Tafamidis Pregnancy Surveillance Study

First published: 09/09/2020

Last updated: 12/05/2025

Study Ongoing

Administrative details

EU PAS number

EUPAS37119

Study ID

47046

DARWIN EU® study

No

Study countries

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

United States

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.

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

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Study contact

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Primary lead investigator

Li Wang

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 28/04/2020

Actual: 28/04/2020

Study start date

Planned: 30/04/2020

Actual: 22/06/2020

Date of interim report, if expected

Planned: 30/04/2021

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

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

Study type:

Non-interventional study

Scope of the study:

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

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

Non-interventional study design, other

Non-interventional surveillance study

Study drug and medical condition

Name of medicine

VYNDAQEL

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

TAFAMIDIS MEGLUMINE

TAFAMIDIS

Anatomical Therapeutic Chemical (ATC) code

(N07XX08) tafamidis

tafamidis

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.

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)

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.

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.

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

Data sources

Data source(s), other

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

Data sources (types)

Administrative healthcare records (e.g., claims)

Disease registry

Electronic healthcare records (EHR)

Other

Spontaneous reports of suspected adverse drug reactions

Data sources (types), other

Prescription event monitoring

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