

# Tafamidis Pregnancy Surveillance Study

**First published:** 09/09/2020

**Last updated:** 12/05/2025

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS37119

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### Study ID

47046

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### DARWIN EU® study

No

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### Study countries

-  Canada
-  France
-  Germany
-  Japan
-  Portugal
-  Spain
-  United Kingdom

## Study description

This study will describe birth outcome frequency within the reported pregnancies in women with transthyretin amyloidosis (ATTR) exposed to tafamidis during or within 1 month prior to pregnancy included in the ongoing Tafamidis Enhanced Surveillance for Pregnancy Outcomes (TESPO) surveillance program and/or standard exposure during pregnancy (EDP) and follow-up information collected in interventional studies.

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## Study status

Ongoing

# Research institutions and networks

## Institutions

**Pfizer**

**First published:** 01/02/2024

**Last updated:** 01/02/2024

**Institution**

## Contact details

### Study institution contact

Andrea Leapley [andrea.leapley@pfizer.com](mailto:andrea.leapley@pfizer.com)

### Study contact

[andrea.leapley@pfizer.com](mailto:andrea.leapley@pfizer.com)

### Primary lead investigator

Li Wang

### Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 28/04/2020

Actual: 28/04/2020

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### Study start date

Planned: 30/04/2020

Actual: 22/06/2020

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### Date of interim report, if expected

Planned: 30/04/2021

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### Date of final study report

Planned: 31/12/2030

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Pfizer 100%

# Study protocol

[B3461091\\_PROTOCOL- TAFAMIDIS ENHANCED SURVEILLANCE FOR PREGNANCY OUTCOMES\\_28Apr2020.pdf](#) (2.04 MB)

## Regulatory

**Was the study required by a regulatory body?**

Yes

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

Non-EU RMP only

## Other study registration identification numbers and links

B3461091

## Methodological aspects

### Study type

### Study type list

**Study topic:**

Human medicinal product

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**Study type:**

Non-interventional study

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**Scope of the study:**

Safety study (incl. comparative)

**Data collection methods:**

Secondary use of data

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**Study design:**

Routine pharmacovigilance activities and data collected via the TESPO program will be used to describe birth outcome frequency (live full term or premature birth, spontaneous or induced abortion, and stillbirth) of women exposed to tafamidis during or within 1 month of pregnancy.

**Main study objective:**

- 1) Describe birth outcome frequency within the reported pregnancies (live full term or premature birth, spontaneous or induced abortion, and stillbirth) in women with ATTR exposed to tafamidis during or within 1 month prior to pregnancy.
- 2) Describe the frequency of reported fetal, neonate and infant outcomes within the reported pregnancies (major congenital malformations/anomalies [overall and specific], low birth weight, small for gestational age (SGA), preterm birth, low Apgar score at 1 and 5 minutes, and infant milestone status at 6 and 12 months) following tafamidis exposure in women with ATTR during or within 1 month prior to pregnancy.

## Study Design

## **Non-interventional study design**

Other

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## **Non-interventional study design, other**

Non-interventional surveillance study

# Study drug and medical condition

## **Medicinal product name**

VYNDAQEL

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

TAFAMIDIS MEGLUMINE

TAFAMIDIS

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## **Anatomical Therapeutic Chemical (ATC) code**

(N07XX08) tafamidis

tafamidis

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## **Medical condition to be studied**

Acquired ATTR amyloidosis

# Population studied

## **Short description of the study population**

All women diagnosed with ATTR exposed to tafamidis during or within 1 month prior to pregnancy, reported as exposure during pregnancy (EDP) events in interventional or noninterventional clinical studies in the tafamidis development

program, via spontaneous reports, solicited cases or compassionate use reports, will be included.

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### **Age groups**

- Preterm newborn infants (0 - 27 days)
  - Term newborn infants (0 - 27 days)
  - Infants and toddlers (28 days - 23 months)
  - Adults (18 to < 46 years)
  - Adults (46 to < 65 years)
  - Adults (65 to < 75 years)
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### **Special population of interest**

Pregnant women

## **Study design details**

### **Setting**

Events of pregnancy are reported to Pfizer:

via clinical trials (when clinical trial participant reports pregnancy during trial);

via non-interventional studies;

via spontaneous reporting (post-marketing);

through direct contact from patients and health care professionals;

through unsolicited requests for pregnancy safety information (via Pfizer Medical Information Department);

or through contact with sales representatives.

Both maternal and paternal exposure to tafamidis were historically captured in TESPO, however, since initiation of this program it has been determined in nonclinical studies that the risk for male-mediated developmental toxicity is

considered low. Consequently, exposure during pregnancy due to paternal exposure will not be required to be captured in study B3461091.

For EDP cases occurring during sponsored clinical trials investigators are trained on how to report the case to the Sponsor, using standard pharmacovigilance forms.

For all other reportings, information regarding how to contact the Sponsor (per country specific labeling guidelines) in the event of a known pregnancy exposure is provided in the summary of product characteristics, the US package insert, the patient information leaflet, and in informational materials provided (eg, scientific meetings and direct communication) to health care professionals.

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## **Outcomes**

Measures of pregnancy and birth outcomes include preterm birth, very preterm birth, low birth weight, very low birth weight, infant mortality, fetal death (stillbirth), and spontaneous fetal losses.

In the event of a live birth, the obstetric HCP will be sent a release form to be signed by the female individual to allow the Sponsor to contact the infant's pediatric HCP for additional infant outcome data. The Sponsor will contact the pediatric HCP to complete the TESPO Infant 6 and 12-Month Follow-up Form by phone or fax.

If anomalies are noted on the EDP Form or the TESPO Infant Follow-up Forms, a more detailed investigation may be initiated by the Sponsor. For efficiency and practicality, the focus is commonly on major structural anomalies. These are defined as structural changes that have significant medical, social or cosmetic consequences for the

affected individual and typically require medical intervention. Examples include cleft lip and spina bifida. Major structural anomalies are the conditions that account for most of the deaths, morbidity and disability related to congenital anomalies.

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### **Data analysis plan**

No formal data analysis or hypothesis testing will be conducted. Data collected on exposures and outcomes will be summarized descriptively.

## 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 source(s), other**

Tafamidis Enhanced Surveillance for Pregnancy Outcomes, routine pharmacovigilance activities, and spontaneous safety reporting

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### **Data sources (types)**

[Administrative healthcare records \(e.g., claims\)](#)

[Disease registry](#)

[Electronic healthcare records \(EHR\)](#)

Other

Spontaneous reports of suspected adverse drug reactions

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**Data sources (types), other**

Prescription event monitoring

## 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