Protocol version for countries where patients treated off-label cannot be included in the disease registry: A Post-Authorisation Disease Registry Safety Study to Generate Data on the Long-Term Safety and Clinical Effectiveness of SCENESSE® (Afamelanotide 16mg) in Patients with Erythropoietic Protoporphyria (EPP). (SCENESSE® PASS-002)

First published: 01/04/2016

Last updated: 02/07/2024

Study Ongoing

Administrative details

PURI

https://redirect.ema.europa.eu/resource/46827

EU PAS number

EUPAS13004

Study ID

46827

DARWIN EU® study

No

Study countries

☐ Germany

Study description

This non-interventional study has been designed to gather long-term safety data and outcome endpoints. The objectives are to assess whether SCENESSE® can be used safely within designated treatment centres and to generate data to support the clinical effectiveness derived from the use of SCENESSE®

Study status

Ongoing

Research institutions and networks

Institutions

Porphyria Center Düsseldorf

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Institution

Contact details

Study institution contact

Pilar Bilbao

Study contact

pilar.bilbao@clinuvel.com

Primary lead investigator

Pilar Bilbao

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/06/2016

Actual: 26/09/2016

Study start date

Planned: 31/07/2016

Actual: 14/11/2016

Date of final study report

Planned: 01/06/2027

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Clinuvel

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)

Methodological aspects

Study type

Study type list

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Main study objective:

The study has been designed to assess whether SCENESSE® can be used safely within porphyria treatment centres and to generate data on long term safety and outcome endpoints derived from the use of SCENESSE®. The recommended risk minimization measures and the effectiveness of the controlled distribution of SCENESSE® will also be assessed.

Study Design

Non-interventional study design

Other

Non-interventional study design, other

Non-Interventional Study

Study drug and medical condition

Name of medicine

SCENESSE

Medical condition to be studied

Porphyria non-acute

Population studied

Age groups

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Estimated number of subjects

200

Study design details

Outcomes

Gather long-term safety data on SCENESSE®, Evaluate compliance with the risk minimization measures

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Data analysis plan

Safety assessment as outlined above will occur via the European EPP Disease Registry. Treatment-emergent adverse events and the use of concomitant medications will be assessed at every visit to the treatment centre and recorded using electronic CRFs. Compliance with the risk minimization measures will be assessed against the designated criteria throughout the study while the effectiveness of the controlled distribution of SCENESSE® will be evaluated every 12 months.Analysis of Clinical EffectivenessAll patients enrolled in the registry will be included in the assessment of clinical effectiveness so long as data are available. In general, baseline will be the data recorded on entry into the Registry. In general, clinical effectiveness will also be summarised by the subgroup variables, SCENESSE exposure at entry and Season of enrolment.

Data management

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