

# 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 Protoporphyrria (EPP) (SCENESSE® PASS-001)

**First published:** 01/04/2016

**Last updated:** 21/01/2026

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS13001

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### Study ID

46824

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### DARWIN EU® study

No

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### Study countries

- Austria
  - Finland
  - Italy
  - Netherlands
  - Slovenia
  - United Kingdom
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### 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®

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### Study status

Ongoing

## Research institutions and networks

### Institutions

[Erasmus Medical Centre Rotterdam](#)

**First published:** 01/02/2024

**Last updated:** 01/02/2024

**Institution**

### Networks

# Porphryia treatment centres

## Contact details

### Study institution contact

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Study contact

[Emilie.Rodenburger@clinuvel.com](mailto:Emilie.Rodenburger@clinuvel.com)

### Primary lead investigator

Emilie Rodenburger

Primary lead investigator

## Study timelines

### Date when funding contract was signed

Planned: 30/04/2016

Actual: 19/06/2016

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### Study start date

Planned: 31/05/2016

Actual: 22/06/2016

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### 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

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### **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

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#### **Study type:**

Non-interventional study

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#### **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

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

AFAMELANOTIDE

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**Anatomical Therapeutic Chemical (ATC) code**

(D02BB02) afamelanotide

afamelanotide

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**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)
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## **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
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## **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 Effectiveness Continuity 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

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### Check completeness

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No