Retrospective analysis of adolescent patients suffering from hereditary angioedema treated with berotralstat in Europe: patients’ and treatment characteristics

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Study Planned

Administrative details

EU PAS number

EUPAS1000000997

Study ID

1000000997

DARWIN EU® study

No

Study countries

Belgium

France

Germany

Italy

Portugal

Spain

United Kingdom

Study description

This study is an international, retrospective, multicenter observational case series sponsored by Neopharmed Gentili Ireland Limited. It aims to describe the clinical characteristics, treatment patterns, and outcomes of European adolescent patients (aged 12–17 years) with hereditary angioedema (HAE) treated with berotralstat.

Data will be collected retrospectively from medical records of patients receiving berotralstat as long-term prophylaxis. The study focuses on baseline patient characteristics, disease history, treatment patterns, effectiveness outcomes (e.g., attack frequency and severity), and safety, including adverse events. Approximately 10 sites across Europe will contribute data on up to 30 patients. The study period runs from Q2 2026 to Q4 2027, with data analysis planned in end of 2027.

The objective is to generate real-world evidence on the use of berotralstat in adolescents, a population underrepresented in clinical trials, to better understand treatment effectiveness, safety, and disease evolution in routine clinical practice.

Study status

Planned

Research institutions and networks

Institutions

Neopharmed Gentili Ireland Ltd.

Contact details

Study institution contact

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Study contact

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Primary lead investigator

Mélisande Bourgoin-Heck

Primary lead investigator

Study timelines

Date when funding contract was signed

Actual: 10/12/2025

Study start date

Planned: 01/09/2026

Date of final study report

Planned: 01/01/2028

Study protocol

Protocol final version march 2025.pdf (168.79 KB)

Protocol_Berotralstat_adolescent__English_version_V3.0.docx_.pdf (522.03 KB)

Regulatory

Was the study required by a regulatory body?

No

Is the study required by a Risk Management Plan (RMP)?

Not applicable

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Study topic, other:

Hereditary angioedema

Study type:

Non-interventional study

Scope of the study:

Drug utilisation

Effectiveness study (incl. comparative)

Evaluation of patient-reported outcomes

Safety study (incl. comparative)

Data collection methods:

Secondary use of data

Study design:

Retrospective observational case series, consisting of analysis of data retrieved from medical files in adolescent patients (diagnosed 12 and 17 years old) with HAE who were started on treatment with berotralstat.

Main study objective:

The primary objective of this retrospective study is to describe the clinical profile of adolescent European HAE patients treated with berotralstat in order to characterize this population with particular attention to their baseline features and clinical context.

Study Design

Non-interventional study design

Cohort

Study drug and medical condition

Study drug International non-proprietary name (INN) or common name

BEROTRALSTAT

Anatomical Therapeutic Chemical (ATC) code

(B06AC06) berotralstat

berotralstat

Medical condition to be studied

Hereditary angioedema

Population studied

Short description of the study population

Patients eligible for inclusion into the retrospective case series must meet all the following criteria:

• Confirmed diagnosis of HAE

• Aged 12–17 years old (inclusive) and weighing at least 40 kg at time of berotralstat initiation

• Ongoing treatment with berotralstat, started after April 2021 in line with the SmPC for at least 6 months with stable dosing and without treatment interruption being advised by the treating healthcare professional.

• Patient or parent/legal guardian willing and able to provide informed consent as applicable (informed consent signed)

Age groups

- Adolescents (12 to < 18 years)

Estimated number of subjects

30

Study design details

Outcomes

Collected data related to effectiveness will include:

• Patients’ demographic characteristics (age and sex)

• History and description of HAE (date of diagnosis, HAE type, biology, familial or sporadic form)

• Description of the disease prior to berotralstat initiation (attack frequency, triggers for HAE attacks, affected body location(s) , average duration of HAE attacks (hours), average severity of attacks (mild: no impact on daily life; moderate: moderate impact; severe: laryngeal attack/abdominal attack or attack with major impact on daily life), number of hospitalizations/hospital visits within last 6 months, acute treatment of attacks, school absenteeism related to HAE, prophylactic treatment of attacks if any

• Rationale for prescribing berotralstat as orally administered LTP

• Description of berotralstat treatment (date of initiation, dose, concomitant therapies, adherence to treatment, as rated by physician

• Description of the disease within 6 months after berotralstat initiation (attack frequency, triggers for HAE attacks, affected body location(s) , average duration of HAE attacks (hours), average severity of attacks (mild: no impact on daily life; moderate: moderate impact; severe: laryngeal attack/abdominal attack or attack with major impact on daily life), number of hospitalizations/hospital visits, acute treatment of attacks, school
absenteeism related to HAE

• Physician’s global evaluation of disease evolution

• Adverse events considered related to berotralstat from berotralstat initiation to the last follow-up visit at the time of case report completion, whether they were reported to local authorities, their timing relative to berotralstat initiation, severity, imputability, and management, collected in a specific form

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Data analysis plan

The data analysis of the case series and its presentation will be purely descriptive and exploratory in nature.

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)

Non-interventional study

Use of a Common Data Model (CDM)

CDM mapping

Yes

Data quality specifications

Check conformance

Unknown

Check completeness

Unknown

Check stability

Unknown

Check logical consistency

Unknown

Data characterisation

Data characterisation conducted

Unknown