

# Patisiran-LNP Pregnancy Surveillance Program

**First published:** 19/02/2021

**Last updated:** 18/09/2024

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS36021

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### Study ID

42816

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### DARWIN EU® study

No

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### Study countries

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

☐ United States

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## 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

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### Study start date

Planned: 01/01/2021

Actual: 01/08/2020

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### **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

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### **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

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**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

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**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

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**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)
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### Special population of interest

Pregnant women

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### 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).

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### **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)**

Other

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### **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

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### **Check completeness**

Unknown

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### **Check stability**

Unknown

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### **Check logical consistency**

Unknown

## Data characterisation

### **Data characterisation conducted**

No