed | 2 / 12 (16.67%) | 0 / 12 (0.00%) | |
| occurrences (all) | 4 | 0 | |
| Neutrophil count increased | | | |

| | | | |
|--------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------|------------------------------------------------------------------------------------------------------|-------------------------------------------------------------------------------------------------------|--|
| subjects affected / exposed occurrences (all) | 2 / 12 (16.67%) 4 | 0 / 12 (0.00%) 0 | |
| Nervous system disorders Headache subjects affected / exposed occurrences (all) | 4 / 12 (33.33%) 9 | 3 / 12 (25.00%) 5 | |
| Gastrointestinal disorders Abdominal pain upper subjects affected / exposed occurrences (all) Diarrhoea subjects affected / exposed occurrences (all) Nausea subjects affected / exposed occurrences (all) Toothache subjects affected / exposed occurrences (all) | 1 / 12 (8.33%) 1 1 / 12 (8.33%) 1 1 / 12 (8.33%) 1 0 / 12 (0.00%) 0 | 1 / 12 (8.33%) 1 1 / 12 (8.33%) 3 1 / 12 (8.33%) 1 2 / 12 (16.67%) 2 | |
| Respiratory, thoracic and mediastinal disorders Rhinorrhoea subjects affected / exposed occurrences (all) | 1 / 12 (8.33%) 2 | 1 / 12 (8.33%) 1 | |
| Skin and subcutaneous tissue disorders Pruritus subjects affected / exposed occurrences (all) | 1 / 12 (8.33%) 1 | 2 / 12 (16.67%) 2 | |
| Musculoskeletal and connective tissue disorders Arthralgia subjects affected / exposed occurrences (all) Myalgia subjects affected / exposed occurrences (all) | 0 / 12 (0.00%) 0 2 / 12 (16.67%) 2 | 2 / 12 (16.67%) 2 0 / 12 (0.00%) 0 | |
| Infections and infestations | | | |

| | | | |
|-------------------------------------------------------------------|---------------------|----------------------|--|
| Tooth abscess subjects affected / exposed occurrences (all) | 0 / 12 (0.00%) 0 | 2 / 12 (16.67%) 3 | |
|-------------------------------------------------------------------|---------------------|----------------------|--|

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported