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

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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

Study contact

Trialandresults.registries@novartis.com

Primary lead investigator

Novartis Clinical Disclosure Officer

Primary lead investigator

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

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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