

# 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

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### Study ID

43576

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### DARWIN EU® study

No

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### Study countries

 France

 Germany

 Norway

 Sweden

 United Kingdom

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## 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.

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## Study status

Ongoing

# Research institutions and networks

## Institutions

### Royal Free Hospital

**First published:** 01/02/2024

**Last updated:** 01/02/2024

**Institution**

## Contact details

### Study institution contact

Study Contact BioCryst [clinicaltrials@biocryst.com](mailto:clinicaltrials@biocryst.com)

**Study contact**

[clinicaltrials@biocryst.com](mailto: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

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### Study start date

Planned: 03/01/2022

Actual: 26/07/2022

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### 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

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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)

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

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### Anatomical Therapeutic Chemical (ATC) code

(B06AC06) berotralstat

berotralstat

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### Medical condition to be studied

Hereditary angioedema

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### 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)
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### 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...

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## 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](#)

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### 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

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**Check completeness**

Unknown

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**Check stability**

Unknown

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**Check logical consistency**

Unknown

## Data characterisation

**Data characterisation conducted**

No