Observatory of ATTR amyloidosis and of patients treated with Vyndaqel® (tafamidis) OBSAMYL study

First published: 09/07/2018

Last updated: 23/04/2024

Study | Finalised

Administrative details

EU PAS number

EUPAS24718

Study ID

42847

DARWIN EU® study

No

Study countries

France

Study description

TTR-related amyloidosis is a systemic trouble in which extracellular transthyretin deposits aggregate in organs and tissues. In hereditary TTR-related amyloidosis, more than 100 gene mutations on transthyretin gene have been identified to be responsible for symptoms such as nerve and neurological peripheral damages. Two main phenotypic forms are encountered: transthyretin familial amyloid polyneuropathy (TTR-FAP) which is an autosomal dominant hereditary disease and transthyretin familial amyloid cardiomyopathy (TTR-FAC), occurring in 80% of patients with TTR-FAP. Prevalence of these two conditions are not well-known: TTR-FAP was estimated at 1/1.106 in general population whereas the French national registry has recorded 482 patients from 1989 to 2014. For TTR-FAC, there is a lack of epidemiological data. TTR-related amyloidosis also includes senile amyloidosis occurring predominantly in men aged over 60 years, in which amyloid deposits are derived from wild transthyretin. Prevalence of WT-TTR in the general population is not known either. Vyndaqel® (tafamidis) is a TTR tetramer stabilizer authorized in the EU market since 2012, for the treatment of TTR-FAP in stage 1 to delay neurologic peripheral impairment and is actually under clinical study for TTR-cardiomyopathy (senile amyloidosis + TTR-FAC). In the Transparency Committee's opinion of 2012, the medical value of Vyndaqel® was considered moderate and added medical value was considered minor. In this report, 200 patients were estimated eligible to receive Vyndaqel®. As part of the five-yearly renewal of tafamidis on the list of reimbursable products, its target population and modalities of use need to be assessed in 'real life’. Thus, Pfizer aims to conduct a real-life study to provide new data regarding TTR-amyloidosis and tafamidis use. This epidemiological study will rely on the French amyloidosis network. fr

Study status

Finalised

Research institutions and networks

Institutions

Kappa Santé

France

First published: 20/09/2010

Last updated: 06/03/2024

Institution | Non-Pharmaceutical company | ENCePP partner

Multiple centres: 30 centres are involved in the study

Contact details

Study institution contact

Olivier DEMARCQ olivier.demarcq@pfizer.com

Study contact

olivier.demarcq@pfizer.com

Primary lead investigator

Olivier DEMARCQ

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 01/07/2018

Actual: 01/11/2018

Study start date
Planned: 16/07/2018

Actual: 21/08/2018

Data analysis start date
Planned: 14/12/2018

Actual: 14/01/2019

Date of interim report, if expected
Planned: 10/01/2019

Actual: 20/02/2019

Date of final study report
Planned: 31/01/2019

Actual: 14/10/2020

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

OBSAMYL_protocole v3.0_20180329_VF.pdf (1.4 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Disease epidemiology

Drug utilisation

Effectiveness study (incl. comparative)

Data collection methods:

Secondary use of data

Main study objective:

1) To estimate the number of alive patients diagnosed with TTR-related amyloidosis (hereditary and senile) in France on 1st June 2017, globally, by stage of the disease, and type of mutation if applicable 2) To evaluate, in a real-life setting, modalities of tafamidis use, its safety and effectiveness profile for patients who have received tafamidis in France whether or not they are still alive

Study Design

Non-interventional study design

Cross-sectional

Other

Non-interventional study design, other

Restrospective, secondary data collection study

Study drug and medical condition

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

TAFAMIDIS

Medical condition to be studied

Hereditary neuropathic amyloidosis

Acquired ATTR amyloidosis

Cardiac amyloidosis

Population studied

Short description of the study population

All centres having the ability to treat patients for this disease have been invited to participate on behalf of the scientific committee. They include the national reference center for amyloidosis, and several regional competence centres

Patients Eligibility for the Census Part:

- Patient ≥18 years old;
- With a diagnosis of hereditary or wild-type ATTR amyloidosis or a pathogenic TTR mutation in participating centres and alive on 1st June 2017;
- Who does not oppose to his/her data collection

No Exclusion criteria

Patients Eligibility for tafamidis part:

Inclusion criteria

- Patient ≥ 18 years old;
- With at least one documented prescription of tafamidis (Vyndaqel®) outside of a clinical trial since its launch in France through an Early Access Programme (EAP)

Exclusion criteria

- Patient who participated and received tafamidis only within an interventional study evaluating the efficacy of tafamidis at time of data collection

---

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

1.300

Study design details

Outcomes

- Estimation of all cases of TTR-related amyloidosis (hereditary and senile) patients (census population) : defined in the study as the total number of patients with TTR-related amyloidosis, alive on 1st June 2017 and diagnosed in participating centers providing care for TTR-related amyloidosis. - Description of modalities of tafamidis use, and its safety and effectiveness profile, - Description of the patient's family potentially carrying a pathogenic TTR mutation (census population except for senile amyloidosis) - Time between first symptom(s) and diagnosis - Estimation of the number of patients with pathogenic TTR mutations

---

Data analysis plan

Statistical analyses will be explorative and descriptive nature. The analysis will be conducted using SAS (version 9.4 or higher, SAS Institute, North Carolina USA). All CRF parameters and evaluation criteria will be summarized using descriptive statistics: −Sample size, mean, standard deviation, minimum, median, Q1, Q3, maximum, 95% CI and number of missing data for continuous variables, −Frequency and percentage for each category, 95% CI and number of missing data for discrete variables.

Documents

Study results

Summary_results_V6.6 rapport_20201014.pdf (92.39 KB)

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.

Signed checklist for study protocols

PFE Scan.pdf (371.91 KB)

Data sources

Data sources (types)

Electronic healthcare records (EHR)

Other

Data sources (types), other

This study is a secondary data colleciton study where data collected are expected to be found mainly in patients medical records as well as in other tools used in each site as competence and reference centers of the disease.

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