An Observational, Multinational, Post-Marketing Registry of Omaveloxolone-Treated Patients With Friedreich’s Ataxia

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Study Ongoing

Administrative details

EU PAS number

EUPAS1000000196

Study ID

1000000196

DARWIN EU® study

No

Study countries

- Austria
- Czechia
- Germany
- United States

Study description

In this study, researchers will learn more about safety of BIIB141, also known as omaveloxolone or SKYCLARYS®. This is a drug available for doctors to prescribe for people with Friedreich's Ataxia (FA).

Participants for this study will be found using a group called Friedreich's Ataxia Global Clinical Consortium (FA GCC) UNIFIED Natural History Study (UNIFAI).

FA-GCC is a group of study research centers that helps provide clinical care for FA patients, also helps researchers learn more about how FA affects patients over a long time.

Main objective of this study is to collect safety information in participants with FA from UNIFAI. Some participants in this study will be prescribed BIIB141 for first time by their own doctors.

Some participants will have started taking BIIB141 after joining UNIFAI, but less than 12 months before joining this study. Main questions researchers want to answer in this study are: How many participants had serious adverse events (SAEs)? An AE is considered serious when it results in death, is life-threatening, causes lasting problems, or requires hospital care.

How many participants had AEs related to heart failure or liver damage caused by drug?

Researchers will also learn more about: Why & when participants stopped treatment, left study, or took more of drug than was prescribed.

This study will be done as follows: Participants will be screened to check if they can join study. After joining, participants who had never started BIIB141 treatment before must start it within 6 months. Otherwise, all participants will take BIIB141 throughout this study as prescribed by their own doctor. During study, each participant's doctor will decide how often participant visits study research center to check on their health.

This will be based on doctor's own clinical judgment & what is recommended by drug's label.

Data from participants will be collected at months 1,2,3,6,12,24,36,48,60.

Each participant will be in the study for up to 5 years.

Study status

Ongoing

Contact details

Study institution contact

Study Director clinicaltrials@biogen.com

Study contact

clinicaltrials@biogen.com

Primary lead investigator

David Lynch

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 18/03/2024

Study start date

Actual: 12/12/2024

Data analysis start date

Actual: 12/12/2024

Date of final study report

Planned: 15/03/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biogen-100%

Study protocol

296FA401_Protocol Amendment 6_Redacted_03 Apr 2025.pdf (8.05 MB)

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:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

Patient registry study

Main study objective:

The primary objective of this study is to assess the long-term safety of omaveloxolone as prescribed to participants with FA in the real-world setting, including characterization of all drug-induced liver injury (DILI) and congestive heart failure (CHF) AEs.
The secondary objective of this study is to capture the reasons and timing of omaveloxolone treatment interruptions, discontinuations, and drug overdose.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Prospective and retrospective observational study

Study drug and medical condition

Medicinal product name

SKYCLARYS

Medicinal product name, other

Omaveloxolone (SKYCLARYS)

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

OMAVELOXOLONE

Anatomical Therapeutic Chemical (ATC) code

(N07XX25) omaveloxolone

omaveloxolone

Medical condition to be studied

Friedreich's ataxia

Population studied

Short description of the study population

All participants for this study will be identified and enrolled via the FA-Global Clinical Consortium (FA-GCC) UNIFIED Natural History Study (UNIFAI study).

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)

Special population of interest

Other

Special population of interest, other

Friedreich’s ataxia patients

Estimated number of subjects

300

Study design details

Outcomes

Number of Participants With Treatment-Emergent Serious Adverse Events (TESAEs), Number of Participants With DILI and CHF AEs, Time to Omaveloxolone Treatment Interruption, Time to Omaveloxolone Treatment Discontinuation, Time to Omaveloxolone Drug Overdose, Number of Participants With Reasons for Omaveloxolone Treatment Interruption, Treatment Discontinuation and Overdose

Data analysis plan

Data analyses will be presented separately for participants in the omaveloxolone-naive cohort and the non-naive cohort. An overall summary of TESAEs will be presented, consisting of the number and percentage of participants experiencing the total number of TESAEs. TESAEs will also be summarized by system organ class (SOC) and Preferred Term (PT).
Incidence rates of all DILI and CHF AEs will be estimated, with two-sided 95%

CIs. Time to treatment interruption, time to discontinuation and time to omaveloxolone drug overdose will be analyzed using the Kaplan-Meier method and competing risk-based survival analysis if needed.
Reasons for treatment interruption, treatment discontinuation and drug overdose will be summarized descriptively.

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 source(s), other

FA-Global Clinical Consortium (FA-GCC) UNIFIED Natural History Study (UNIFAI study)

Data sources (types)

Disease registry

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