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-001)

First published: 01/04/2016

Last updated: 21/01/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS13001

Study ID

46824

DARWIN EU® study

No

Study countries

Austria
Finland
Italy
Netherlands
Slovenia
United Kingdom

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

Erasmus Medical Centre Rotterdam

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Institution

Networks

Porphryia treatment centres

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Emilie Rodenburger

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 30/04/2016

Actual: 19/06/2016

Study start date

Planned: 31/05/2016

Actual: 22/06/2016

Date of final study report

Planned: 01/04/2027

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 topic:

Disease /health condition

Human medicinal product

Study type:

Non-interventional study

Scope of the study:

Other

If ‘other’, further details on the scope of the study

The study has been designed to assess whether SCENESSE® can be used safely within designated treatment centres and to generate data to support the clinical effectiveness of SCENESSE®.

Main study objective:

• Gather long-term safety data of SCENESSE®• Evaluate compliance with the risk minimization measures

Study Design

Non-interventional study design

Other

Study drug and medical condition

Medicinal product name

SCENESSE

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

AFAMELANOTIDE

Anatomical Therapeutic Chemical (ATC) code

(D02BB02) afamelanotide

afamelanotide

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 of SCENESSE®• Evaluate compliance with the risk minimization measures, • Evaluate adherence with the controlled distribution program• Generate data to contribute to knowledge about clinical benefits and to add data on potential clinical effectiveness of SCENESSE

Data analysis plan

All patients enrolled in the registry will be included in the safety assessment. In general longitudinal comparisons will be within Treated Groups (including separate groups that take into account patients who commence or discontinue treatment) and between groups, Treated versus Untreated.The number of participants with treatment-emergent adverse events will be summarized by MedDRA PT and body system. Adverse events will be further summarized by intensity, seriousness and outcome. Adverse events will also be summarized by 6 monthly time intervals from entry into the registry to assess the longitudinal effect of the treatment and EPP.Assessment of Clinical EffectivenessContinuity on treatment - together with logs detailing reasons for discontinuing (and those recommencing)Quality of Life (EPP-QoL) – will be measured using the EPP-QoL questionnaire, provided to the patient for completion at baseline prior to

treatment in Year 1 and at each subsequent visit.

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