

# Registry study to assess the long-term safety of patients with B lymphocyte malignancies treated with tisagenlecleucel

**First published:** 30/11/2019

**Last updated:** 03/06/2025

Study

Ongoing

## Administrative details

### EU PAS number

EUPAS32497

---

### Study ID

42020

---

### DARWIN EU® study

No

---

### Study countries

☐ Australia

☐ Austria

☐ Belgium

☐ Canada

- ☐ Croatia
  - ☐ Czechia
  - ☐ Denmark
  - ☐ Finland
  - ☐ France
  - ☐ Germany
  - ☐ Greece
  - ☐ Israel
  - ☐ Italy
  - ☐ Korea, Republic of
  - ☐ Netherlands
  - ☐ Norway
  - ☐ Poland
  - ☐ Spain
  - ☐ Switzerland
  - ☐ Taiwan
  - ☐ United Kingdom
  - ☐ United States
- 

### **Study description**

This post-authorization safety study (PASS) is a global, non-interventional, multi-database study that obtains data on patients treated with marketed tisagenlecleucel in an authorized indication.

Patient data is retrieved from established Registries conducted by the following groups:

- The European Society for Blood and Marrow Transplantation (EBMT) and
  - The Center for International Blood and Marrow Transplant Research (CIBMTR)
- US
- 

### **Study status**

Ongoing

## Research institutions and networks

## Institutions

### Novartis Pharmaceuticals

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

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

Institution

## Networks

European Society for Blood and Marrow  
Transplantation (EBMT), Center for International  
Blood and Marrow Transplant Research (CIBMTR)

## Contact details

### Study institution contact

Novartis Clinical Disclosure Officer

[Trialandresults.registries@novartis.com](mailto:Trialandresults.registries@novartis.com)

Study contact

[Trialandresults.registries@novartis.com](mailto:Trialandresults.registries@novartis.com)

### Primary lead investigator

Novartis Clinical Disclosure Officer

## Study timelines

### **Date when funding contract was signed**

Planned: 21/09/2018

Actual: 21/09/2018

---

### **Study start date**

Planned: 20/12/2018

Actual: 20/12/2018

---

### **Data analysis start date**

Planned: 30/09/2042

---

### **Date of interim report, if expected**

Planned: 30/09/2020

Actual: 04/09/2020

---

### **Date of final study report**

Planned: 29/06/2043

## Sources of funding

- Pharmaceutical company and other private sector

## More details on funding

Novartis Pharma AG

# Study protocol

[ctl019b2401-v02--protocol amendment\\_Redacted.pdf](#)(396.27 KB)

[cctl019b2401-v07-protocol amendment\\_Redacted.pdf](#)(3.15 MB)

## Regulatory

### **Was the study required by a regulatory body?**

Yes

---

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

EU RMP category 1 (imposed as condition of marketing authorisation)

## Other study registration identification numbers and links

CCTL019B2401

## Methodological aspects

### Study type

### Study type list

### **Study topic:**

Human medicinal product

---

**Study type:**

Non-interventional study

---

**Scope of the study:**

Effectiveness study (incl. comparative)

Safety study (incl. comparative)

**Main study objective:**

The primary objective is to evaluate the long-term safety and the risk of secondary malignancies in patients with B lymphocyte malignancies treated with tisagenlecleucel in a real-world setting as measured by type and frequency of AEs.

## Study Design

**Non-interventional study design**

Other

---

**Non-interventional study design, other**

Non-interventional post-authorization safety study (PASS)

## Study drug and medical condition

**Name of medicine**

KYMRIAH

---

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

TISAGENLECLEUCEL

---

## **Anatomical Therapeutic Chemical (ATC) code**

(L01XL04) tisagenlecleucel

tisagenlecleucel

---

## **Medical condition to be studied**

Diffuse large B-cell lymphoma

Acute lymphocytic leukaemia

Follicular lymphoma

## **Population studied**

### **Short description of the study population**

In cohort 1: a 5-year enrollment period is planned to enroll approximately 2,500 patients with either r/r pediatric/young adult B-cell ALL (at least 1,000 patients) or with r/r large B-cell lymphoma (at least 1,500 patients).

In cohort 2: a 3-year enrollment is planned to enroll approximately 300 patients with r/r follicular lymphoma.

---

### **Age groups**

Infants and toddlers (28 days – 23 months)

Children (2 to < 12 years)

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)

---

### **Special population of interest**

Hepatic impaired

Immunocompromised

Pregnant women

Renal impaired

---

### **Estimated number of subjects**

2800

## **Study design details**

### **Outcomes**

The type and frequency of AEs (including secondary malignancies).

The identification of patients with secondary malignancies for detection of CAR transgene and/ or CAR surface expression and presence of replication-competent lentivirus.

1) Evaluate the long-term effectiveness of tisagenlecleucel by approved indication

2) Evaluate any pregnancy occurring in women of child-bearing potential or female partners of males after infusion with tisagenlecleucel

---

### **Data analysis plan**

Safety data will be summarized and listed by approved indication in interim reports up until the end of the study.

The final Clinical Study Report will be prepared including all planned effectiveness and safety analyses at the end of the study.

## **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

Center for International Blood and Marrow Transplant Research (CIBMTR) United States

European Society for Blood and Marrow Transplantation (EBMT)

---

### Data sources (types)

[Other](#)

---

### Data sources (types), other

Cellular therapy registry

## 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