Patisiran-LNP Pregnancy Surveillance Program

First published: 19/02/2021

Last updated: 18/09/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS36021

Study ID

42816

DARWIN EU® study

No

Study countries

- ☐ France
- ☐ Germany
- ☐ Italy
- ☐ Netherlands
- ☐ Portugal
- ☐ Spain

United States

Study status

Ongoing

Research institutions and networks

Institutions

IQVIA

United Kingdom

First published: 12/11/2021

Last updated: 22/04/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Hospital Clinico San Carlos

First published: 01/02/2024

Last updated: 01/02/2024

Institution

University of Iowa Hospitals & Clinics United States, Fondazione IRCCS Policlinico San Matteo Italy, CHU Nantes - Hôtel Dieu France,

Universitaetsklinikum Muenster Germany, Hospital Universitario Clinico San Carlos Spain, Centro Hospitalar de Lisboa Norte, E.P.E. - Hospital de Santa Maria Portugal, Universitair Medisch Centrum Groningen (UMCG) Netherlands

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Sophie Zhang

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 12/10/2018

Study start date

Planned: 01/01/2021

Actual: 01/08/2020

Date of final study report
Planned: 12/10/2030

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Alnylam Pharmaceuticals Inc.

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

ALN-TTR02-010, FDA postmarketing requirement PMR 3425-1

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

Pregnancy exposure surveillance

Main study objective:

To estimate the frequency of selected fetal/neonatal/infant outcomes at birth and through the first year of life of infants from pregnancies in women exposed to patisiran-LNP.

Study Design

Non-interventional study design

Cohort

Other

Non-interventional study design, other

Pregnancy exposure surveillance program

Study drug and medical condition

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

PATISIRAN

Medical condition to be studied

Hereditary neuropathic amyloidosis

Familial amyloidosis

Amyloidosis

Cardiac amyloidosis

Population studied

Age groups

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Infants and toddlers (28 days – 23 months)

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

10

Study design details

Outcomes

Prevalence of major congenital malformations. Prevalence of minor congenital malformations, pregnancy outcomes (live birth, spontaneous abortions, stillbirths, elective abortions, molar or pregnancy, ectopic pregnancy, preterm births, and maternal death) and other adverse fetal/neonatal/infant outcomes (low birth weight, failure to thrive, small for gestational age, postnatal growth and development, neonatal, and perinatal, or infant death).

Data analysis plan

Descriptive statistics will be used to summarize the findings. The prevalence of all outcomes will be calculated per 100 pregnancies or births, as appropriate, along with 95% CI. Exact 95% CIs including Clopper-Pearson CI for binomial proportion will be presented. The prevalence of congenital malformations will be calculated using Metropolitan Atlanta Congenital Defects Program (MACDP) and European Concerted Action on Congenital Anomalies and Twins (EUROCAT) conventions. Prevalence of congenital malformations and spontaneous abortions will be estimated based on first trimester exposure to patisiran-LNP. If too few patients are enrolled to perform the analyses described above, a case series analysis may be performed. If sample size is sufficiently large, exploratory analyses will be conducted to study the effects of the timing of patisiran-LNP exposure before and during pregnancy and cumulative exposure periods on each outcome.

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

Prospective patient-based data collection, Exposure registry

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