An Observational Registry-Based Study to Evaluate the Long-Term Safety of Tofersen in People With SOD1-ALS

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Study Ongoing

Administrative details

EU PAS number

EUPAS1000000365

Study ID

1000000365

DARWIN EU® study

No

Study countries

United States

Study description

In this study, researchers will learn more about the safety of tofersen, also known as Qalsody®. This is a drug available for doctors to prescribe for participant with a certain type of amyotrophic lateral sclerosis, also known as ALS. This type is in participant who have a mutation in the superoxide dismutase 1 gene, also known as SOD-1.

This is known as an “observational” study, which collects health information about study participants without changing their medical care. Participants for this study will be found using 2 different groups of study research centers that help provide clinical care for participant with ALS. These groups are in Europe and the United States and are called:
• the Precision-ALS programme
• the ALS/Motor Neuron Disease (MND) Natural History Consortium (NHC)

The main goal of this study is to collect safety information in participants with SOD-1 ALS who were in either of the groups.

The main question researchers want to answer in this study is:
• What are the characteristics of the participants in this study?
• How many participants had serious adverse events (SAEs), including ones that affect the brain, spinal cord, or nerves?

An adverse event is a health problem that may or may not be caused by a drug during the study. An adverse event is considered serious when it results in death, is life-threatening, causes lasting problems, or requires hospital care.

Researchers will also learn more about:
• How many participants develop other health conditions or become pregnant, including how the pregnancy turned out
• Why and when participants stopped treatment

This study will be done as follows:

• Participants will be screened to check if they can join the study.

• Data from the participants' regular visits to their clinic will be collected based on which study research center they are in.

• Each participant will be in the study until they decide to leave or until death. Currently, the study is planned to last at least 7 years.

Study status

Ongoing

Research institutions and networks

Institutions

Biogen

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Institution

Contact details

Study institution contact

Study Director clinicaltrials@biogen.com

Study contact

clinicaltrials@biogen.com

Primary lead investigator

Study Director

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 19/01/2026

Actual: 05/08/2025

Study start date
Planned: 31/03/2026

Actual: 02/01/2026

Data analysis start date
Planned: 30/01/2026

Date of final study report
Planned: 30/07/2034

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biogen-100%

Study protocol

233AS401_Protocol V3_Redacted.pdf (2.55 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Drug utilisation

Safety study (incl. comparative)

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

This study is an observational, registry-based study of people with SOD1-ALS using data collected by 2 existing networks of ALS disease registries.

Main study objective:
The primary objectives of this study are to describe demographic and clinical characteristics of participants with superoxide dismutase 1-amyotropic lateral sclerosis (SOD1-ALS); to describe the frequency of serious adverse events (SAEs) among participants with SOD1-ALS, including serious neurologic events previously reported in clinical trial participants (e.g., myelitis, radiculitis, aseptic meningitis, increased intracranial pressure, and/or papilloedema).

The secondary objectives of this study are to describe the frequency of new comorbid conditions, pregnancy and pregnancy outcome among participants with SOD1-ALS; and to describe the frequency of treatment discontinuation among participants with SOD1-ALS treated with tofersen.

Study Design

Non-interventional study design
Cohort

Study drug and medical condition

Medicinal product name
QALSODY

Medicinal product name, other
Tofersen

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

TOFERSEN

Anatomical Therapeutic Chemical (ATC) code

(N07XX22) tofersen

tofersen

Medical condition to be studied

Amyotrophic lateral sclerosis

Population studied

Short description of the study population

Participants with SOD1-ALS enrolled in TRICALS network’s Precision-ALS programme with data from participating clinical centers across multiple European countries or at ALS/MND NHC with data from participating clinical centers in the United States (tofersen users and tofersen non-users). NHC registry has started the data collection while TRICALS is yet to start.

Age groups

- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)

Estimated number of subjects

69

Study design details

Setting

TRICALS network's Precision-ALS programme and ALS/MN DNHC in the United States.

Outcomes

Baseline Demographic [Age, Participant Sex, Race/Ethnicity, Weight, Height, Body Mass Index (BMI), Family History of Amyotrophic Lateral Sclerosis (ALS)], Clinical Characteristics (Age at Diagnosis and Symptom Onset, Revised El Escorial Classification, Classification of SOD1-ALS Clinical Phenotypes, SOD1 Mutation Type, Medical History, Concomitant Medications, Disease History, Pregnancy Status), Number of Participants With SAEs, Number of Participants With New Comorbid Conditions, Number of Participants With Pregnancy and Pregnancy Outcomes, Number of Participants With Reported Treatment Discontinuation, Number of Participants With Reason for Treatment Discontinuation

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Data analysis plan

Data will be analysed separately for each registry network. Continuous variables will be described with summary statistics including number of observations, mean, standard deviation, median, first quartile, third quartile, and minimum and maximum values. Categorical data will be described in terms of frequencies and percentages.

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

Treatment Research Initiative to Cure ALS (TRICALS) network’s, Precision-ALS prog (ALS/MND NHC).

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