A Prospective, Non-interventional (NIS), Long-term, Post-Authorisation Safety Study (PASS) of Patients Treated With Lonapegsomatropin (SkyPASS)

First published: 31/01/2023

Last updated: 18/03/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS50671

Study ID

50672

DARWIN EU® study

No

Study countries

Germany

United States

Study description

The goal of this study is to further characterise the potential long-term safety risks of lonapegsomatropin in patients treated with lonapegsomatropin under real-world conditions in the post-marketing setting.

Study status

Ongoing

Research institutions and networks

Institutions

Ascendis Pharma

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Institution

Endocrinology Division

Contact details

Study institution contact

Mandeep Singh msh@ascendispharma.com

Study contact

msh@ascendispharma.com

Primary lead investigator

Anders B. Eriksen

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 02/01/2023

Study start date

Planned: 29/09/2023

Actual: 20/03/2023

Date of final study report

Planned: 29/09/2034

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

Ascendis Pharma Endocrinology Division A/S

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:

Other

If ‘other’, further details on the scope of the study

The goal of this study is to further characterise the potential long-term safety risks of lonapegsomatropin in patients treated with lonapegsomatropin under real-world conditions in the post-marketing setting.

Main study objective:

To evaluate the occurrence of neoplasms (benign, malignant and unspecified) and type 2 diabetes mellitus in patients treated with lonapegsomatropin

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Medicinal product name

SKYTROFA

Medical condition to be studied

Growth hormone deficiency

Population studied

Age groups

- Adolescents (12 to < 18 years)
- Children (2 to < 12 years)

Estimated number of subjects

500

Study design details

Outcomes

1. To evaluate the occurrence of neoplasms (benign, malignant and unspecified) Time Frame: 5 years 2. To evaluate the occurrence of type 2 diabetes mellitus Time Frame: 5 years, 3. Occurrence of renal, hepatic, immunologic and neurologic adverse events 4. Occurrence of medication errors in patients treated with lonapegsomatropin 5. Characterise Insulin-like Growth Factor-1 (IGF-1) response 6. Compare the occurrence of neoplasms in patients treated with lonapegsomatropin 7. Compare the occurrence of type 2 diabetes mellitus in patients treated with lonapegsomatropin

Data analysis plan

The Analysis Population will be all patients enrolled. Unless otherwise specified, categorical endpoints will be summarised using counts and percent, continuous endpoints will be summarised using mean, standard deviation, median, minimum, and maximum values.

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)

Other

Data sources (types), other

Prospective patient-based data collection

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