A non-interventional, post-authorisation safety study of patients treated with efgartigimod alfa

First published: 13/03/2024

Last updated: 01/07/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000019

Study ID

1000000019

DARWIN EU® study

No

Study countries

- Austria
- Belgium
- Germany
- Italy

Spain

United States

Study description

This is a non-interventional, prospective, post authorization safety study. Patients with Generalised Myasthenia Gravis (gMG) who are expected to start treatment with efgartigimod at enrolment or are within their first cycle of efgartigimod at enrolment will be eligible to enroll into the efgartigimod cohort. Patients with gMG who have not been exposed to efgartigimod and for whom it is not planned to start treatment with efgartigimod at enrolment will be eligible to enroll into the non-efgartigimod cohort.
Enrolment of patients will be over at least a 5-year period. The follow up of patients will continue for 5 years from the time the last patient has been enrolled. Patients will be followed up regardless of whether they continue or discontinue efgartigimod.

Study status

Ongoing

Research institutions and networks

Institutions

United BioSource Corporation (UBC)

Switzerland

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Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Contact details

Study institution contact

Sabine Coppieters Clinicaltrials@argenx.com

Study contact

Clinicaltrials@argenx.com

Primary lead investigator

Not yet assigned

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 31/03/2024

Study start date

Actual: 14/12/2023

Date of final study report

Planned: 01/06/2034

Study protocol

ARGX-113-PASS-2208-Protocol_Redacted.pdf (451.46 KB)

Regulatory

Was the study required by a regulatory body?

Yes

Is the study required by a Risk Management Plan (RMP)?

EU RMP category 3 (required)

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Other

Safety study (incl. comparative)

If ‘other’, further details on the scope of the study

Post-Authorization Safety Study (PASS)

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

This is a non-interventional, prospective, post authorisation safety study.

Main study objective:

A non-interventional 10-year Post-Authorization Safety Study (PASS) in a post-marketing setting comparing cohorts of patients with Generalised Myasthenia Gravis (gMG) treated with efgartigimod to those treated with other Myasthenia Gravis (MG) medication, in order to estimate the incidence rates of serious infection and other safety events.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

VYVGART

Study drug International non-proprietary name (INN) or common name

EFGARTIGIMOD ALFA

Medical condition to be studied

Myasthenia gravis

Population studied

Age groups

- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)
  - Elderly (≥ 65 years)
    - Adults (65 to < 75 years)
    - Adults (75 to < 85 years)
    - Adults (85 years and over)

Estimated number of subjects

680

Study design details

Setting

Patients with gMG who are expected to start treatment with efgartigimod at enrolment or are within their first cycle of efgartigimod at enrolment will be eligible to enrol into the efgartigimod cohort. Patients with gMG who have not been exposed to efgartigimod and for whom it is not planned to start treatment with efgartigimod at enrolment will be eligible to enrol into the non-efgartigimod cohort.

Outcomes

To evaluate the overall long-term safety of efgartigimod including the occurrence of serious infections in gMG patients treated with efgartigimod compared to gMG patients not exposed to efgartigimod.

Data analysis plan

Categorical endpoints will be summarised using counts and percent; continuous endpoints will be summarised using mean, standard deviation, median, minimum and maximum values. Statistical analysis will be mainly descriptive in nature using metrics such as incidence proportion, exposure adjusted incidence rate, confidence intervals, etc. Details of the analysis plan will be specified in the Statistical Analysis Plan (SAP).

Data management

ENCePP Seal

The use of the ENCePP Seal has been discontinued since February 2025. The ENCePP Seal fields are retained in the display mode for transparency but are no longer maintained.

Data sources

Data sources (types)

Electronic healthcare records (EHR)

Use of a Common Data Model (CDM)

CDM mapping

No

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

No