STATISTICAL ANALYSIS PLAN

A Long-Term, Single-Arm, Open-Label, Multicenter, Phase 3 Follow-on Trial of ARGX-113-1704 to Evaluate the Safety and Tolerability of ARGX-113 in Patients With Myasthenia Gravis Having Generalized Muscle Weakness

Protocol:	ARGX-113-1705
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Development phase:	Phase 3
Sponsor:	argenx
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SIGNATURE PAGE

Name and function	Date and Signature (ddMMMyyyy)
SGS CR author:	
[redacted], Biostatistical Coordinator	[redacted]
Sponsor's approval: The approver agrees the statistical analysis will be performed according to this statistical analysis plan.	
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PROTOCOL HISTORY

Protocol:		
Version or ID	**Date (ddMMMyyyy)**	**Impact of the changes on the statistical analysis**
Final 1.0	15JUN2018	NAP
Final 2.0	12AUG2019	NAP
Final 3.0	18DEC2019	Addition of part B - 2 years extension
Final 2.1	15APR2020	NAP
Final 3.1	15APR2020	NAP
Final 2.2	30JUN2020	NAP
Final 3.2	30JUN2020	NAP
Final 4.0	19JAN2021	Patients can enrol in SC study ARGX-113-2002 - no impact on analysis; collection of vaccination history - no impact on analysis; ECG assessment added to part B

Protocol amendments:		
Version or ID	**Date (ddMMMyyyy)**	**Applicable country of the amendment**
Amendment 1.1	04DEC2018	UK specific amendment
Amendment 1.1	28DEC2018	Japan specific amendment
Amendment 1.1	18JAN2019	Czech Republic specific amendment
Amendment 1.1	08FEB2019	The Netherlands specific amendment
Amendment 1.2	25MAR2019	The Netherlands specific amendment
Amendment 2.1	30AUG2019	UK, The Netherlands and Czech Republic specific amendment
Amendment 2.2	19SEP2019	Japan specific amendment
Amendment 2.3	06NOV2019	Japan specific amendment
Amendment 3.1	14FEB2020	Japan specific amendment
Amendment 3.1	18MAR2020	Czech Republic specific amendment
Amendment 3.1	21MAR2020	Denmark specific amendment
Amendment 3.1	03APR2020	The Netherlands specific amendment
Amendment 2.2	30APR2020	The Netherlands and Czech Republic specific amendment

Amendment 3.2	30APR2020	The Netherlands, Japan, Denmark and Czech Republic specific amendment
Amendment 2.4	30APR2020	Japan specific amendment
Amendment 3.3	08JUL2020	The Netherlands, Japan, Denmark and Czech Republic specific amendment
Amendment 4.1	19JAN2021	The Netherlands, Germany, Japan, Denmark and Czech Republic specific amendment
Amendment 4.2	25JUN2021	Germany specific amendment

This statistical analysis plan (SAP) only considers the latest version of the protocol, and of the protocol amendments, as listed above.

LIST OF ABBREVIATIONS

1704	ARGX-113-1704
1705	ARGX-113-1705
Ab	Antibody
AChR	Acetylcholine receptor
ADA	anti-drug antibody
ADaM	analysis data model
ADYP	relative day in period
AE	adverse event
AESI	adverse event of special interest
ALQ	above limit of quantification
BLQ	below limit of quantification
bpm	beats per minute
CI	confidence interval
CKD-EPI	Chronic Kidney Disease Epidemiology Collaboration
CnB	cycle n baseline
CRF	case report form
DBP	diastolic blood pressure
DSMB	data safety monitoring board
ECG	electrocardiogram
eGFR	estimated glomerular filtration rate
EoA	end of part A of the study
EU	European Union
gMG	generalized myasthenia gravis
HR	heart rate
ICF	informed consent form
ICH	International Council for Harmonisation
IMP	investigational medicinal product
ITP	inter treatment period
ITT	Intent-To-Treat
J-NDA	Japan new drug application
LLOQ	lower limit of quantification
mITT	Modified Intent-To-Treat

MAA	marketing authorisation application
MedDRA	Medical Dictionary for Regulatory Activities
MG-ADL	myasthenia gravis - activities of daily living
MuSK	muscle-specific kinase
NAP	not applicable
OLE	open-label extension
PD	pharmacodynamics
PYFU	patient years of follow-up
QTc	corrected QT interval
QTcB	Bazett's corrected QT interval
QTcF	Fridericia's corrected QT interval
RO	roll-over
SAP	statistical analysis plan
SAF	safety analysis set
SBP	systolic blood pressure
SD	standard deviation
SE	standard error
SGS CR	SGS Clinical Research
SoA	schedule of assessments
SOP	standard operating procedure
STAT	statistics
TEAE	treatment-emergent adverse event
ULOQ	upper limit of quantification
VS	vital signs
WHO	World Health Organisation
WI	work instruction

DEFINITION OF TERMS

AChR-Ab seronegative patients	AChR-Ab seronegative patients refers to patients in whom the anti-AChR-Ab cannot be detected as stratified in 1704
case report form (CRF)	A printed, optical, or electronic document designed to record protocol required information to be reported to the sponsor for each trial subject.
display	Analysis table, figure or listing
phase	Interval of time in the planned conduct of a study associated with a specific purpose: for example, screening, treatment, follow-up.
study drug	Pharmaceutical form of an active ingredient or placebo, being tested or used as a reference in a clinical study.
treatment-emergent abnormality/ toxicity	Any post-baseline abnormality/toxicity that was not present at baseline (e.g. haemoglobin normal at baseline and grade 1 post-baseline; glucose low at baseline and high post-baseline; QTcFri]450; 480] ms at baseline and >500 ms post-baseline)

TABLE OF CONTENTS

1. INTRODUCTION

This SAP describes the final statistical analysis to be performed for the ARGX-113-1705 (BE-80-1801535) study.

This SAP covers the safety, efficacy, PD, immunogenicity and general characteristics parts of the statistical analysis. It specifies the analysis displays to be presented and describes the methods and procedures in a more elaborated way than in the statistical methods section of the protocol.

The statistical analysis will process and present the results following the International Council for Harmonisation (ICH) standards, in particular the ICH-E3, ICH-E6, and ICH-E9 guidelines.

1.1 STUDY OBJECTIVES

According to the protocol, the primary objective of this study is:

- To evaluate the long-term safety and tolerability of ARGX-113 in acetylcholine receptor antibody (AChR-Ab) seropositive patients.

According to the protocol, the secondary objective of this study is:

- To evaluate the long-term safety and tolerability of ARGX-113 in the overall population (AChR-Ab seropositive and AChR-Ab seronegative patients).

According to the protocol, the tertiary objectives of part A of this study are:

- To evaluate the disease severity as assessed by total Myasthenia Gravis Activities of Daily Living (MG-ADL) score changes in AChR-Ab seropositive patients.
- To evaluate the disease severity as assessed by total MG-ADL score changes in the overall population (AChR-Ab seropositive patients and AChR-Ab seronegative patients).
- To evaluate disease severity as assessed by total Quantitative Myasthenia Gravis (QMG) score changes in AChR-Ab seropositive patients.
- To evaluate disease severity as assessed by total QMG score changes in the overall population (AChR-Ab seropositive patients and AChR-Ab seronegative patients).
- To evaluate the effect of ARGX-113 on pharmacodynamics (PD) (total immunoglobulin G [IgG], IgG subtypes, autoantibodies [anti-AChR antibodies and anti-muscle-specific kinase (MuSK) antibodies]).

According to the protocol, the tertiary objectives of part A and B of this study is:

- To evaluate the immunogenicity of ARGX-113.

1.2 STUDY DESIGN

This is a phase 3 multicentre, single arm, Open-Label Extension (OLE), Follow-on study of ARGX-113-1704 assessing the long-term safety and tolerability of (intravenous) ARGX-113 in adults with generalized myasthenia gravis (gMG).

At the End of Study visit of trial ARGX-113-1704, eligible patients were offered the option to roll over into this trial. Hence, the maximum number of patients in this trial is the number of patients who participated in trial ARGX-113-1704.

Within trial ARGX-113-1705, a variable number of Treatment periods (i.e. 4 weekly infusions of ARGX-113 infused over a period of 3 weeks) were administered to eligible patients on an "as needed basis" on top of their SoC in Treatment Periods.

The anticipated study duration was a maximum of 3 year after entry into the OLE. After the first year (part A), the number and frequency of assessments decreased for the remaining time in the study (part B). During part B, patients had the opportunity to enter study ARGX-113-2002 to be treated with subcutaneous ARGX-113 and PH20.

The schedule of assessments is in appendix 9.2.

1.3 EXPECTED SAMPLE SIZE

The maximum number of patients in ARGX-113-1705 is the number of patients who participated in trial ARGX-113-1704 which is 167.

1.4 RANDOMISATION AND BLINDING

Not applicable.

1.5 INTERIM ANALYSIS

Interim analyses were performed. These are described in separate SAPs.

1.6 SOFTWARE

SAS version 9.4 or later will be used for programming.

1.7 VALIDATION MODEL

SGS statistics (STAT) standard operating procedures (SOPs) and work instructions (WIs) as effective at the project start will be followed throughout the project, provided the applicable regulatory requirements are still met.

Analysis Data Model (ADaM) datasets, analysis tables and listings will be validated according to model B: review by an independent person.

2. GENERAL METHODOLOGY

2.1 ANALYSIS SETS

2.1.1 Analysis sets

The following analysis sets will be considered in the statistical analysis:

Roll-over set (RO):	patients who rolled over from the 1704 study
Safety analysis set (SAF):	all patients who received at least one dose or part of a dose.

All analyses will be performed on the safety analysis set.

A patient is considered to have rolled over if he has signed the ARGX-113-1705 specific informed consent, defined as having a complete informed consent signature date in the database.

The AChR-Ab seropositive subset population is defined based on the stratification factor as randomized in the 1704 study.

2.1.2 As planned versus as actual analysis

The actual treatment of the patient will be considered for both 1704 and 1705 studies (see section 2.4.2).

2.2 PHASES, PERIODS AND TIME POINTS

2.2.1 Phases and periods

All events and assessments will be allocated to phases and periods (see Table 1). A patient can start in 1705 in an inter-treatment period or in a cycle, depending on the patient status regarding the need for (re-)treatment upon roll-over. Each period following ITP0 coincides with a cycle and includes the 4 administrations of efgartigimod and the inter-treatment period (ITP) visits. The length of the ITP is patient specific and can differ between the different periods. The number of patients may decline for each subsequent cycle.

Table 1: phase/period definition

Phase	Period	Start	End
Treatment	ITP0	Earliest of (date of signing the informed consent form (ICF) and date of roll-over), with 00:00 added as time part.	First administration date/time in cycle 1 – 1 minute
	Cycle 1	First administration date/time in cycle 1	First administration date/time in next cycle – 1 minute or if last cycle: date of last contact, with 23:59 added as time part
	Cycle n	First administration date/time in cycle n	First administration date/time in next cycle – 1 minute or if last cycle: date of last contact, with 23:59 added as time part
	Cycle x	First administration date/time in cycle x	Date of last contact, with 23:59 added as time part

AEs and concomitant medications will be allocated to phases and periods as described in sections 5.1.2 and 3.5.2 respectively. All other assessments will be allocated to phases and periods based on the assessment date/time.

In case of (partially) missing date/time fields disabling allocation or date(time) equal to dosing date(time), information from visit label and protocol schedule of assessments (SoA) will be used to allocate to the correct period. If this is not possible, assessments will be allocated to the first possible cycle (not ITP0) unless the available parts of the assessments start or stop date/time provide evidence the assessments did not occur during that period.

2.2.2 Baseline and change from baseline

Two baselines are defined:

- Baseline is the last available value prior to first administration of efgartigimod in the first cycle in 1705.
- CnB: Cycle n Baseline i.e. last available value prior to first administration of efgartigimod in cycle n, with n=1, 2, 3, etc..

Note: for MG-ADL and QMG, all assessments on the day of first administration of the IMP in cycle n are considered in the baseline selection, also those post-administration.

Change can be calculated from baseline or CnB. Note: baseline coincides with C1B. Baseline will be considered for analyses that assess across all cycles and the CnB will be used for analyses restricted to a specific cycle, n.

Change for safety parameters will be calculated from baseline only.

For efficacy and PD, a (percent) change can be calculated from baseline or CnB, according to the analysis.

Change from baseline at time point t = value at time point t – baseline value.

Percent change from baseline at time point t = (actual value at time point t -baseline value)*100/baseline value.

Change from CnB at time point t = value at time point t – CnB value.

Percent change from CnB at time point t = (actual value at time point t - CnB value)*100/CnB value.

2.2.3 Relative day

Relative days in the period (ADYP) will be calculated according to the following rule:

- Concerned date < reference date: ADYP = concerned date – reference date
- Concerned date ≥ reference date: ADYP = concerned date – reference date + 1

The reference date is the date of first administration of study drug in the specific period, except for assessments in ITP0, for which the date of informed consent in 1705 will be taken as reference date.

2.2.4 Analysis visits

All assessments (from first ARGX-113-1705 cycle on), including unscheduled assessments, will be allocated to analysis windows. Tables and listings will present the analysis windows as defined below, not the CRF visits. Allocation of assessments will be done using their relative day in the period (see section 2.2.3).

Table 4: Analysis visits

Phase/Period	Analysis window	Target ADYP	Lower limit ADYP	Upper limit ADYP
Treatment/ cycle n (=Treatment Period n + ITP_n)				
	CnB	1	-INF	1*
	Week 1	8	1*	11
	Week 2	15	12	18
	Week 3	22	19	35
	Week 7	50	36	63
	Week 7 + (4*x)	50 + (x*28)	50 + (x*28) - 14	50 + (x*28) + 13

* An assessment on day 1 will be attributed to CnB in case it is before the infusion, to week 1 otherwise, except for MG-ADL and QMG (see section 2.2.2)

Baseline is defined in section 2.2.2.

For those patients who will start a new cycle, the last assessment in a cycle, prior to the infusion of the new cycle, will be allocated to the appropriate visit within the previous cycle and will also be allocated as the CB visit of the new cycle.

Per parameter and analysis window, the value closest to the target ADYP will be used in analysis tables and figures, other values will only be listed. If more than one value is located at the same distance from the target, then the latest in time will be selected.

The value latest in time will be identified using, in order of preference, the assessment time, the visit label or the group identifier. Missing values are removed before the selection is made.

2.2.5 Worst-case

A worst-case analysis visit will be created for parameters for which abnormalities and/or toxicity grades are defined to summarize values considered as the worst-case. For abnormalities it is derived per parameter and in case both the lowest and the highest values are considered abnormal, a patient can have two worst-case analysis visits for a same parameter. For toxicity grades the worst-case is the value associated to the highest toxicity grade and is derived per parameter and toxicity direction (hypo / hyper).

All non-missing post-baseline values, including unscheduled assessments will be considered when deriving the worst-case analysis visit.

2.3 IMPUTATION AND ROUNDING RULES

2.3.1 Missing values

No imputation will be done of missing values (i.e. observed cases analysis).

2.3.2 Values below or above a threshold

Safety values expressed as below the detection limit will be imputed by the value of the detection limit itself. Listings will always show the non-imputed values.

2.3.3 Rounding of variables

Variables will be rounded to the appropriate number of decimals at display level:

- Time since diagnosis will be rounded to 1 decimal.
- Estimated glomerular filtration will be rounded to 2 decimals
- Ratios will be rounded to the number of decimals of the parameter with the least number of decimals.
- Safety laboratory results will be rounded to a maximum of 3 decimal.

2.3.4 Outliers

There will be no outlier detection. All measured values will be included in the analyses.

2.4 PRESENTATION OF RESULTS

2.4.1 Calculation of descriptive statistics and percentages

In tables by analysis visit, only analysis visits with at least 10 subjects (overall) will be shown.

For continuous variables, full descriptive statistics will only be presented if there are at least 2 non-missing observations. Alternatively, only the number of non-missing data points and mean are shown.

Descriptive statistics will include the number of non-missing data points, the arithmetic mean, the standard deviation (SD), the median, minimum, Q1, Q3 and maximum.

Mean, median, Q1 and Q3 will be presented with one more decimal place than the individual values. SD will be presented with two more decimal places than the individual values. Minimum and maximum will be presented with the same number of decimal places as the individual values.

For event-type safety data, the number and percentage of patients with an event will be shown. The denominator will be all patients in the analysis set per treatment and cycle. All cycles will be shown, even if no events are present.

For frequency tabulations and cross-tabulations, the denominator will be all patients in the analysis set per treatment. For tables where results are shown by analysis visit, the denominator will be all patients in the analysis set per treatment and per analysis visit. Missing values will never be included in the denominator count when computing percentages. For cross-tabulations of post-baseline results versus baseline results, a 'missing' category will be shown for baseline results if applicable.

2.4.2 Presentation of treatments

The following treatment labels will be used in the tables and listings:

- efgartigimod
- placebo-efgartigimod
- Total efgartigimod

Total efgartigimod will be shown last.

2.4.3 Ordering in tables, figures and listings

For presentations by analysis visits, this analysis will only tabulate data collected from the first OLE cycle on. In case a patient starts with ITP0 visits, these will not be considered in the tables. Part B data will not be tabulated separately but will be allocated to cycles like part A data.

All tables will be presented per treatment and cycle, unless specified otherwise. If present, worst-case will be shown last. Cycles will be indicated as a subtitle or within the table like "Cycle n".

Listings for general characteristics, results will be ordered by treatment and patient, unless specified otherwise.

All other listings will be ordered by treatment, patient, period, analysis visit and time point, unless specified otherwise.

In tables showing several parameters, each parameter will begin on a new page and parameters will be sorted alphabetically, within the parameter category if applicable.

Efgartigimod-efgartigimod group will always be shown first and then Placebo-efgartigimod.

3. GENERAL CHARACTERISTICS ANALYSES

3.1 SUBJECT DISPOSITION

The following patient data will be tabulated:

- The number of patients in each analysis set
- The number and percentage of patients by country and investigator
- The number and percentage of patients for each period/analysis visit.
- The number and percentage of patients for each period/analysis visit who had a missed visit and who had a phone call visit due to COVID-19 (as documented in the CRF comment).
- Descriptive statistics and tabulation in weeks of the cycle duration (see section 2.2.1), calculated as cycle end date – cycle start date + 1 day
- Descriptive statistics of the study duration, calculated as study end date – study start date + 1 day.
- Frequency tabulation of the study duration per 6-monthly period, showing the number and percentage in each category and additionally cumulative number and percentages. Categories are defined as follows: <6 months (< 168 days); 6 to <12 months (168 - 350 days); 12 to <18 months (351 - 532 days); 18 to <24 months (533 - 715 days); 24 to <30 months (716 - 897 days); 30 to <36 months (898 - 1080 days); 36 to <42 months (1081 - 1262 days).
- The number and percentage of patients who completed or discontinued the study as documented on the study termination page and the number and percentage of patients for each study discontinuation reason.
- The number and percentage of patients who completed or discontinued the study as documented on the study termination page and the number and percentage of patients for each study discontinuation reason by cycle.
- The number and percentage of patients who completed or discontinued the treatment as documented on the end of treatment page and the number and percentage of patients for each treatment discontinuation reason.
- The number and percentage of patients who completed or discontinued the treatment as documented on the end of treatment page and the number and percentage of patients for each treatment discontinuation reason by cycle.

All information collected in the CRF concerning study and treatment discontinuation will be listed.

3.2 PROTOCOL DEVIATIONS

The number and percentage of patients with major protocol deviations will be tabulated, overall and per class of deviation.

All available information concerning major protocol deviations will be listed.

3.3 DEMOGRAPHIC AND OTHER BASELINE CHARACTERISTICS

3.3.1 Available data

The following parameters will be collected during the roll-over visit:

- Demographics: sex at birth, women of childbearing potential, age, race, ethnicity, height, weight at screening, body mass index at screening, date of birth, date of signing informed consent form (IC).
- Baseline disease characteristics: date of diagnosis, MGFA Classification at 1704 screening and at diagnosis, receiving NSID as SoC (yes/no) at 1704 screening, AChR-Ab status (1704), MuSK-Ab status (1704), MG-ADL questionnaire (Total MG-ADL Score) at baseline (see 2.2.2), QMG (Total QMG Score) at baseline (see 2.2.2).

3.3.2 Derivation rules

The following parameters will be derived:

- Age category: 18-64 years, ≥ 65 years
- Japanese vs non-Japanese (including Hispanic or Latino, Not Hispanic or Latino and Not allowed to ask per local regulations)
- Region: Japan; US; rest of the world
- Time since diagnosis (years): (date of ICF – date of diagnosis)/365.25. Partially missing date of diagnoses will be imputed as follows:
  - Missing start day will be imputed with 1
  - Missing start day and month will be imputed with 1JAN

  Note: Result will be rounded as detailed in section 2.3.3.

3.3.3 Presentation of results

Demographics will be presented using descriptive statistics for age, height, weight and BMI and frequency tabulations for age category, sex at birth, race, region, ethnicity, Japanese/non-Japanese patients.

Baseline disease characteristics will be presented using descriptive statistics for time since diagnosis, total MG-ADL score and total QMG score and frequency tabulations for MGFA Classification (at 1704 screening and at diagnosis), AChR-Ab and MuSK status in 1704 and Standard of Care (SoC).

All demographic data and baseline disease characteristics will be listed.

3.4 MEDICAL HISTORY AND CONCOMITANT DISEASES

3.4.1 Available data

Medical history findings are coded using the medical dictionary for regulatory activities (MedDRA), version 24.1 (SEP2021) into system organ classes and preferred terms. For each finding, a start and stop date or ongoing flag is collected.

3.4.2 Derivation rules

Medical history finding: not ongoing at roll-over visit, ended before date of signing informed consent.

3.4.3 Presentation of results

Medical history (not ongoing at roll-over visit) and concomitant diseases (still ongoing at roll-over visit) will be tabulated in a separate table. Both tables will show:

- The number and percentage of patients with findings
- The number and percentage of patients with findings by system organ class and preferred term

All medical history data will be listed.

3.5 PRIOR AND CONCOMITANT THERAPIES

3.5.1 Available data

All therapies are coded using the March 2020 version of the WHO-DRUG. ATC selection is performed. ATC coding up to level 4 is available in the clinical database. For each therapy, a start date or prior flag and stop date or ongoing flag are collected.

3.5.2 Derivation rules

Based on their start and stop date, therapies will be allocated to each analysis period during which they were administered. A therapy can therefore be reported in more than one analysis period.

Analysis periods are defined in section 2.2.1. Therapies with (partially) missing dates will be allocated to each period unless the available parts of the therapy start or stop date or prior and ongoing flags provide evidence the therapy was not taken during that period.

Concomitant therapies are defined as therapies taken on or after the first OLE dose date.

3.5.3 Presentation of results

Concomitant therapies will be tabulated, by ATC class (level 1 and level 3) and generic term. This table will be provided overall, not by cycle.

All concomitant therapies data will be listed with detailed information about ATC classes.

3.6 STUDY DRUG ADMINISTRATION

3.6.1 Available data

For each study drug administration, the start and end date/times and the volumes will be recorded.

3.6.2 Derivation rules

The following parameters will be derived:

- Number of administrations: number and percentage of patients who had 1, 2, 3 etc.… administrations per cycle and overall
- Actual dose (mg/kg) per administration per cycle, using categories <9mg/kg, 9-11 mg/kg, >11mg/kg.
- Treatment compliance per cycle defined as (number of doses received/4)*100%
- The actual dose in mg/kg will be calculated as = $\left(\frac{\text{actual volume extracted from vials (mL)} * 20\ (\text{mg/mL})}{\text{actual volume extracted from vials (mL)} + \text{actual volume of NaCl solution added to IV bag (mL)}}\right) * \left(\frac{\text{actual volume infused (mL)}}{\text{last available patient weight (kg) before or at day of dosing}}\right)$
- Note: As per protocol a variation of ± 10% of the amount of 10 mg/kg, will not be considered an overdose/under dose.

3.6.3 Presentation of results

A frequency table for the number of administrations per cycle and overall, and the actual dose per administration will be created.

Descriptive statistics of the overall number of administrations and treatment compliance.

All study drug administration data will be listed.

4. EFFICACY, PHARMACODYNAMICS AND ANTI-DRUG ANTIBODIES ANALYSES

4.1 EFFICACY

4.1.1 Available data

Efficacy will be assessed using MG-ADL (part A and B) and QMG (part A only).

4.1.2 Endpoints and derivation rules

1) Actual values and change from baseline and CnB in total MG-ADL score will be analysed descriptively and categorically.

Categories to be used for actual values in MG-ADL total score: 0, 1, 2, 3, 4, 5-7, 8-9, 10-12, 13-16, 17-20 and 21-24.

Categories to be used for changes from baseline and CnB in MG-ADL total score: >0, 0, -1, -2, -3, -4, -5, -6, -7, -8, -9, -10, <-10. Number of patients, percentages and cumulative percentages will be shown.

In addition to the planned timepoints, following timepoints will be shown for each cycle:

- Maximum drop from baseline and CnB in MG-ADL
- Minimum MG-ADL score: descriptively and categorically

Missing data will not be considered.

Analysis will be done on AChR-Ab seropositive patients, AChR-Ab seronegative patients and overall.

2) Actual values and change from baseline and CnB in total QMG score will be described descriptively and categorically as for the MG-ADL score. For the categorization of the QMG total score actual values a category of >24 will be added.

4.1.3 Statistical analysis

Summary statistics will be provided in terms of absolute values and changes from baseline (overall) and CnB (for each cycle) for MG-ADL and QMG total scores in AChR-Ab seropositive patients, AChR-Ab seronegative patients and the overall safety population.

Absolute values and changes from baseline (overall) and CnB (for each cycle) in MG-ADL and QMG total scores will also be categorized, including the number of patients within each category, the percentage, and the cumulative percentage.

See section 8 for a list of tables and listings.

4.2 PHARMACODYNAMICS

4.2.1 Available data

The following pharmacodynamic parameters are collected during part A of the study and will be analysed: PD markers (total IgG and IgG subtypes [IgG1, IgG2, IgG3 and

IgG4], and autoantibodies (anti-AChR antibodies for the AChR-Ab seropositive patients and anti-MuSK antibodies)

4.2.2 Endpoint and derivation rules

1) Change and percent change (compared to Baseline and CnB) in total IgG level and IgG subtypes [IgG1, IgG2, IgG3 and IgG4] at each visit.

Analysis will be done by AChR-Ab status and overall.

2) Change and percent change (compared to Baseline and CnB) in anti-AChR antibodies (in AChR-Ab seropositive patients) at each visit.

See section 2.2.2 for calculation of change and percent change from baseline.

Values Below the limit of quantification (BLQ) will be imputed with the lower limit of quantification. Values Above the limit of quantification (ALQ) will be excluded from the statistical analysis and will thus also not be considered for the baseline selection. Patient with a baseline value BLQ will be excluded from the statistical analysis and only be listed.

4.2.3 Statistical analysis

Summary statistics will be provided in terms of absolute values and changes from baseline and CnB for each treatment cycle. Moreover, percent changes from baseline and CnB will also be presented.

All data will be listed.

4.3 ANTI-DRUG ANTIBODIES

4.3.1 Available data

During part A of the study, presence of anti-drug antibodies (ADA) to efgartigimod is measured at the roll-over visit, at baseline and week 3 of each cycle and at EoA. During part B, ADA is measured at baseline and EoS only.

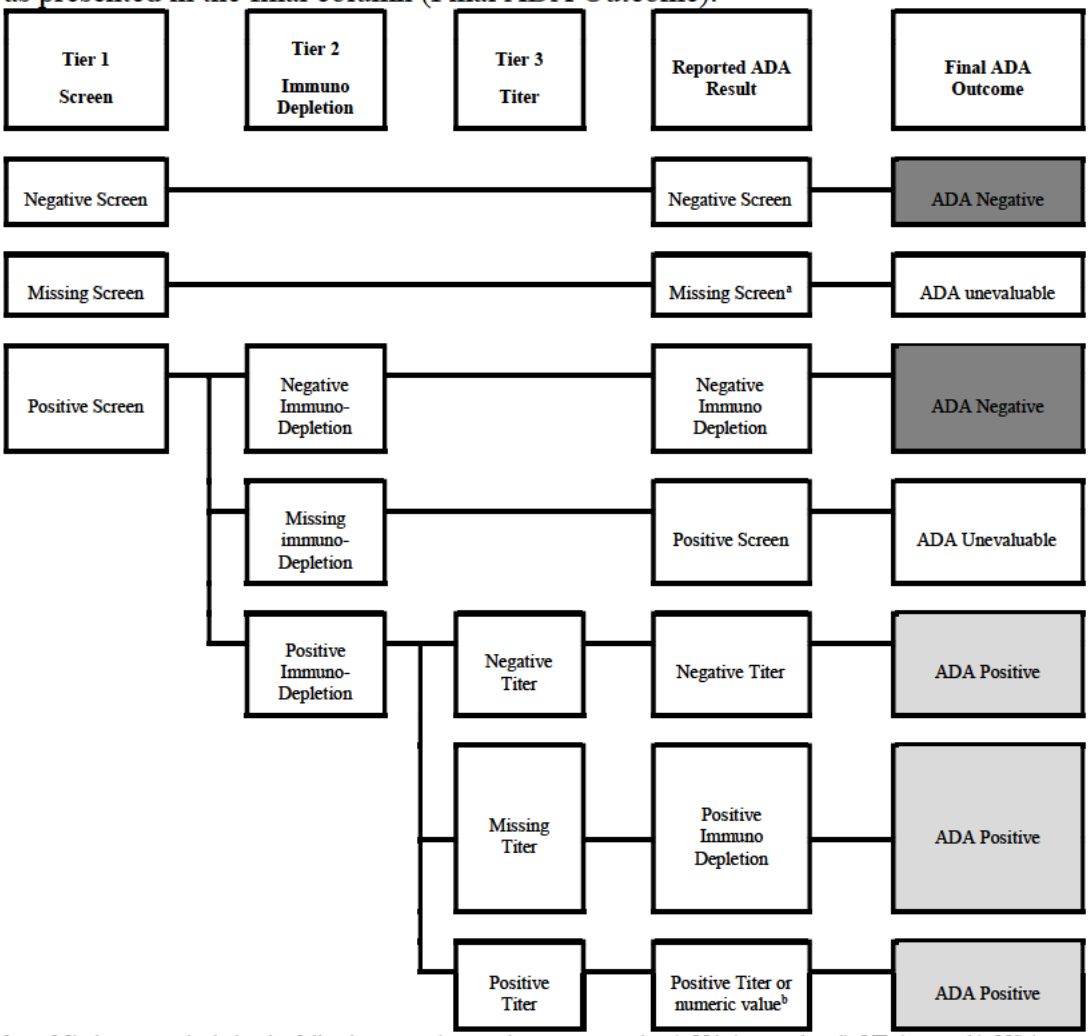
ADA samples are analysed in a 3-tiered approach:

- All samples are evaluated in the ADA screening assay and are scored ADA screening positive or negative.
- If a sample scored ADA screening positive, it is further evaluated in the confirmatory assay and is scored confirmed positive (positive immunodepletion) or confirmed negative (negative immunodepletion).
- If a sample is scored as confirmed positive, the samples are further characterized in the ADA titration assay (to determine titer) and are also further analysed in the Nab assay to confirm neutralizing activity (positive or negative).

If available, a titer result will be reported for the ADA confirmed positive samples. However, a titer result is not always available:

- In case the ADA confirmed positive sample could not be run in the titration assay (e.g. due to insufficient sample volume/quality to perform the titer analysis) the result will be described as 'positive immuno-depletion' and the sample should be considered as ADA positive.
- If a sample is negative in the titration assay, it will be reported as 'negative titer' but the sample should be considered as 'ADA positive' since it was confirmed positive in the second tier.
- If a sample is not reported or reported as 'positive screen', the ADA sample status is ADA unevaluable.

An overview of this 3-tiered approach and all possible ADA sample results that will be reported by the laboratory is given below. From these reported ADA sample results a final ADA sample status needs to be derived during the statistical analysis, as presented in the final column (Final ADA Outcome):



[a] Missing screen includes the following terms (reported as reason not done): NA (not analysed), NR (no result), NS (no sample) and SL (sample lost). More details can be found in the IS data transfer agreement from SGS France to SGS SD office.

[b] 'positive titer' is reported in case it was not possible to retrieve a numeric value.

4.3.2 Derivation rules

4.3.2.1 Subject Classification for ADA – Overall and by Cycle

Table below gives an overview of how the ADA subject classification will be derived, starting from the patient baseline ADA sample status.

Subject ADA classification	**Highest[c] post-baseline[d] sample status**				
	ADA negative	**ADA positive (missing titer[a])**	**ADA positive (negative titer[b] or numeric titer value)**		**ADA not evaluable**
Baseline ADA sample status					
ADA negative	ADA negative	Treatment Induced ADA	Treatment Induced ADA		*ADA unevaluable*
ADA positive (missing titer[a])	Treatment Unaffected ADA	*ADA unevaluable*	*ADA unevaluable*		*ADA unevaluable*
ADA positive (negative titer[b] or numeric titer value)	Treatment Unaffected ADA	*ADA unevaluable*	**titer < 4 x baseline titer:** Treatment Unaffected ADA	**titer ≥ 4x baseline titer:** Treatment Boosted ADA[e]	*ADA unevaluable*
ADA not evaluable	*ADA unevaluable*	*ADA unevaluable*	*ADA unevaluable*		*ADA unevaluable*

[c] Samples with missing titer have as reported ADA result 'positive immunodepletion' or 'positive titer';
[d] Results reported as 'negative titer', i.e. titer value <1 will be set to value of 1;
[e] Highest sample status, with order: (from low to high): ADA unevaluable, ADA negative, ADA positive (missing titer /positive immunodepletion), ADA positive with titer < 1 ('negative titer' as reported ADA result, titer value set to 1), ADA positive with titer ≥ 1 (i.e. positive titer and selecting the sample with highest titer)
[f] For the overall ADA subject classification, the highest post baseline sample status over all cycles is used for comparison with the baseline ADA sample status.
For the cycle ADA subject classification, the highest post baseline sample status within the cycle is used for comparison with the baseline ADA sample status.
[g] Note: Four-fold difference in titer values is considered significant in case a twofold serial dilution is applied (=two times the dilution factor) (reference to Shankar et al., 2014).

ADA evaluable patient = patient classified as any of following categories: ADA negative, treatment unaffected ADA, treatment induced ADA, treatment boosted ADA.

ADA unevaluable patient = patient classified as unevaluable or with missing baseline ADA sample or without post-baseline ADA samples.

In case no ADA data is available at all, the participant cannot be classified.

ADA incidence = percentage of patients with treatment-induced or treatment-boosted ADAs (denominator: number of evaluable patients). Treatment-unaffected ADAs are not taken into account.

ADA prevalence = percentage of patients with treatment-unaffected ADA, treatment-induced ADA or treatment-boosted ADA (denominator: number of evaluable patients).

4.3.2.2 *Subject Classification for NAb – Overall and by Cycle*

ADA confirmed positive samples were further evaluated in the NAb assay. All ADA negative samples were thus not analysed in the NAb assay but should be considered per default as NAb negative in the analysis.

If a NAb sample is not reported, the NAb sample status will be NAb unevaluable.

All samples evaluated in the NAb assay will be scored as NAb positive, NAb negative or NAb unevaluable by the laboratory. Based on these results, the patients will be categorized based on their baseline and post-baseline sample status as detailed in following table.

Subject NAb classification	**Highest[a] post baseline[b] NAb sample status**		
	NAb negative	**NAb positive**	**NAb not evaluable**
Baseline NAb sample status			
NAb negative	baseline neg – post-baseline neg	baseline neg – post-baseline pos	*NAb unevaluable*
NAb positive	baseline pos - post-baseline neg	baseline pos – post-baseline pos	*NAb unevaluable*
NAb not evaluable	*NAb unevaluable*	*NAb unevaluable*	*NAb unevaluable*

[a] Highest sample status in order: (from low to high): NAb unevaluable, NAb negative, NAb positive.
[b] For the overall NAb subject classification, the highest post baseline sample status over all cycles is used for comparison with the baseline NAb sample status.
For the cycle NAb subject classification, the highest post baseline sample status within the cycle is used for comparison with the baseline NAb sample status.

NAb unevaluable patient = patient classified as NAb unevaluable or with missing baseline NAb sample or without post-baseline NAb samples

NAb incidence=percentage of patients with subject classification 'baseline neg – post-baseline pos' and 'baseline pos – post-baseline pos' (denominator: number of evaluable patients).

NAb prevalence=percentage of patients with subject classification 'baseline neg – post-baseline pos', 'baseline pos – post-baseline pos' or 'baseline pos – post-baseline neg'. (denominator: number of evaluable patients).

4.3.3 *Statistical analysis*

Analysis will be done on all patients.

Frequency tabulations (number and percentages) will be provided with ADA negative/positive/unevaluable samples per analysis visit.

Frequency tabulations (number and percentages) will be provided by cycle and overall on:

- patients per ADA subject category
- prevalence and incidence of ADA
- ADA unevaluable patients
- ADA baseline positive/negative samples

For details on the definitions, see the above section 4.3.2.1.

The ADA subject category of the applicable cycle will be used for all tables.

ADA titer values will be summarized by means of descriptive statistics by overall ADA subject category.

The above frequency tabulations will be repeated for NAb assay using the definitions as defined in section 4.3.2.2.

In addition, a frequency tabulation (number and percentages) will be provided of NAb positive patients within the overall ADA subject category (treatment-unaffected ADA, treatment-induced ADA, treatment-boosted ADA, ADA negative and ADA unevaluable).

5. SAFETY ANALYSES

5.1 ADVERSE EVENTS

5.1.1 Available data

Adverse events (AEs) are coded into system organ classes and preferred terms using the MedDRA version 24.1 (SEP2021). AEs were graded using the National Cancer Institute (NCI) Common Terminology Criteria for Adverse Events (CTCAE) version 5.0. For each AE, start and stop date/times are collected as well as severity, a seriousness flag, treatment relatedness, relatedness to procedures, action taken towards the study drug and outcome.

5.1.2 Derivation rules

Based on their start date/time, AEs will be allocated to the analysis period during which they started. Each AE will therefore be reported in only one period. Periods are defined in section 2.2.1. In case the AE start date/time is incomplete or missing and the AE could consequently be allocated to more than one period, AE will be allocated to the first possible cycle unless the available parts of the AE start or stop date/time provide evidence the assessments did not occur during that period.

Event rates per patient years of follow-up (PYFU) will be defined as the number of events divided by the sum of follow-up time (i.e. duration of treatment phase) of all participants per treatment expressed in years.

A death case is defined as an AE with outcome ‘fatal’.

Adverse events of special interest will be defined using MedDRA SOC ‘Infections and infestations’.

Infusion-related reactions (IRR) will be defined as all AEs with a MedDRA preferred terms that are listed in either:

- MedDRA Hypersensitivity SMQ broad selection
- MedDRA Anaphylactic reaction SMQ broad selection
- MedDRA extravasation events (injections, infusions and implants) SMQ broad selection, excluding implants

AND occurring within 48 hours of an infusion, or within 2 days in case no AE start time is available.

An AE for which the study drug was discontinued is defined as an AE with action taken ‘drug withdrawn’.

Treatment-relatedness will be dichotomised as follows in tables:

- Treatment-related: related, probably related, possibly related or missing
- Not treatment-related: not related, unlikely related, not applicable

AE onset and duration will be calculated as follows when start and stop dates are fully known

- AE onset day (vs. first administration)
  - AE start date ≥ date of first administration: AE start date – date of first administration + 1 day
  - AE start date < date of first administration: AE start date – date of first administration
- AE onset day (vs. start of period) = AE start date – analysis period start date + 1 day
- AE duration (days) =
  - AE end date – AE start date + 1 day
  - study discontinuation date – AE start date + 1 day (when the AE start date is fully known but the AE is not resolved at the end of the study)

    In this case the duration will be presented as ">x days".

5.1.3 Presentation of results

Adverse events occurring in ITP0 will only be listed. AEs will be tabulated by AChR-Ab status and overall and within AChR-Ab status, by cycle and overall.

An overview table will show the number and percentage of patients with at least one event, the number of events and event rates per patient years of follow-up for the following:

- TEAEs
- Serious TEAEs
- Grade ≥ 3 TEAEs
- TEAEs of special interest
- IRR events
- Fatal TEAEs
- Treatment-related TEAEs according to the Principle Investigator
- Procedures-related TEAEs according to the Principle Investigator
- Serious treatment-related TEAEs
- TEAEs for which the study drug was discontinued

Summary tables by MedDRA system organ class and preferred term will show the number and percentage of patients with at least one event. The table of TEAEs will additionally show the number of events.

Separate tables will be prepared for the following TEAEs:

- Serious TEAEs
- Non-serious TEAEs
- Grade ≥ 3 TEAEs
- IRR events
- Treatment-related TEAEs
- Procedures-related TEAEs
- Serious treatment-related TEAEs
- Serious IRR events
- TEAEs for which the study drug was discontinued

All AEs, including pre-treatment events will be listed.

COVID related events (events with preferred term COVID-19, COVID-19 pneumonia, suspected COVID-19 and coronavirus infection) will be listed. This listing will include all collected AE information and additionally the cycle, onset since first efgartigimod dose, onset since last efgartigimod dose before AE onset, last total IgG before AE onset, the last percent change in total IgG before AE onset and the time when that reported total IgG was taken compared to AE onset.

5.2 CLINICAL LABORATORY EVALUATION

5.2.1 Available data

Per protocol, for part A, the following laboratory parameters are expected:

- Biochemistry: Creatinine, creatinine clearance (BSA unadjusted), blood urea nitrogen (BUN), glucose, glycosylated haemoglobin (HbA1c), alanine aminotransferase (ALT), aspartate aminotransferase (AST), total bilirubin, gamma-glutamyl transferase (GGT), C-reactive protein (CRP), alkaline phosphatase (ALP), lactate dehydrogenase (LDH), uric acid, albumin, potassium, sodium, total calcium, lipid panel (total cholesterol, high density lipoprotein (HDL), low density lipoprotein (LDL), and triglycerides), international normalized ratio (INR) and activated partial thromboplastin time (aPTT).

  Note: for lipids and glucose, only fasted samples (fasting status Y) will be considered
- Haematology: Haemoglobin, platelet count, white blood cell (WBC) count with WBC differential
- Urinalysis: Colour, clarity/appearance, specific gravity, pH, protein, glucose, ketones, blood, bilirubin, urobilinogen, nitrite, leukocyte esterase, and microscopic examination including red blood cell (RBC) count, WBC, cast crystals, bacteria.

Normal ranges are available as provided by the laboratory.

For part B, laboratory samples are analysed locally. Results are not entered in the clinical database, but in case of a clinically significant abnormal result, it is reported as an adverse event.

5.2.2 Derivation rules

The following parameters will be derived:

- Estimated glomerular filtration rate (eGFR) (Chronic Kidney Disease Epidemiology Collaboration (CKD-EPI)) (mL/min/1.73m²) = 141 * minimum(creatinine (µmol/L)/ (88.4*K); 1)$^{\alpha}$ * maximum(creatinine (µmol/L)/(88.4*K); 1) - 1.209 * 0.993 $^{\text{age (years)}}$ * [1.018 if female] * [1.159 if race = black]

  where K = 0.7 if female and K = 0.9 if male;

  α = -0.329 if female and α = -0.411 if male
- Lipid ratios will be calculated based on fasted samples only (missing fasting status is considered as non-fasted) and rounded as detailed in section 2.3.3:
  - total cholesterol/HDL
  - LDL/HDL
  - HDL/LDL
- The following abnormality categories will be defined:
  - Low: value < lower limit of normal range
  - Normal: lower limit of normal range ≤ value ≤ upper limit of normal range
  - High: value > upper limit of normal range

  Note:
  - Classification will be done in standardised units, using non imputed values and limits.
  - For the worst-case analysis visits, as defined in section 2.2.5, an additional category low + high is defined in case there are both low and high post-baseline values.

Toxicity grades will be computed according to the common toxicity criteria for adverse events (CTCAE) toxicity grading list. The implementation of these toxicity grades for analysis is presented in appendix 9. Only the parameters described in appendix 9 will be computed, according to the declared limits for each grade.

5.2.3 Presentation of results

The statistical analysis will present results in standardised units, except for corrected GFR, which will be reported in mL/min/1.73m2.

Laboratory data will be tabulated by AChR-Ab status and overall.

Continuous laboratory parameters will be summarised by means of descriptive statistics at each analysis visit. Actual values and changes from baseline will be shown in the same table. Categorical urinalysis results will be listed only.

Laboratory abnormalities will be presented as cross-tabulations of the abnormality at each post-baseline analysis visit and at the worst-case analysis visit versus the baseline abnormality. Numbers of patients with treatment-emergent abnormalities (see Definition of terms) will also be shown. The denominator for the percentage is the total number of patients having data for the parameter per AChR-Ab status, treatment and analysis visit in the safety analysis set.

Laboratory toxicity grades will be presented as cross-tabulations of the toxicity at each post-baseline analysis visit and at the worst-case analysis visit versus the baseline toxicity. Numbers and cumulative numbers over decreasing toxicity grading of patients with treatment-emergent toxicities will also be shown. The denominator for the percentage is the total number of patients having data for the parameter per AChR-Ab status, treatment and analysis visit in the safety analysis set. Parameters having toxicity grades defined in both directions (hypo and hyper) will be shown by direction.

All laboratory data will be listed, but only for patients with any post-baseline abnormality.

5.3 VITAL SIGNS

5.3.1 Available data

The following vital signs parameters are collected: systolic (SBP) and diastolic blood pressure (DBP) in supine position, pulse rate, body temperature and weight (only fixed on few visits, at other visits only in case of obvious weight change).

5.3.2 Derivation rules

Abnormalities are defined in below table.

	Pulse rate (bpm)	**SBP (mmHg)**	**DBP (mmHg)**	**Temperature (°C)**
Low	<40	<90	<45	<35.8
Normal	40-100	90-150	45-90	35.8-37.5
High	>100	>150	>90	>37.5

Note: For the worst-case analysis visits, as defined in section 2.2.5, an additional category low + high is defined in case there are both low and high post-baseline values.

5.3.3 Presentation of results

Vital signs data will be tabulated by AChR-Ab status and overall.

Vital signs parameters supine SBP, DBP and pulse rate will be summarised by means of descriptive statistics at each analysis visit. Actual values and changes from baseline will be shown in the same table.

Abnormalities will be presented as cross-tabulations of the abnormality at each post-baseline analysis visit versus the baseline abnormality and as cross-tabulations of the worst-case abnormality versus the baseline abnormality. Numbers of patients with treatment-emergent abnormalities will also be shown.

All vital signs data will be listed, but only for patients with any post-baseline abnormality.

5.4 ELECTROCARDIOGRAMS

5.4.1 Available data

The following electrocardiogram (ECG) parameters will be collected: heart rate (HR), QRS interval, PR interval, QT interval, QTcF and QTcB.

5.4.2 Derivation rules

Abnormalities for HR, QRS and PR interval are defined in below table.

	HR (bpm)	PR (ms)	QRS (ms)
Low	<40	<120	-
Normal	40-100	120-220	0-120
High	>100	>220	>120

Note: For the worst-case analysis visit, as defined in section 2.2.5, an additional category low + high is defined in case there are both low and high post-baseline values.

For QTc interval (ms), the following categories are defined:

- Actual values:
  - ≤ 450 (normal)
  -]450; 480]
  -]480; 500]
  - > 500
- Changes:
  - ≤ 30 (normal)
  -]30; 60]
  - > 60

Note: The worst-case, as defined in section 2.2.5, is the highest post-baseline value and associated change.

5.4.3 Presentation of results

Uncorrected QT interval and RR will only be listed.

ECG data will be tabulated by AChR-Ab status and overall.

Continuous ECG parameters will be summarised by means of descriptive statistics at each analysis visit. Actual values and changes from baseline will be shown in the same table.

Abnormalities of the actual values will be presented as cross-tabulations of the abnormality at each post-baseline analysis visit, and at the worst-case analysis visit versus the baseline abnormality. Numbers and cumulative numbers over decreasing abnormalities (QTc only) of patients with treatment-emergent abnormalities will also be shown. The denominator for the percentage is the total number of patients having data for the parameter per AChR-Ab status, treatment and analysis visit in the safety analysis set.

Abnormalities of the QTc changes will be presented as tabulations of the change abnormality at each post-baseline analysis visit and at the worst-case analysis visit. Cumulative numbers over decreasing change abnormalities of patients will also be shown. The denominator for the percentage is the total number of patients having data for the parameter per AChR-Ab status, treatment and analysis visit in the safety analysis set.

All ECG data will be listed, but only for patients with any post-baseline abnormality.

5.5 SUICIDALITY ASSESSMENT

5.5.1 Available data

This so-called suicidality assessment will be conducted during part A by specifically asking the following question, derived from the PHQ-9: "Over the last 2 weeks, how often have you been bothered by thoughts that you would be better off dead, or of hurting yourself in some way?" Possible outcomes are: Not at all (0), Several days (1), More than half the days (2), Nearly every day (3)

5.5.2 Presentation of results

Suicidality assessment results will be presented using a frequency tabulation by analysis visit and worst over time. The denominator for the percentage is the total number of patients per treatment and analysis visit in the safety analysis set.

All suicidality assessment data will be listed, but only for patients with any post-baseline category ≥ 1.

6. CHANGES TO THE PLANNED ANALYSIS

6.1 CHANGES NOT COVERED BY PROTOCOL AMENDMENTS BEFORE DATABASE LOCK

NAP

6.2 CHANGES NOT COVERED BY PROTOCOL AMENDMENTS AFTER DATABASE LOCK

NAP

6.3 CHANGES TO THE FINAL STATISTICAL ANALYSIS PLAN

NAP

7. REFERENCES

Simon, G. E., C. M. Rutter, D. Peterson, M. Oliver, U. Whiteside, B. Operskalski and E. J. Ludman (2013). "Does response on the PHQ-9 Depression Questionnaire predict subsequent suicide attempt or suicide death?" Psychiatr Serv 64(12): 1195-1202.

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16.2.5.1	Administration of study drug	SAF
EFFICACY		
16.2.6.1	MG-ADL: Total Score and Derived Variables	SAF
16.2.6.2	QMG: Total Score and Derived Variables	SAF
PHARMACODYNAMICS		
16.2.6.3	Total IgG and IgG Subtypes	SAF
16.2.6.4	Anti-AChR Antibodies	SAF
16.2.6.5	Anti-MuSK Antibodies	
SAFETY		
ADVERSE EVENTS		
16.2.7.1	Adverse Events	SAF
16.2.7.2	Serious Adverse Events	SAF
16.2.7.3	Fatal Adverse Events	RO
16.2.7.4	Treatment Emergent Adverse Events Leading to Discontinuation of Study Drug	SAF

16.2.7.5	Treatment Emergent Adverse Events Leading to Interruption of Study Drug	SAF
16.2.7.6	Treatment Emergent Adverse Events of Special Interest	SAF
16.2.7.7	Infusion-Related Reactions	SAF
16.2.7.8	COVID-Related Events	SAF
LABORATORY DATA		
16.2.8.1	Laboratory Test Results for Subjects with Abnormal Values	SAF
VITAL SIGNS		
16.2.9.1	Vital Signs Results for Subjects with Abnormal Values	SAF
ECG		
16.2.10.1	ECG Results for Subjects with Abnormal Values	SAF
SUICIDALITY ASSESMENT		
16.2.11.1	Suicidality assessment for Subjects with Abnormal Values	SAF
IMMUNOGENECITY		
16.2.12.1	Anti-drug Antibodies and Neutralizing Antibodies	SAF

9. APPENDICES

9.1 TOXICITY GRADES

Below table documents how the Common Terminology Criteria for Adverse Events CTCAE, v5.0: November 27, 2017 is implemented in the statistical analysis.

PARAMETER	Unit	GRADE 1	GRADE 2	GRADE 3	GRADE 4
Amylase (pancreatic)		>1.0-1.5 *ULN	>1.5-2.0 *ULN	>2.0-5.0 *ULN	>5.0 *ULN
Alanine amino transferase		>1-3 *ULN	>3-5 *ULN	>5-20 *ULN	>20 *ULN
Albumin	g/L	<LLN-30	<30-20	<20	-
	g/dL	<LLN-3	<3-2	<2	-
Alkaline phosphatase		>1.0-2.5 *ULN	>2.5-5.0 *ULN	>5.0-20.0 *ULN	>20.0 *ULN
Aspartate amino transferase		>1-3 *ULN	>3-5 *ULN	>5-20 *ULN	>20 *ULN
Bilirubin (total)		>1.0-1.5 *ULN	>1.5-3.0 *ULN	>3.0-10.0 *ULN	>10.0 *ULN
Calcium (ionized) low	mmol/L	<LLN-1.0	<1.0-0.9	<0.9-0.8	<0.8
	mg/dL	<LLN-4.0	<4.0-3.6	<3.6-3.2	<3.2
Calcium (ionized) high	mmol/L	>ULN-1.5	>1.5-1.6	>1.6-1.8	>1.8
	mg/dL	>ULN-6.0	>6.0-6.4	>6.4-7.2	>7.2
Calcium (corrected) low	mmol/L	<LLN-2.00	<2.00-1.75	<1.75-1.50	<1.50
	mg/dL	<LLN-8	<8-7	<7-6	<6
Calcium (corrected) high	mmol/L	>ULN-2.9	>2.9-3.1	>3.1-3.4	>3.4
	mg/dL	>ULN-11.5	>11.5-12.5	>12.5-13.5	>13.5
Cholesterol	mmol/L	>ULN-7.75	>7.75-10.34	>10.34-12.92	>12.92

	mg/dL	>ULN-300	>300-400	>400-500	>500
Creatine kinase		>1.0-2.5 *ULN	>2.5-5.0 *ULN	>5.0-10.0 *ULN	>10.0 *ULN
Creatinine		>1.0-1.5 *ULN	>1.5-3.0 *ULN	>3.0-6.0 *ULN	>6.0 *ULN
Gamma-glutamyl transferase		>1.0-2.5 *ULN	>2.5-5.0 *ULN	>5.0-20.0 *ULN	>20.0 *ULN
Glucose (fasting) low [1]	mmol/L	<LLN-3.0	<3.0-2.2	<2.2-1.7	<1.7
	mg/dL	<LLN-55	<55-40	<40-30	<30
Lipase		>1.0-1.5 *ULN	>1.5-2.0 *ULN	>2.0-5.0 *ULN	>5.0 *ULN
Magnesium low	mmol/L	<LLN-0.5	<0.5-0.4	<0.4-0.3	<0.3
	mg/dL	<LLN-1.2	<1.2-0.9	<0.9-0.7	<0.7
Magnesium high	mmol/L	>ULN-1.23	-	>1.23-3.30	>3.30
	mg/dL	>ULN-3.0	-	>3.0-8.0	>8.0
Potassium low	mmol/L	-	<LLN-3.0	<3.0-2.5	<2.5
	mEq/L	-	<LLN-3.0	<3.0-2.5	<2.5
Potassium high	mmol/L	>ULN-5.5	>5.5-6.0	>6.0-7.0	>7.0
	mEq/L	>ULN-5.5	>5.5-6.0	>6.0-7.0	>7.0
Sodium low	mmol/L	<LLN-130	-	<130-120	<120
	mEq/L	<LLN-130	-	<130-120	<120
Sodium high	mmol/L	>ULN-150	>150-155	>155-160	>160
	mEq/L	>ULN-150	>150-155	>155-160	>160
Triglycerides	mmol/L	1.71-3.42	>3.42-5.70	>5.70-11.4	>11.4
	mg/dL	150-300	>300-500	>500-1000	>1000

Partial thromboplastin time (activated or not specified		>1.0-1.5 *ULN	>1.5-2.5 *ULN	>2.5 *ULN	-
CD4 count	giga/L	<LLN-0.50	<0.50-0.20	<0.20-0.05	<0.05
	counts/mm^3	<LLN-500	<500-200	<200-50	<50
Fibrinogen		<1.00-0.75 *LLN	<0.75-0.50 *LLN	<0.50-0.25 *LLN	<0.25 *LLN
International normalized ratio		>1.2-1.5 *ULN	>1.5-2.5 *ULN	>2.5 *ULN	-
Lymphocytes (absolute count) low	giga/L	<LLN-0.80	<0.80-0.50	<0.50-0.20	<0.20
	counts/mm^3	<LLN-800	<800-500	<500-200	<200
Lymphocytes (absolute count) high	giga/L	-	>4-20	>20	-
	counts/mm^3	-	>4000-20000	>20000	-
Neutrophils (absolute count)	giga/L	<LLN-1.5	<1.5-1.0	<1.0-0.5	<0.5
	counts/mm^3	<LLN-1500	<1500-1000	<1000-500	<500
Platelets	giga/L	<LLN-75	<75-50	<50-25	<25
	counts/mm^3	<LLN-75000	<75000-50000	<50000-25000	<25000
White blood cells	giga/L	<LLN-3	<3-2	<2-1	<1
	counts/mm^3	<LLN-3000	<3000-2000	<2000-1000	<1000

Note: In case ULN/LLN is higher/lower than the upper/lower limit of grade 1 (or even higher grades), ULN/LLN will be ignored and only the fixed values of CTCAE will be considered.

9.2 GENERAL SCHEDULE OF ASSESSMENTS

9.2.1 Part A

Assessment	Roll-Over Visit[a]	Treatment Period				Inter Treatment Period[a,b,c]	End of Part A[c] / Early Discontinuation[s]	Unscheduled[d]
Visits Treatment Period 1	**SEB**	**TP_1V1[a]**	**TP_1V2**	**TP_1V3**	**TP_1V4**	**ITP_nVn**	**EoA/ED**	**UNS**
Trial Day (Visit Window)		**1**	**8±1**	**15±1**	**22±1**			
Visits Subsequent Treatment Periods		**TP_nV1 (TP_nB)**	**TP_nV2**	**TP_nV3**	**TP_nV4**			
Trial Day (Visit Windows)		**X**	**(X+7)±1**	**(X+14)±1**	**(X+21)±1**	**Y + 30 (±2)**	**365±7**	
Informed consent[e]	X							
In- and exclusion criteria	X							
Medical/surgical history	X							
Demographic characteristics	X							
MG-ADL[f]	X	X	X	X	X	X	X	X
QMG[f]	X	X	X	X	X	X	X	X
Suicidality assessment[g]	X	X	X	X	X	X	X	X
Physical examination[h]	X	X	X	X	X	X	X	X
Weight and Height[i]	X						X	
Vital signs[j]	X	X	X	X	X	X	X	X
ECG[k]	X	X			X	X	X	X
Clinical laboratory tests[l]	X	X	X	X	X	X	X	X
Urinalysis[m]	X	X	X	X	X	X	X	X
Urine pregnancy test[n]	X	X	X	X	X	X	X	X
Anti-AChR/anti-MuSK antibodies[o]	X	X	X	X	X	X	X	X

Assessment	Roll-Over Visit[a]	Treatment Period				Inter Treatment Period[a,b,c]	End of Part A[c] / Early Discontinuation[s]	Unscheduled[d]
Visits Treatment Period 1	SEB	TP_1V1[a]	TP_1V2	TP_1V3	TP_1V4	ITP_nVn	EoA/ED	UNS
Trial Day (Visit Window)		1	8±1	15±1	22±1			
Visits Subsequent Treatment Periods		TP_nV1 (TP_nB)	TP_nV2	TP_nV3	TP_nV4			
Trial Day (Visit Windows)		X	(X+7)±1	(X+14)±1	(X+21)±1	Y + 30 (±2)	365±7	
Total IgG and its subtypes[o]	X	X	X	X	X	X	X	X
ADA[p]	X	X			X		X	
ARGX-113 administration[q]		X	X	X	X			
Prior/concomitant/rescue therapy[r]	<----------X---------->							
Adverse events	<----------X---------->							

AChR: acetylcholine receptor; ADA: anti-drug antibodies; ECG: electrocardiogram; EoA/ED: End of Part A/Early Discontinuation; QMG: Quantitative Myasthenia Gravis score; IgG: immunoglobulin G; ITPV: Inter Treatment Period Visit; MG-ADL: Myasthenia Gravis-Activities of Daily Living; MuSK: Muscle-Specific Kinase; SEB: Study Entry Baseline; TPB: Treatment Period Baseline; TPV: Treatment Period Visit; Y: previous visit; UNS: unscheduled visit

[a] The roll-over visit can be either ITP_0V1 or TP_1V1, depending on the patient status regarding the need for (re-)treatment upon roll-over from trial ARGX-113-1704. The first visit in trial ARGX-113-1705 will always coincide with the EoS visit in trial ARGX-113-1704. The assessments done for the last visit in trial ARGX-113-1704 should not be repeated.

[b] The Inter Treatment Period visits (ITP_nVn) occur every 30 days after the previous visit (Y). The visit denominator ('n') will start at 1 at each period. At each ITP_nVn, an evaluation of the need for (re-)treatment should be done prior to decide whether assessments listed for ITP_nVn or TP_nV1 are to be performed. If an ITP visit for Part A is scheduled within 14 days of the EoA visit, then this EoA visit should be performed instead. For patients that discontinue at the ITP visit this will then serve as the EoA/ED visit.

[c] If a patient becomes eligible for (re-)treatment with ARGX-113 per total MG-ADL score but cannot complete the entire Treatment Period within Part A of the trial (re-treatment in Part A can start at the latest on Day 336), the patient can be transitioned to Part B after completing the EoA visit. If the patient is not willing or able to continue to Part B then he/she should continue to the EoA visit. The transition to Part B can be either ITPE0Vn or TPE1V1, depending on the patient status regarding the need for (re-)treatment upon transition from Part A. The first visit in Part B will always coincide with the EoA visit in Part A. The assessments done for the last visit in Part A should not be repeated in Part B.

[d] An unscheduled (UNS) visit can occur at the request of the patient or the Investigator. During the UNS visit, additional assessments can be performed at the discretion of the Investigator, depending on the reason for the UNS visit.

[e] No trial-related assessment is to be carried out before the patient has signed the informed consent form (ICF).

[f] Assessments of disease severity should be completed pre-dose on dosing days and should be performed prior to any other trial specific assessment, except for obtaining informed consent at SEB and the weight assessment, if applicable. The MG-ADL scale needs to be performed prior to the QMG scale. Acetylcholinesterase inhibitors must be halted for at least 12 hours before the QMG assessment (consistent with the revised manual for the QMG test as recommended by the Myasthenia Gravis Foundation of America [MGFA]).

[g] Suicidal ideation and behavior will be assessed pre-dose on dosing days via a targeted question based on the Patient Health Questionnaire item 9 (PHQ-9) (Simon, Rutter et al. 2013).

[h] The physical examination will be performed pre-dose on dosing days. See Section 7.2.3 in the Protocol for an overview of the different assessments.

[i] Weight will be measured at SEB, at the EoA/ED visit, and when there is an obvious weight change compared to the last weight assessment. Height will be measured at SEB.

[j] Vital signs (supine blood pressure, heart rate, body temperature) will be performed pre-dose on dosing days. It is recommended that the method used to measure body temperature at SEB is maintained throughout the trial for each patient.

k ECG will be performed pre-dose on dosing days.
l Samples for clinical laboratory tests (hematology and clinical chemistry) will be collected pre-dose on dosing days (see Appendix 3 in the Protocol). Patients need to be fasted at least 8 hours prior to each sampling.
m Urine samples will be collected pre-dose on dosing days (see Appendix 3 in the Protocol).
n A urine pregnancy test will be performed pre-dose on dosing days on the urine samples taken for urinalysis (only for women of childbearing potential, see Definition of Terms).
o Samples for pharmacodynamic (PD) biomarkers will be collected pre-dose on dosing days (see Appendix 3 in the Protocol). Anti-AChR antibodies will be measured in AChR-Ab seropositive patients only. Anti-MuSK antibodies will be measured in MuSK-Ab seropositive patients only. Information on the AChR/MuSK-Ab serotype (seronegative or seropositive) is available from trial ARGX-113-1704.
p Samples for anti-drug antibodies (ADAs) will be collected pre-dose on dosing days.
q ARGX-113 will be administered as an intravenous (IV) infusion over a period of 1 hour at Visits 1, 2, 3 and 4 of each Treatment Period. Patients will remain at the site for at least 1 hour following the end of the infusion for safety monitoring based on the patient's clinical status. At TP_nV1, the conditions for (re-)treatment will be checked before administration of ARGX-113.
r Clinically relevant prior treatment will only be recorded at SEB.
s For patients who discontinue early from trial, the assessments will depend on the visit at which it was decided that the patient had to discontinue trial treatment (see Section 5.4 in the Protocol). Patients who discontinue early from trial treatment within a Treatment Period should perform the planned assessments of the corresponding Treatment Period visit.. These patients will not receive any further administration of ARGX-113 during the trial and will return for the ED visit 1 month (30 ± 2 days) after the last dose administration. An unscheduled visit can be organized if deemed necessary by the Investigator. Patients who discontinue early from the trial between the last visit of a Treatment Period (TPnV4) and the next Inter Treatment Period visit (ITPnVn) should perform the ED assessments 1 month (30 ± 2 days) after the last dose administration. Patients who discontinue early from the trial at the ITP visit should perform the ED assessments, instead of the ITPnVn assessments. Patients who discontinue early from the trial after the ITP visit should perform the ED assessments.

9.2.2 Part B

Assessment	Treatment Period				Inter Treatment Period[a,b]	End of Study[c]/Early Discontinuation[d,e]	Unscheduled[f]
Visits Subsequent Treatment Periods	TPEnV1[g] (TPEnB)	TPEnV2	TPEnV3	TPEnV4	ITPEnVn[g]	EoS/ED	UNS
Trial Day (Visit Windows)	X	(X+7)±1	(X+14)±1	(X+21)±1			
					Y+90 (±7)	1095±7	
*MG-ADL[h]	X	X	X	X	X	X	X
Physical examination						X	X
Weight[i]	X					X	X
Vital signs[j]	X	X	X	X	X	X	X
ECG[k]	X			X	X	X	X
Clinical laboratory tests[l]	X			X		X	X
Urinalysis[m]	X			X		X	X
Urine pregnancy test[n]	X			X	X	X	X
ADA[o]	X					X	X
ARGX-113 administration[p]	X	X	X	X			
**Prior/concomitant therapy*[q]	<----------X---------->						
**Adverse events*	<----------X---------->						

ADA=anti-drug antibodies; EoS/ED=End of Study/Early Discontinuation; ITPEV=Inter Treatment Period Visit in Part B; MG-ADL=Myasthenia Gravis-Activities of Daily Living; TPEV=Treatment Period Visit in Part B; Y=previous visit; UNS=unscheduled visit

[a] The Inter Treatment Period visits (ITPEnVn) occur every 90 days ±7 days after the previous visit (Y). The visit denominator ('n') will start at 1 at each period. At each ITPEnVn, an evaluation of the need for (re-) treatment should be done prior to decide whether assessments listed for ITPEnVn or TPEnV1 are to be performed.

[b] If a patient becomes eligible for (re-)treatment with efgartigimod but cannot complete the entire treatment period within the duration of the trial, the patient should continue to the EoS visit.

[c] EoS will be up to maximum 3 years (1095±7 days) or until efgartigimod becomes commercially available or another option to access efgartigimod is available, whichever option comes first.

[d] For patients who discontinue early from trial, the assessments will depend on the visit at which it was decided that the patient had to discontinue (see Section 5.4 of the protocol). Patients who discontinue early from the trial within a Treatment Period should perform the planned assessments of the corresponding Treatment Period visit. These patients will not receive any further administration of ARGX-113 during the trial and will return for the ED visit one month (30 ± 2 days) after the last dose administration. An unscheduled visit can be organized if deemed necessary by the Investigator. Patients who discontinue early from the trial between

the last visit of a Treatment Period (TPEnV4) and the next Inter Treatment Period visit (ITPEnVn) should perform the ED assessments one month (30 ± 2 days) after the last dose administration. Patients who discontinue early from the trial at the ITPE visit should perform the ED assessments, instead of the ITPEnVn assessments. Patients who discontinue early from the trial after the ITPE visit should perform the ED assessments.

[e] Before enrolling in ARGX-113-2002 from Part B, patients must first complete the early discontinuation (ED) visit assessments. The previous efgartigimod IV dose must have been received at least 30 days before entry into ARGX-113-2002.

[f] An unscheduled (UNS) visit can occur at the request of the patient or the Investigator. During the UNS visit, additional assessments as indicated in the SoA can be performed at the discretion of the Investigator, depending on the reason for the UNS visit.

[g] The transition to Part B can be either ITPE0Vn or TPE1V1, depending on the patient status regarding the need for (re-)treatment upon transition from Part A. The first visit in Part B will always coincide with the EoA visit in Part A. The assessments done for the last visit in Part A should not be repeated in Part B.

[h] Assessments of disease severity should be completed pre-dose on dosing days and should be performed prior to any other trial specific assessment, except for the weight assessment, if applicable.

[i] Weight will be measured at Visit 1, at the EoS/ED visit and when there is an obvious weight change compared to the last weight assessment.

[j] Vital signs (supine blood pressure, heart rate, body temperature) will be performed pre-dose on dosing days. It is recommended that the method used to measure body temperature is maintained throughout the trial for each patient.

[k] A 12-lead ECG will be recorded locally. The assessments on heart rate, PR, QT, and QRS intervals will be read centrally.

[l] Samples for clinical laboratory tests (hematology, clinical chemistry) will be collected pre-dose on dosing days, if applicable (see appendix 3 of the protocol).

[m] Urine samples will be collected pre-dose on dosing days (see appendix 3 of the protocol).

[n] If a urine pregnancy test is carried out on a dosing day then it should be performed pre-dose (only for women of childbearing potential, see definition of terms of the protocol).

[o] Samples for anti-drug antibodies (ADAs) will be collected pre-dose on dosing days (if applicable) and will only be collected in the case of early discontinuation and not at the EoS visit.

[p] Efgartigimod will be administered as an intravenous (IV) infusion over a period of 1 hour at Visits 1, 2, 3, and 4 of each Treatment Period. Patients will remain at the site for at least 1 hour following the end of the infusion for safety monitoring based on the patient's clinical status. At TPEnV1, the conditions for (re-)treatment will be checked before administration of efgartigimod.

[q] All available vaccination history will be recorded as part of the participant's prior medication for vaccinations received in the past or concomitant medication for vaccinations received during the trial. For vaccines where multiple doses or boosters are received, only the most recent one must be recorded.

* Assessments highlighted by use of bold italics indicate assessments that may be carried out as a telephone assessment visit under the conditions outlined in appendix 8 of the protocol.