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





Administrative details

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

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Study contact

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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

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 Effectiveness All 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

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