Non-Interventional Post-Authorisation Study to Evaluate the Safety, Tolerability and Effectiveness of Berotralstat for Patients with Hereditary Angioedema (HAE) in a Real-World Setting (APeX-N)

First published: 08/10/2021

Last updated: 16/07/2024

Study Ongoing

Administrative details

EU PAS number

EUPAS43575

Study ID

43576

DARWIN EU® study

No

Study countries

France

Germany

Norway

Sweden

United Kingdom

Study description

The purpose of this study is to evaluate the long-term safety and effectiveness of berotralstat in a voluntary category 3 PASS study in a real-world setting in accordance with EMA- and MHRA-approved labelling.

Study status

Ongoing

Research institutions and networks

Institutions

Royal Free Hospital

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Institution

Contact details

Study institution contact

Study Contact BioCryst clinicaltrials@biocryst.com

Study contact

clinicaltrials@biocryst.com

Primary lead investigator

Sorena Kiani

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 03/05/2021

Actual: 27/05/2022

Study start date

Planned: 03/01/2022

Actual: 26/07/2022

Date of final study report

Planned: 05/04/2027

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

BioCryst Pharmaceuticals

Study protocol

BCX7353-401 Protocol v3.0_Redacted_10 Jul 2024.pdf (11.17 MB)

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)

Safety study (incl. comparative)

Main study objective:

To monitor safety and tolerability of berotralstat for routine prevention of attacks of hereditary angioedema in adult and adolescent patients during long-term administration in a real world setting

Study Design

Non-interventional study design

Other

Study drug and medical condition

Medicinal product name

ORLADEYO

Anatomical Therapeutic Chemical (ATC) code

(B06AC06) berotralstat

berotralstat

Medical condition to be studied

Hereditary angioedema

Additional medical condition(s)

Orladeyo is indicated for routine prevention of recurrent attacks of hereditary angioedema (HAE) in adult and adolescent patients aged 12 years and older.

Population studied

Age groups

- 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)

Estimated number of subjects

Study design details

Outcomes

- Incidence of adverse drug reactions (ADRs) - Duration of exposure to berotralstat, Height and weight in patients starting berotralstat (12 - 18 years of age only) Rate of patient-reported HAE attacks during observation period Rate of HAE attacks requiring treatment with standard of care medication Change in Angioedema Control Test Change in AE-QoL Number of days with HAE symptoms

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Data analysis plan

All safety and effectiveness variables collected in this study will be analysed descriptively. For continuous data, the mean, sample size (n), standard deviation (SD), standard error (SE), median, minimum, maximum, 25th percentile (Q1) and 75th percentile (Q3) will be provided. Categorical data will be displayed by absolute and relative frequencies (percentages). For means or proportions, 95% confidence intervals will be provided

Documents

Study publications

Lee A. Berotralstat: First Approval. Drugs. 2021 Feb;81:405-9.

Bork K, Hardt J, Witzke G. Fatal laryngeal attacks and mortality in hereditary ...

Longhurst H, Cicardi M. Hereditary angio-oedema. The Lancet. 2012 Feb 4;379(981...

Aygören-Pürsün E, Bygum A, Grivcheva-Panovska V, Magerl M, Graff J, Steiner UC,...

Lumry WR, Castaldo AJ, Vernon MK, Blaustein MB, Wilson DA, Horn PT. The humanis...

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, Retrospective 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