# A Registry of Patients with Biallelic Pro Opiomelanocortin (POMC), Proprotein Convertase Subtilisin/Kexin Type 1 (PCSK1), or Leptin Receptor (LEPR) Deficiency Obesity, or Bardet-Biedl Syndrome (BBS), Treated with Setmelanotide

**First published:** 04/01/2023

**Last updated:** 23/04/2024

Study | Planned

## Administrative details

**EU PAS number**

EUPAS48822

**Study ID**

48823

**DARWIN EU® study**

No

**Study countries**

France
Germany
Italy
Netherlands
Spain
United Kingdom
United States

**Study description**

To collect real-world, long-term safety data for the use of setmelanotide in patients with biallelic (homozygous or compound heterozygous) pro-opiomelanocortin (POMC), proprotein convertase subtilisin/kexin type 1 (PCSK1), or leptin receptor (LEPR) deficiency obesity, or Bardet-Biedl Syndrome (BBS). The primary objective of this study is to assess the long-term safety of setmelanotide as prescribed in routine practice for patients with biallelic (homozygous or compound heterozygous) POMC/PCSK1, or LEPR deficiency obesity, or BBS, according to the current local prescribing information. The secondary objectives of this study are to document and characterise adverse events of special interest (AESIs), including prolonged penile erections and depression (including suicidal ideation) for all patients, to document and characterise AESIs and adverse events (AEs) in special populations (including patients with hepatic impairment, patients with severe renal impairment, and those who become pregnant or are breastfeeding), to evaluate long-term effectiveness of setmelanotide as prescribed in routine clinical practice, and to describe baseline characteristics and history of obesity in patients treated with setmelanotide. Exploratory objectives are to document any cases of melanoma and their characteristics, and to document obesity-related hospitalisations and surgeries.

**Study status**

Planned

## Research institutions and networks

# Institutions

## PPD Evidera

Sweden

United Kingdom

United States

**First published:** 20/11/2013

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**Institution** | **Laboratory/Research/Testing facility** | **Non-Pharmaceutical company** | **ENCePP partner**

# Contact details

**Study institution contact**

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**Study contact**

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**Primary lead investigator**

Alice ROULEAU

**Primary lead investigator**

# Study timelines

**Date when funding contract was signed**
Planned: 18/08/2021

Actual: 18/08/2021

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**Study start date**
Planned: 31/03/2023

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**Date of final study report**
Planned: 30/09/2032

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Rhythm Pharmaceuticals

## 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 3 (required)

# Methodological aspects

## Study type

## Study type list

**Study type:**

Non-interventional study

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**Scope of the study:**

Assessment of risk minimisation measure implementation or effectiveness

Effectiveness study (incl. comparative)

**Main study objective:**

To assess the long-term safety of setmelanotide as prescribed in routine practice for patients with biallelic POMC/PCSK1 or LEPR deficiency obesity, or BBS according to the current local prescribing information.

## Study Design

**Non-interventional study design**

Cohort

## Study drug and medical condition

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

SETMELANOTIDE

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**Medical condition to be studied**

Pro-opiomelanocortin deficiency

Leptin receptor deficiency

Laurence-Moon-Bardet-Biedl syndrome

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**Additional medical condition(s)**

Proprotein convertase subtilisin/kexin type 1 deficiency

## Population studied

**Age groups**

- Children (2 to < 12 years)
- Adolescents (12 to < 18 years)
- Adults (18 to < 46 years)
- Adults (46 to < 65 years)
- Adults (65 to < 75 years)
- Adults (75 to < 85 years)
- Adults (85 years and over)

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**Special population of interest**

Renal impaired

Hepatic impaired

Pregnant women

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**Estimated number of subjects**

50

## Study design details

**Outcomes**

The primary outcome measure for this study is the occurrence (including seriousness and relatedness) of all AEs in patients with biallelic POMC/PCSK1 or LEPR deficiency obesity, or BBS, treated with setmelanotide. •The occurrence and characterisation of AESIs (prolonged penile erection and depression). •The occurrence and characterisation of all AEs and AESIs in patients with hepatic impairment, severe renal impairment, and pregnant/breastfeeding women. •Long-term weight change after initiation of setmelanotide •The characterisation of biallelic POMC/PCSK1 or LEPR deficiency obesity, or BBS

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**Data analysis plan**

Primary outcomes will be summarised as frequency (counts and percentages) and incidence rates for the overall population, and for new (incident), current (prevalent), or past setmelanotide users, separately. Incidence rates will be calculated by dividing the number of AEs by the number of person-years at risk. Adverse events/AESIs will be summarised using counts and percentages for the entire study population and for new (incident), current (prevalent), and past users of setmelanotide, separately. To assess setmelanotide use (past vs. current vs. new users) with baseline demographic and disease-related characteristics, a multivariable logistic regression model will be fitted against all explanatory variables, including baseline demographics and disease-related characteristics. All secondary outcomes will be summarised as frequency (counts and percentages) and incidence rates for the overall population and for new (incident), current (prevalent), and past users of setmelanotide.

# Data management

## Data sources

**Data sources (types)**

Other

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**Data sources (types), other**

Clinical information recorded in patients' medical records and/or diagnostic reports will be abstracted and entered into the eCRF in the electronic data capture (EDC) system.

## 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