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

First published: 10/05/2023

Last updated: 26/02/2025

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/104369

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

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Clinical Trial Transparency Biogen

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 Protocol V2 Final 07Apr2023_Redacted.pdf(823.62 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:

To evaluate pregnancy complications and outcomes in subjects with SMA, birth outcomes and adverse effects in infants born to subjects exposed to nusinersen up to 14 months before first day of last menstrual period before conception, 14.5 months before conception, and/or at any time during pregnancy.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

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

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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

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)

Other

Data sources (types), other

The study will be utilizing data within existing SMA (ISMAR-US, SMArtCARE and UK-Adult SMA REACH) registries designed to evaluate pregnancy and birth outcomes in women with SMA who were exposed to nusinersen.

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