A THAOS Sub-Study Evaluating the Effects of Tafamidis on Disease Progression in Patients with non-V30M Mutations and Symptomatic Neuropathy

First published: 30/05/2013

Last updated: 14/03/2024

Study Finalised

Administrative details

EU PAS number

EUPAS4027

Study ID

48109

DARWIN EU® study

No

Study countries

Argentina

Belgium

Brazil

Bulgaria
Cyprus
Denmark
France
Germany
Israel
Italy
Japan
Mexico
Portugal
Spain
Sweden
Taiwan
United States

Study status

Finalised

Research institutions and networks

Institutions

ICON Commercialisation & Outcomes

Germany
Ireland

First published: 19/03/2010

Last updated: 05/07/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Multiple centres: 52 centres are involved in the study

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Mary Bachinsky

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/06/2013

Actual: 24/05/2013

Study start date

Planned: 30/06/2013

Actual: 30/07/2013

Date of final study report
Planned: 31/05/2022

Actual: 11/04/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

B3461029 Tafamidis THAOS Substudy Protocol.pdf (526.91 KB)

B3461029 THAOS Substudy Protocol Amendment #1 .pdf (673.59 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 2 (specific obligation of marketing authorisation)

Other study registration identification numbers and links

B3461029

Methodological aspects

Study type

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Data collection methods:

Secondary use of data

Main study objective:

compare disease progression over at least 12 months of pre-treatment (patients will receive standard of care according to their treating physician) with disease progression over 12 months of tafamidis administration in symptomatic TTR-FAP patients with non-V30M mutations

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Descriptive analysis of a sub-set of THAOS - a multi-center longitudinal, observational, non-interventional survey

Study drug and medical condition

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

TAFAMIDIS MEGLUMINE

Medical condition to be studied

Hereditary neuropathic amyloidosis

Population studied

Short description of the study population

The study population for the described analyses will be the sub-set of patients enrolled in the THAOS registry who have a non-V30M mutation and have at least
12 months of pre-treatment data prior to receiving tafamidis. The data from all non-V30M patients enrolled in THAOS that are prescribed tafamidis will be evaluated at the time of starting tafamidis. Those that have approximately 12 months or more of pre-treatment data and a modified polyneuropathy disability (mPND) score <3 at the start of the treatment period will be included in the cohort.

Patients must meet all of the following inclusion criteria to be eligible for inclusion in the analysis cohort:
1. Patient has symptomatic peripheral and/or autonomic neuropathy with documented non-V30M mutation.
2. Must be a participant in the THAOS registry.
3. Patient must have already provided written informed consent to participate in the THAOS registry
4. Patient must be prescribed tafamidis and have >12 months of pre-treatment data in the THAOS registry. Given the non-interventional nature of this study,

patient visit schedules are according to standard of care and timing of visits will vary. Although the intent is that the pre-treatment data period will be >12 months, a minimum of 9 months will be permitted.
5. Patient must have a visit recorded in the THAOS registry within 3 weeks prior to, or 3 week after starting tafamidis (the start of treatment visit).
6. Patient must have an mPND score of <3 (does not require assistance with ambulation) at the start of tafamidis treatment.

Patients meeting any of the following criteria will not be included in the analysis cohort:
1. Patient has a documented V30M mutation.
2. Patient has received a liver transplant.
3. Patient does not have symptomatic TTR-FAP.
4. Patient has chronically (>3-4 times a month) used non-steroidal anti-inflammatory drugs (NSAIDs), other than acetylsalicylic acid, etodolac, ibuprofen, indomethicin, ketoprofen, nabumetone, naproxen, nimesulide, piroxicam, and sulindac.
5. Patient received an investigational drug, other than tafamidis, in another clinical investigational study.

Age groups

- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)
- Adults (85 years and over)

Estimated number of subjects

50

Study design details

Outcomes

disease progression, effect of tafamidis on disease progression, evaluate safety of tafamidis in TTR-FAP

Data analysis plan

Conclusions regarding the effects of tafamidis will be made based on a preponderance of evidence, including consistency and directionality, across all the endpoints. Descriptive statistics will be provided for each period (the standard of care period and the tafamidis treatment period) for each neuropathy endpoint.

Documents

Study results

B3461029 CT24-GSOP-RF26 1.0 NI Study Report Abstract .pdf (1.21 MB)

Study report

B3461029 CT24-GSOP-RF27 1.0 NI Study Report (1).pdf (2.23 MB)

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)

Disease 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