# Patisiran-LNP Pregnancy Surveillance Program

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# Administrative details

EU PAS number	
EUPAS36021	
Study ID	
42816	
DARWIN EU® study No	
Study countries  France	
Germany	
Italy	
☐ Netherlands	
Portugal	
Spain	

United States	
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#### **Study status**

Ongoing

### Research institutions and networks

### Institutions



### Hospital Clinico San Carlos

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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**

Karien Verhulst kverhulst@alnylam.com

Study contact

kverhulst@alnylam.com

### 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

### **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)

#### 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