A population based cohort study to monitor the safety and effectiveness of sirolimus in patients with sporadic lymphangioleiomyomatosis (S-LAM)

First published: 08/07/2019

Last updated: 23/04/2024

Study Finalised

Administrative details

EU PAS number

EUPAS30455

Study ID

47297

DARWIN EU® study

No

Study countries

United Kingdom

United States

Study description

This will be a descriptive cohort study using existing LAM data sources in the UK and the USA to evaluate S-LAM patients treated with sirolimus for long-term safety and effectiveness endpoints. Data within each of the UK and USA data sources will be used to estimate the incidence proportion of adverse events among S-LAM patients treated with sirolimus and to evaluate the selected effectiveness endpoints among S-LAM patients treated with sirolimus. In both countries, this descriptive study will include data collected in the respective data sources until 02 August 2020 which would be two years post EU S-LAM approval date of 02 August 2018. Therefore, the study observation period in the UK is from 2011 to 02 August 2020 and in the USA the study observation period is from 2015 to 02 August 2020.

Study status

Finalised

Research institutions and networks

Institutions

Pfizer

First published: 01/02/2024

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Institution

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Wu Juan (Joanne)

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 21/06/2019

Study start date

Planned: 03/08/2019

Actual: 03/08/2019

Date of interim report, if expected

Planned: 03/02/2020

Actual: 03/02/2020

Date of final study report

Planned: 03/02/2022

Actual: 31/01/2022

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Pfizer

Study protocol

B1741224 NIS Protocol_Final_25 February 2019.pdf (423.23 KB)

Regulatory

Was the study required by a regulatory body?

Yes

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

EU RMP category 3 (required)

Other study registration identification numbers and links

B1741224

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Disease /health condition

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Main study objective:

Data within each of the UK and USA data sources will be used to: 1. Estimate the incidence proportion of adverse events among S-LAM patients treated with sirolimus. 2. Evaluate the selected effectiveness endpoints among S-LAM patients treated with sirolimus.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Anatomical Therapeutic Chemical (ATC) code

(L04AA10) sirolimus

sirolimus

Medical condition to be studied

Lymphangioleiomyomatosis

Population studied

Short description of the study population

In this descriptive study all S-LAM patients enrolled in the UK and USA data sources who are treated or have been treated with sirolimus during the study observation period will be included.

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)

Special population of interest

Other

Special population of interest, other

Sporadic lymphangioleiomyomatosis patients

Estimated number of subjects

195

Study design details

Outcomes

incidence proportion of adverse events, and effectiveness endpoints such as change from baseline for forced expiratory volume in 1 second FEV1).

Data analysis plan

All analyses will be conducted by the UK and the USA principal researchers. Patient-level data will not be provided to the MAH for analysis. There will be no hypothesis testing in this study. Data will be analysed using descriptive statistical methods only. Data will be presented separately for each country (ie, data will not be pooled).

Documents

Study results

b1741224-abstract.pdf (1.75 MB)

b1741224-report-body.pdf (4.05 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