Patisiran-LNP Pregnancy Surveillance Program

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Study | Ongoing

Administrative details

PURI
https://redirect.ema.europa.eu/resource/42816

EU PAS number
EUPAS36021

Study ID
42816

DARWIN EU® study
No

Study countries
France
Germany
Italy
Netherlands
Portugal
Spain
United Kingdom
United States

Study status
Ongoing

Research institution 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 College London United Kingdom, 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
Lockwood Taylor

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 collect and evaluate all pregnancies in participating countries and to estimate the frequency of selected pregnancy outcomes and pregnancy complications in women exposed to patisiran-LNP during the defined exposure window. 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
Acquired ATTR 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