A Post-marketing, Observational, Descriptive Study to Assess the Risk Associated With Pregnancy, the Maternal Complications and Adverse Effects on the Developing Fetus, Neonate, and Infant Among Individuals Exposed to Omaveloxolone During Pregnancy and/or Lactation

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Study Planned

Administrative details

EU PAS number

EUPAS1000000327

Study ID

1000000327

DARWIN EU® study

No

Study countries

United States

Study description

The objective is to conduct a worldwide descriptive study to collect prospective and retrospective data in women exposed to omaveloxolone during pregnancy and/or lactation to assess risk associated with pregnancy, the maternal complications, and adverse effects on the developing fetus, neonate, and infant (through at least the first year of life) in the post-marketing setting.

Study status

Planned

Research institutions and networks

Institutions

Biogen

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Institution

Contact details

Study institution contact

Study Director ctrr@biogen.com

Study contact

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Primary lead investigator

Ronna Chan

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 17/05/2024

Study start date

Planned: 26/10/2026

Date of final study report

Planned: 30/04/2036

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Biogen-100%

Study protocol

296FA402 Protocol V1_Ready for HMA-EMA Upload_Redacted.pdf (3.5 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

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:

A post-marketing, prospective and retrospective study

Main study objective:

In this study, researchers will learn more about the safety of BIIB141, also known as omaveloxolone or SKYCLARYS. This is a drug available for doctors to prescribe for people with Friedrich’s Ataxia, also known as FA. This is known as an “observational” study, which collects health information about study participants without changing their medical care. Participants for this study will have taken BIIB141 at any time during pregnancy and/or while breastfeeding or pumping up through the first year after delivery. Participants can join this study on their own or they may be enrolled by their regular doctors. This study is also known as the “SKYCLARYS (Omaveloxolone) Pregnancy and Lactation Surveillance Program.”

The main objective of this study is to learn more about how BIIB141 may affect pregnancy, as well as any effects on the health of the mother and of the baby during its first year of life.

The main question researchers want to answer in this study is:

· Does taking BIIB141 during pregnancy or breastfeeding lead to any major birth defects?

Researchers will also learn more about:

· Does taking BIIB141 during pregnancy or breastfeeding lead to any minor birth defects?

· Does taking BIIB141 during pregnancy or breastfeeding affect the following:

· Gestational diabetes, a disease that can happen during pregnancy that affects how your body uses sugar

· Pre-eclampsia, a pregnancy-related high blood pressure disease

· Unborn baby being small for its expected age (usually in weeks)

· Loss of an unborn baby

· Live birth

· Premature birth

· Loss of a newborn

· Growth or developmental delays in the baby

· Serious illness in the baby resulting in hospitalization

· Serious infections in the baby, or ones in babies with a weakened immune system

This study will be done as follows:

· Participants will join the study after signing an informed consent form, also known as an ICF.

· During the study, health information from the participants’ regular visits to their doctor will be collected based on whether participant joined the study while pregnant or after the baby is born.

· Each participant will be in the study for up to 1 year after the birth of their child, unless they decide to leave early. Overall, this study is expected to last at least 10 years.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

SKYCLARYS

Medicinal product name, other

Omaveloxolone

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

The study population will include participants with FA who were exposed to omaveloxolone at any time during pregnancy and/or lactation. Participants will be enrolled prospectively and retrospectively. Women who enroll prior to pregnancy outcome, and/or prior to start of breastfeeding will be enrolled as prospective participants. Women who enroll after the pregnancy has occurred and/or following the start of breastfeeding will be enrolled as retrospective participants. Retrospective participants will not be included in the analysis population. For prospective and retrospective participants exposed during lactation only, data will be reported separately from pregnancy exposure cases.

Age groups

- **Adult and elderly population (≥18 years)**
  - Adults (18 to < 65 years)
    - Adults (18 to < 46 years)
    - Adults (46 to < 65 years)

Special population of interest

Nursing women

Pregnant women

Estimated number of subjects

10

Study design details

Outcomes

Number of Major Congenital Malformations, Number of Minor Congenital Malformations, Number of Participants With Gestational Diabetes and Number of Participants With Preeclampsia, Number of Fetal Losses due to Stillbirth, Spontaneous Abortions, Elective or Therapeutic Abortions, Number of Live Births, and Number of Preterm Births, Number of Small Gestational Age, Number of Neonatal Deaths and Number of Infant Deaths, Number of Infants With Abnormal Postnatal Growth and Development, Number of Infant Hospitalization due to Serious Illness, Number of Infant Serious or Opportunistic Infections

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Data analysis plan

Epidemiologic methods will be employed for data collection and analyses. For each continuous variable, the number of observations, median, mean, standard deviation, interquartile range, minimum, and maximum will be reported. For each categorical variable, the frequency and percentage in each category will be reported.

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 sources (types)

Administrative healthcare records (e.g., claims)

Disease registry

Electronic healthcare records (EHR)

Other

Pregnancy registry

Published literature

Data sources (types), other

Patient support groups and external data sources, such as pharmacy/medical claims or electronic medical records.

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