SCENESSE® (Afamelanotide 16mg) Retrospective Chart Review (SCENESSE® Chart Review)

First published: 02/05/2016

Last updated: 08/08/2024

Study Planned

Administrative details

EU PAS number

EUPAS13350

Study ID

46830

DARWIN EU® study

No

Study countries

- ☐ Austria
- ☐ Netherlands

Study description

In December 2014, marketing authorization for SCENESSE® was granted in Europe under exceptional circumstances (Article 14(8) of Regulation (EC) No 726/2004). To address the missing long-term safety data, the establishment of a Disease Registry was imposed as a specific obligation with data to be collected from both EPP patients and physicians. In addition, there was an obligation that the MAH should also undertake a retrospective chart review study (RCR).

On 11 April 2024, a positive opinion was received from the EMA to terminate the obligation to conduct a RCR study. This was as a consequence of no patient enrolment in the study while comprehensive scientific data on long-term safety and outcome endpoints is being generated through the specific obligation of the EPP Disease Registry (EUPAS13001/study ID 46824 and EUPAS13004/study ID 46827). The discontinuation of the RCR study will not impact the benefit-risk profile of SCENESSE® as data continues to be obtained from the Disease Registry which remains positive. Therefore the RCR study has been cancelled and will not be conducted.

Study status

Planned

Research institutions and networks

Institutions

Erasmus Medical Centre Rotterdam

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Networks

European Porphyria Initiative

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: 01/06/2016

Study start date

Planned: 01/06/2016

Data analysis start date
Planned: 01/06/2016

Date of interim report, if expected
Planned: 29/12/2016

Date of final study report
Planned: 24/12/2021

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 3 (required)

Methodological aspects

Study type

Study type list

Study type:

Clinical trial

Main study objective:

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

Study drug and medical condition

Name of medicine

SCENESSE

Medical condition to be studied

Porphyria

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

Data analysis plan

The MAH deemed the sample size to be too small to arrive at a meaningful statistical comparisons. Consequently only descriptive statistics will be used in the analyses.Safety Data Analyses:-Changes in cutaneous efflorescence -Application site reactions -Allergy and hypersensitivity -Off-label use (in children or adults without EPP) -Use in pregnancy or lactation -Administration errors Assessment of Clinical Effectiveness:-Quality of Life (EPP-QoL) -Impact on Daily Activity-Continuity on treatment Risk minimisation measures:-Changes in cutaneous efflorescence-Application site reactions -Allergy and hypersensitivity-Off-label use (in children or adults without EPP) -Use in pregnancy or lactation-Administration error

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