Spinraza (nusinersen) SMA Pregnancy Exposure Study Within Existing SMA Registries

First published: 10/05/2023

Last updated: 20/06/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS104368

Study ID

104369

DARWIN EU® study

No

Study countries

- [ ] United Kingdom
- [ ] United States

Study description

A Study of Spinraza (Nusinersen) Exposure in Pregnant Women With Spinal Muscular Atrophy (SMA) Within Existing SMA Registries

Study status

Ongoing

Research institutions and networks

Institutions

Biogen

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Institution

Contact details

Study institution contact

Clinical Trial Transparency Biogen clinicaltrials@biogen.com

Study contact

clinicaltrials@biogen.com

Primary lead investigator

Study Director Biogen

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 30/11/2023

Actual: 15/12/2023

Study start date
Planned: 30/11/2023

Actual: 15/12/2023

Data analysis start date
Planned: 30/11/2023

Actual: 15/12/2023

Date of final study report
Planned: 31/10/2033

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biogen

Study protocol

232SM405 PASS Protocol V3.0 Final.pdf (701.32 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

Other study registration identification numbers and links

NCT05789758

Link to Clinicaltrials.gov

Methodological aspects

Study type

Study type list

Study topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Combined primary data collection and secondary use of data

Study design:

An observational cohort prospective study

Main study objective:

In this study, researchers will know more about the effects of nusinersen, also known as Spinraza®, in pregnant participants with spinal muscular atrophy, also known as SMA. This is a drug available for doctors to prescribe for people with SMA. Due to the current treatment options that exist, people with SMA may now reach the age where they can become pregnant. But, there is not enough information known yet about what the effects of nusinersen may be on pregnant people with SMA or on their babies.

This is known as an “observational” study, which collects health information about study participants without changing their medical care. The pregnant participants for this study will be found using 3 different groups of SMA study research centers:

- ISMAR-US (International SMA Registry in the United States)
- UK Adult SMA-REACH (Adult SMA Research and Clinical Hub in the United Kingdom)
- SMArtCARE (Austria, Germany, and Switzerland)

The main goal of this study is to collect birth and health information from 3 groups of participants and their babies. These groups are:

· Those who received nusinersen 14 months before the first day of their last period before getting pregnant

· Those who received nusinersen 14.5 months before the day they got pregnant
· Those who received nusinersen during any time in their pregnancy

The main questions researchers want to learn about in this study are:

· Loss of pregnancy overall
· Loss of pregnancy before the baby was 20 weeks old
· Loss of pregnancy after the baby becomes 20 weeks old
· Live births
· Loss of the baby after birth
· Babies who have problems in their body that develop during pregnancy
· Babies who are small for their age while in the participant's uterus
· Pregnancy that happens outside of the uterus
· How many participants die during pregnancy, while the baby is being born, and up to 12 weeks after delivering the baby
· Babies who develop problems in their body after birth

Researchers will also compare this information to people without SMA who have not received nusinersen.

This study will be done as follows:

· Information will start being collected when the participant decides to join the study.
· Participants will be contacted at each trimester (about every 3 months) to learn about their health and pregnancy.
· Participants' doctors will be contacted at each trimester, when the participants are about 6 or 7 months pregnant, and about 4 weeks after the delivery of the baby.

· The babies’ doctors will be contacted when the baby is 1, 2, 6, 12, 18, and 24 months old.
· Each participant will be in the study until the end of their pregnancy and for up to 12 weeks after delivery. Each baby will be in the study for up to 2 years after birth.
· The study overall will last at least 10 years from when the first participant joins the study.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

SPINRAZA

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

NUSINERSEN

Medical condition to be studied

Spinal muscular atrophy

Population studied

Short description of the study population

Pregnant participants with SMA who are exposed to nusinersen from the UK-Adult SMA REACH, ISMAR-US and SMArtCARE registries will be enrolled to obtain information on effects of nusinersen on pregnancy complications and outcomes.

Age groups

- Adults (18 to < 46 years)
- Adults (46 to < 65 years)

Special population of interest

Pregnant women

Estimated number of subjects

20

Study design details

Outcomes

Number of Pregnancy Terminations, Spontaneous Abortions, Fetal Deaths, Live Births, Neonatal, Perinatal, Infant Deaths, Major Congenital Malformations(MCMs), Infants Small for Gestational Age Birth, Ectopic, Molar Pregnancies, Maternal Deaths and Infants With Abnormal Postnatal Growth and Development, Neurobehavioral Impairment

Data analysis plan

All analyses will be conducted on an overall basis, as well as stratified by earliest trimester of exposure. For MCMs, analyses will be conducted only for participants who have exposure in the first trimester.
The prevalence and 95% CIs of spontaneous abortions, MCMs, SGA births, and abnormal postnatal growth and development will be calculated.
Other negative pregnancy outcomes will be similarly examined as the sample

size permits.

Infants with minor malformations, chromosomal abnormalities, genetic syndromes, positional defects, and prematurity-related defects will be excluded from the primary analyses related to MCM prevalence, these outcomes will be reported in the interim and final reports.

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)

Longitudinal Data Collection from Patients with Spinal Muscular Atrophy (SMArtCARE)

Data source(s), other

ISMAR, United Kingdom-Adult SMA REACH

Data sources (types)

Disease registry

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