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

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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

Evidera

United Kingdom

First published: 20/11/2013

Last updated: 07/03/2024

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

Contact details

Study institution contact

Alice ROULEAU alice.rouleau@evidera.com

Study contact

alice.rouleau@evidera.com

Primary lead investigator

Alice ROULEAU

Primary lead investigator

Study timelines

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

Actual: 18/08/2021

Study start date
Planned: 31/03/2023

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

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

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

Medical condition to be studied

Pro-opiomelanocortin deficiency

Leptin receptor deficiency

Laurence-Moon-Bardet-Biedl syndrome

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)

Special population of interest

Renal impaired

Hepatic impaired

Pregnant women

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

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

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