Risdiplam Single-Arm Pregnancy Safety Study

First published: 14/06/2022

Last updated: 02/07/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS47679

Study ID

49676

DARWIN EU® study

No

Study countries

- ☐ Germany
- ☐ Italy
- ☐ United States

Study description

This single-arm pregnancy safety study will collect primary data from risdiplam-exposed pregnant women and their healthcare professionals (HCPs), as well as their infant's HCP. Patients who have been exposed to risdiplam during 14 days prior to their last menstrual period (LMP) or at any time during pregnancy will be eligible for participation. Inclusion of exposure prior to LMP is based on pharmacokinetic characteristics of risdiplam. Dosing and treatment duration of risdiplam as part of this non-interventional study is at discretion of physician in accordance with local clinical practice and local labeling. Total duration of participation is up to 21 months (e.g. 9 months of pregnancy and 12 months of infant follow-up), and study duration is expected to be maximum of 10 years. Shorter duration may be adopted based on cases reported and in agreement with Health Authorities. Participants may enroll prospectively or retrospectively (e.g.after pregnancy outcome has occurred).

Study status

Ongoing

Research institutions and networks

Institutions

F. Hoffmann-La Roche

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Institution

Fondazione I.R.C.C.S. Istituto Carlo Besta Milan, Italy, Universitätsklinikum Essen (AöR), Klinik für Neurologie Essen, Germany, Helen DeVos Children's Hospital at Spectrum Health Grand Rapids, USA

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Marianne Gerber

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 13/09/2021

Study start date

Planned: 30/11/2022

Actual: 13/11/2023

Date of final study report
Planned: 01/05/2032

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Hoffmann-La Roche

Study protocol

Prot BN42833 risdiplam v3, Published Output-1_Redacted.pdf(1.54 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Other study registration identification numbers and links

BN42833

Methodological aspects

Study type

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Main study objective:

To collect and describe selected pregnancy outcomes and complications in women with SMA exposed to risdiplam during the defined exposure window, and to collect and describe selected fetal/neonatal/infant outcomes at birth and through up to first year of life of infants born to women exposed to risdiplam during the defined pregnancy exposure window and during breastfeeding up to 1 year after birth.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Name of medicine

EVRYSDI

Medical condition to be studied

Spinal muscular atrophy

Population studied

Age groups

Adolescents (12 to < 18 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Special population of interest

Pregnant women

Estimated number of subjects

46

Study design details

Outcomes

1) Spontaneous Abortions 2) Fetal Death or Stillbirth 3) Live Birth 4) Elective or Therapeutic Pregnancy Terminations 5) Preterm Birth 6) Congenital Malformations 7) Size for Gestational Age 8) Low Birth Weight 9) Failure to Thrive 10) Ectopic Pregnancies 11) Molar Pregnancies 12) Hospitalization of Infants 13) Neonatal Death 14) Perinatal Death 15) Infant Death 16) Maternal Death

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Data analysis plan

Data analyses will be reported in aggregate and analyzed separately for prospectively and retrospectively enrolled pregnancies. For prospectively enrolled pregnancies, descriptive analysis will be stratified considering the availability of informative prenatal testing prior to enrolment. When an informative prenatal test result is known, analyses will be reported separately for positive and negative test results, if sample size permits. Stratified analysis by prenatal test result will also be conducted for retrospectively enrolled pregnancies. Cases where the infant is diagnosed with SMA will also be reported

separately. Descriptive analyses will be performed to understand the qualitative and quantitative nature of the data collected and the characteristics of the sample studied.

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)

Other

Data sources (types), other

Prospective patient-based data collection, Retrospective patient-based data collection

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