

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

**First published:** 31/10/2025

**Last updated:** 08/04/2026

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS1000000365

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### Study ID

1000000365

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### DARWIN EU® study

No

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### Study countries

 United States

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### 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.

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## Study status

Ongoing

## Research institutions and networks

### Institutions

Biogen

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Institution

## Contact details

### Study institution contact

Study Director [clinicaltrials@biogen.com](mailto:clinicaltrials@biogen.com)

Study contact

[clinicaltrials@biogen.com](mailto: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

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### Study start date

Planned: 31/03/2026

Actual: 02/01/2026

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### Data analysis start date

Planned: 30/01/2026

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### 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

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## 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

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#### **Study type:**

Non-interventional study

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#### **Scope of the study:**

Disease epidemiology

Drug utilisation

Safety study (incl. comparative)

#### **Data collection methods:**

Combined primary data collection and secondary use of data

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#### **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-amyotrophic 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

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**Medicinal product name, other**

Tofersen

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

TOFERSEN

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## **Anatomical Therapeutic Chemical (ATC) code**

(N07XX22) tofersen

tofersen

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## **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.

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### **Age groups**

- **Adult and elderly population ( $\geq 18$  years)**
    - Adults (18 to < 65 years)
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### **Estimated number of subjects**

69

## Study design details

## **Setting**

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

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## **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).

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### **Data sources (types)**

[Disease registry](#)

## Use of a Common Data Model (CDM)

### **CDM mapping**

No

## Data quality specifications

### **Check conformance**

Unknown

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No