Periodic Assessment Survey of Patients/Caregivers to Assess Understanding of the Risks with the BKEMV® Risk Evaluation and Mitigation Strategy (REMS)

First published: 03/02/2025

Last updated: 07/09/2026

Study Ongoing

Administrative details

EU PAS number

EUPAS1000000368

Study ID

1000000368

DARWIN EU® study

No

Study countries

United States

Study description

The proposed study is a Risk Evaluation and Mitigation Strategy (REMS) post-marketing safety program that will be monitored by the FDA to ensure the drug (ABP 959 – BKEMV) benefits outweigh its risks.

The study will survey patients who are at least 18 years of age and caregivers of patients of all ages who have been dispensed at least 1 dose of BKEMV to assess their knowledge and understanding of the risk of severe meningococcal infections with BKEMV, the BKEMV REM requirements, and the REMS goals and materials.

Study status

Ongoing

Research institutions and networks

Institutions

Amgen

United States

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Institution

Contact details

Study institution contact

Global Development Leader Amgen Inc.
medinfo@amgen.com

Study contact

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Primary lead investigator

Global Development Leader Amgen Inc.

Primary lead investigator

Study timelines

Date when funding contract was signed
Planned: 04/11/2024

Actual: 15/05/2026

Study start date
Planned: 15/12/2025

Actual: 15/12/2025

Data analysis start date
Planned: 28/03/2028

Date of interim report, if expected
Planned: 28/05/2027

Date of final study report
Planned: 28/05/2028

Sources of funding

- Pharmaceutical company and other private sector

More details on funding

This is a study funded by Amgen.

Study protocol

Protocol-Published Original eculizumab biosimilar 20240214 .pdf (796.41 KB)

Protocol-Published Amendment eculizumab biosimilar 20240214 1 .pdf (1.14 MB)

Regulatory

Was the study required by a regulatory body?

Yes

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

Non-EU RMP only

Other study registration identification numbers and links

20240214

Methodological aspects

Study type

Study type list

Study topic:

Human medicinal product

Other

Study topic, other:

US-based, observational, cross-sectional survey of patients who are at least 18 years of age and caregivers of patients of all ages who have been dispensed at least 1 dose of BKEMV.

Study type:

Non-interventional study

Scope of the study:

Assessment of risk minimisation measure implementation or effectiveness

Data collection methods:

Primary data collection

Study design:

This US-based, observational, cross-sectional survey involves patients and caregivers who received at least one dose of BKEMV. It is self-administered via secure internet/telephone using validated vendor survey platform.

Main study objective:

The primary objectives of the study are:

To describe patient and/or caregiver knowledge of:

• Survey Knowledge Domain 1: Patients should understand that receiving treatment with BKEMV increases the chance of getting serious meningococcal infections, which may quickly become life-threatening and cause death if not recognized and treated early.

• Survey Knowledge Domain 2: Patients should understand the need to be vaccinated against meningococcal infection and receive antibacterial drug prophylaxis if needed.
• Survey Knowledge Domain 3: Patients should be able to recognize the signs and symptoms of meningococcal infection and the need for immediate medical evaluation.

Study Design

Non-interventional study design

Cross-sectional

Study drug and medical condition

Medicinal product name, other

Eculizumab-aeeb (BKEMV)

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

ECULIZUMAB

Anatomical Therapeutic Chemical (ATC) code

(L04AJ01) eculizumab

eculizumab

Medical condition to be studied

Paroxysmal nocturnal haemoglobinuria

Atypical haemolytic uraemic syndrome

Population studied

Short description of the study population

The study will include patients who are at least 18 years of age and caregivers of patients of all ages who have been dispensed at least 1 dose of BKEMV.

Age groups

- **Adult and elderly population (≥18 years)**

Estimated number of subjects

75

Study design details

Setting

Online survey

Comparators

N/A

Outcomes

The primary endpoint of the study is:

• The number of respondents who score 80% or greater in each survey knowledge domain.

Data analysis plan

Statistical analyses will focus on descriptive summaries, with formal hypothesis testing used to determine whether pre-defined knowledge rate thresholds are met. The statistical analysis plan has been revised to replace the previously used Clopper-Pearson method for confidence interval (CI) calculation with the hypergeometric method. This change provides improved accuracy for the

calculation of 95% CIs, particularly in small or finite populations, thereby strengthening the reliability of the knowledge rate estimates. The primary analytic criterion remains the lower bound of the 95% CI for each knowledge rate, which will be used to determine whether pre-defined thresholds are met.

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 source(s), other

We are conducting primary data collection, therefore we are not using any of these data sources.

Data sources (types)

Other

Data sources (types), other

We are conducting primary data collection, therefore we are not using any of these data sources.

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

Not applicable