Healthcare Professional (HCP) Survey to Assess the Effectiveness of the Additional Risk Minimization Measures (aRMM) for Casgevy® (exagamglogene autotemcel)

First published: 08/08/2025 Last updated: 08/08/2025





Administrative details

EU PAS number	
EUPAS100000704	
Charles ID	
Study ID	
100000704	
DARWIN EU® study	
No	
Study countries	
Germany	
Italy	
Spain	

Study description

This multi-national, observational cross-sectional design aims to measure the effectiveness of the aRMM for Casgevy by assessing HCPs' knowledge and behaviour in relation to the educational materials. It will specifically assess the HCPs' understanding of the important safety information in the Guide for HCPs and their awareness and utilization of the aRMM tools.

This web-based survey will target all HCPs who have counseled patients regarding Casgevy as a treatment option across all ATCs in all EU countries where Casgevy is launched approximately 18 months after first drug availability has been achieved in any EU country. The same survey will be used for all participating countries to ensure consistency in testing the target population.

Study status

Planned

Research institutions and networks

Institutions

Vertex Pharmaceuticals

First published: 01/02/2024

Last updated: 01/02/2024

Institution

Contact details

Study institution contact

Vertex Pharmaceuticals Inc Global Medical Information vertexmedicalinfo@vrtx.com

Study contact

vertexmedicalinfo@vrtx.com

Primary lead investigator

Monik Botero

Primary lead investigator

Study timelines

Date when funding contract was signed

Planned: 11/03/2024 Actual: 11/03/2024

Study start date

Planned: 30/09/2026

Data analysis start date

Planned: 01/01/2027

Date of interim report, if expected

Planned: 30/04/2027

Date of final study report

Planned: 31/12/2027

Sources of funding

• Pharmaceutical company and other private sector

More details on funding

Vertex Pharmaceuticals Incorporated

Study protocol

Vertex HCP Survey (290-102) Protocol Version 3.0_ 01April2025_Redacted.pdf (4.2 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 topic:

Other

Study topic, other:

Study of effectiveness of additional risk minimization materials

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 study uses a multi-national, observational cross-sectional design. It is a web-based survey using close-ended multiple-choice questions.

Main study objective:

- 1. The HCPs' understanding of the important safety information in the Guide for HCPs regarding the important identified risk of delayed platelet engraftment, the important potential risks of neutrophil engraftment failure and gene editing-related oncogenesis, and the missing information on long-term effects.
- 2. The HCPs' awareness of the aRMM tools.
- 3. The HCPs' utilization of the aRMM tools (behavior).

Study Design

Non-interventional study design

Cross-sectional

Study drug and medical condition

Name of medicine

CASGEVY

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

EXAGAMGLOGENE AUTOTEMCEL

Anatomical Therapeutic Chemical (ATC) code

(B06AX05) exagamglogene autotemcel exagamglogene autotemcel

Additional medical condition(s)

sickle cell disease; beta thalassemia

Population studied

Short description of the study population

The target population will be HCPs at Authorized Treatment Centers (ATC) in EU countries who have counseled patients regarding Casgevy as a treatment option.

Age groups

Adult and elderly population (≥18 years)

Adults (18 to < 65 years)

Adults (18 to < 46 years)

Adults (46 to < 65 years)

Adults (65 to < 75 years)

Adults (75 to < 85 years)

Special population of interest

Special population of interest, other

Health Care Professionals

Estimated number of subjects

30

Study design details

Setting

Data collection via a web-based survey will be initiated in all countries in the EU where Casgevy is launched approximately 18 months after first drug availability has been achieved in any EU country.

Comparators

N/A

Outcomes

The survey will commence with screening questions and will collect responses to each question required to address the study objectives:

- Response to questions about important safety information detailed in the Guide for HCPs.
- HCPs' awareness of the Guide for HCPs, Guide for Patients/Carers and Patient Card.
- Whether or not the Guide for Patients/Carers and the Patient Card are being distributed to patients treated with Casgevy.

In addition, demographic information and information on where the HCP sourced the safety information will be collected.

Data analysis plan

Data collected from the survey will be reported as descriptive statistics. Categorical variables will be summarized using counts and percentages.

Eighteen knowledge questions will test the HCPs understanding of 4 KRMs. The response to each of the 18 questions will be categorized as correct or incorrect. The table below outlines the 4 KRMs and the individual questions evaluating each message.

Using the Completed Surveys analysis set, the rate of correctness (percentage of correct answers) will be summarized for each of the 4 KRMs. The aRMM pertaining to each KRM will be considered successful if the correct rate for the specific KRM is at least 80%.

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

The data source will be surveys completed by HCPs who have counseled patients regarding Casgevy as a treatment option at ATCs.

Data sources (types)

Other

Data sources (types), other

Health Care Provider survey

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

Unknown