A Non-interventional Post Authorisation Safety Study (PASS) of Patients with MoCD Type A Treated with NULIBRY (fosdenopterin)

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Study Planned

Administrative details

EU PAS number

EUPAS1000000093

Study ID

1000000093

DARWIN EU® study

No

Study countries

☐ European Union

☐ United Kingdom

Study description

The NI-PASS is to facilitate the collection of additional safety and effectiveness data on NULIBRY use, including in patients with late-onset MoCD Type A, patients 1 year of age and older, who initiate treatment without dose titration, and medication errors and administration complications in the home setting, as well as pregnancy and lactation (if reported).

Treatment with NULIBRY requires specific storage, dosing, and administration, which may increase the possibility of medication errors in the home setting. There is a need to assess the risk of medication errors in the home setting in a post-approval framework. An infusion diary will be available with the drug product for the patient/caregiver to complete and monitor dates and doses administered, adverse events (AEs), medication errors and administration complications.

Objectives:

- Primary objective is the active collection of long-term safety data;
- Secondary objective is the collection of effectiveness data of all treated patients.

Study status

Planned

Research institutions and networks

Institutions

Sciensus

United Kingdom

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Institution | Pharmaceutical company

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Mathieu Loiseau

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 02/02/2024

Actual: 02/02/2024

Study start date

Planned: 01/06/2024

Date of final study report

Planned: 01/06/2025

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Sentynl Therapeutics, Inc. is the owner of the product with TMC Pharma (EU) Ltd the MAH in the EU (including EEA) and the U.K. Sciensus B.V. (Netherlands) is the appointed distributor and institution for conducting the study. Sentynl Therapeutics, Inc. is financing Sciensus B.V. to conduct the study.

Study protocol

ni-pass-protocol v1.3_7MAY2024.pdf(851.41 KB)

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 type:

Non-interventional study

Study design:

This is a multicentre, non-interventional post-authorisation safety study (NI-PASS). The purpose of this study is to describe contemporary, real-world presentation, and clinical outcomes in patients with MoCD Type A.

Main study objective:

Safety is the primary objective of this NI-PASS. Safety analyses will be performed for all patients who consented and received at least one dose of NULIBRY. The secondary objective of this NI-PASS is to evaluate the effectiveness of NULIBRY in MoCD Type A patients.

Study drug and medical condition

Name of medicine

NULIBRY

NULIBRY 9.5 MG - POWDER FOR SOLUTION FOR INJECTION

Name of medicine, other

Fosdenopterin

Anatomical Therapeutic Chemical (ATC) code

(A16AX19) fosdenopterin

fosdenopterin

Medical condition to be studied

Molybdenum cofactor deficiency

Additional medical condition(s)

MoCD Type A

Population studied

Short description of the study population

Male and female patients who have been diagnosed with MoCD Type A and have been prescribed and treated with NULIBRY will be approached for formal consent to participate in the study. Due to the life-threatening nature of the disease, and because NI-PASS will consider patients being treated and who previously received NULIBRY, patients of any age will be considered for NI-PASS inclusion.

Age groups

Paediatric Population (< 18 years)

Preterm newborn infants (0 – 27 days)

Term newborn infants (0 – 27 days)

Children (2 to < 12 years)

Adolescents (12 to < 18 years)

Adult and elderly population (≥18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Elderly (≥ 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Adults (85 years and over)

Data management

Data sources

Data sources (types)

Other

Data sources (types), other

The data will be recorded using a Case Report Form (CRF) for data collected from routine clinical care at the HCP’s office and from the infusion diary, with a unique identification for each patient. Source may include reviews of medical records, taking into account that only existing data will be entered in the CRF.

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

Unknown