A Long-term, Single-Arm, Open-label, Multicenter, Follow-on Trial of ARGX-113-2006 to Evaluate Safety of Efgartigimod Administered Intravenously in Children With Generalized Myasthenia Gravis

Published: 28-06-2022
Last updated: 14-09-2024

This study has been transitioned to CTIS with ID 2023-507379-23-00 check the CTIS register for the current data. The aim of this trial is to investigate the long-term safety, tolerability, and immunogenicity of efgartigimod administered...

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Autoimmune disorders
Study type	Interventional

Summary

ID

NL-OMON53506

Source
ToetsingOnline

Brief title
ARGX-113-2008 / ADAPT JR+

Condition

- Autoimmune disorders

Synonym
Generalized Myasthenia Gravis, gMG

Research involving

Human

Sponsors and support

Primary sponsor: Argenx BVBA
Source(s) of monetary or material Support: argenx BV

Intervention

Keyword: Efgartigimod, Generalized Myasthenia Gravis, gMG, Myasthenia Gravis

Outcome measures

Primary outcome

Safety and tolerability of efgartigimod (IV)

Secondary outcome

Immunogenicity of efgartigimod (IV).

Study description

Background summary

gMG is a rare, chronic, neuromuscular autoimmune disease caused by pathogenic IgGs targeting the neuromuscular junction, producing reduced neuromuscular transmission and debilitating and potentially life-threatening muscle weakness and chronic fatigue. Generalized muscle weakness results in difficulties in mobility, speech, swallowing, vision, and respiration.

Efgartigimod is a human immunoglobulin (Ig) G1 (IgG1)-derived Fc fragment that binds with nanomolar affinity to human neonatal Fc receptor (FcRn) that is being developed for the treatment of generalized myasthenia gravis. Overall, efgartigimod IV has been well tolerated in healthy adult participants and in participants with gMG. No major safety findings have arisen in ongoing and completed studies with efgartigimod. Further nformation can be found in the IB.

This trial is a follow-on of the ARGX-113-2006 trial (NL78028.058.21), the first clinical trial administering efgartigimod intravenously in a pediatric population.
Only participants of ARGX-113-2006 who have completed the trial will be enrolled in the ARGX-113-2008 trial.

Study objective

This study has been transitioned to CTIS with ID 2023-507379-23-00 check the CTIS register for the current data.

The aim of this trial is to investigate the long-term safety, tolerability, and immunogenicity of efgartigimod administered intravenously (IV) in pediatric participants rolling over from the ARGX-113-2006 trial and to ensure access to the drug before commercial availability or until another option to access efgartigimod is available.

Study design

ARGX-113-2008 is an open-label, multicenter, uncontrolled clinical trial in prediatric participants with gMG who have completed the ARGX-113-2006 trial.
The trial consists of Treatment periods and Treatment-Free periods. A treatment period lasts 4 weeks with a weekly visit and infusion with study medication.
The amount of Treatment periods per patient depends on the course of the disease in that patient.
The Treatment Free period will last for at least 28 days and after that the duration is depending on when a new treatment period is considered needed.
Patients are seen 1 week after the last dose, and as long as no retreatment is required, patients are followed up by a phone call every 3 monts and seen at the site every 6 months.
Participants will start with either a Treatment Period or Treatment Free period based on their personal status as evaluated by the investigator and the number of days since the last day of the last treatment.

The trial end when commercial access to the drug or until another option to access efgartigimod is available.

Intervention

Intravenous infusion with efgartigimod. This consists of 4 weekly infusions per Treatment period.

Study burden and risks

In previous clinical studies with efgartigimod in adults, some people experienced side effects. Some side effects could be related to efgartigimod. Many of these side effects were mild, lasted a short time, and required little or no treatment.
The most commonly reported side effects in studies where healthy adults received efgartigimod or placebo were:
• Headache
• decreases in white blood cell counts

• increases in levels of a blood test marker for inflammation (C-reactive protein)
• fatigue
• common cold
• mouth/throat discomfort
• feeling cold
• injection site redness
• injection site bruise
• back pain

Some of the side effects occurred in healthy participants who received the placebo, and some occurred in healthy participants who received efgartigimod at higher doses than the dose that will be used in this study.

The most common reported side effects in other studies in adults with gMG and thrombocytopenia (a blood problem) who received efgartigimod were:
• headache
• common cold
• diarrhea
• upper respiratory tract infection
• nausea
• urinary tract infection
• muscle pain
• mouth/throat discomfort
Most of the side effects mentioned above were of mild to moderate intensity, resolved quickly, and assessed as not related to efgartigimod treatment. The most common side effects that were related to the study drug included inflammation of the airway passages, upper respiratory tract infection, urinary tract infection, headache, and muscle pain.

The study will be executed in line with the Code of conduct for resisting minors.

Contacts

Public
Argenx BVBA

Industriepark Zwijnaarde 7
Zwijnaarde 9052
BE

Scientific
Argenx BVBA

Industriepark Zwijnaarde 7
Zwijnaarde 9052
BE

Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)

Inclusion criteria

1. The participant completed ARGX-113-2006, defined as:
a. The participant reached EoT (End of Trial) in trial ARGX-113-2006 and agreed to participate in the ARGX-113-2008 trial.
b. The participant qualifies for retreatment in trial ARGX-113-2006, but cannot complete a Treatment Period (TP) and the required Intertreatment Period visits within the ARGX-113-2006 trial's timeframe

Exclusion criteria

1. Female Adolescents Of Child Bearing Potential: Pregnancy or lactation, or the participant intends to become pregnant during the trial or within 90 days after the last dose of investigational medicinal product (IMP)
2. Discontinued early from ARGX-113-2006 treatment due to: pregnancy, receiving prohibited medication, participating in another trial with an investigational product, or the occurrence of a life-threatening or an investigational medicinal product-related AE, as assessed by the investigator
3. A known hypersensitivity reaction to efgartigimod or any of its excipients
4. Received a live-attenuated vaccine fewer than 4 weeks before trial entry.

5. Any of the following medical conditions:
Clinically significant uncontrolled chronic bacterial, viral, or fungal infection at trial entry
b. Any other known autoimmune disease that, in the opinion of the investigator, would interfere with accurate assessment of clinical symptoms of gMG or put the participant at undue risk

Study design

Design

Study phase:	3
Study type:	Interventional
Masking:	Open (masking not used)
Control:	Uncontrolled
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	08-12-2022
Enrollment:	2
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	Efgartigimod
Generic name:	Efgartigimod

Ethics review

Approved WMO

Date:	28-06-2022
Application type:	First submission
Review commission:	METC Leiden-Den Haag-Delft (Leiden)

	metc-ldd@lumc.nl
Approved WMO Date:	14-10-2022
Application type:	First submission
Review commission:	METC Leiden-Den Haag-Delft (Leiden) metc-ldd@lumc.nl
Approved WMO Date:	26-03-2023
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden) metc-ldd@lumc.nl
Approved WMO Date:	14-04-2023
Application type:	Amendment
Review commission:	METC Leiden-Den Haag-Delft (Leiden) metc-ldd@lumc.nl

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EU-CTR	CTIS2023-507379-23-00

Register	ID
EudraCT	EUCTR2021-002460-46-NL
CCMO	NL80575.058.22