# PLAIN LANGUAGE SUMMARY OF CLINICAL STUDY RESULTS

**Study Sponsor:** Kite, a Gilead company

**Kite Protocol Number:** KTE-C19-105

**Date of Study:** June 2017 to December 2024

**Short Study Title:** Study of Axicabtagene Ciloleucel in Participants with Relapsed/Refractory Indolent non-Hodgkin lymphoma (iNHL)

**Study Nickname:** ZUMA-5

**Date of this Plain Language Summary:** September 2025

The information in this summary does not include any information available after this date.

## Thank you

Thank you to the participants who contributed to the clinical study for **axicabtagene ciloleucel**, also known as **KTE-C19**, brand name: **Yescarta**.

Kite, a Gilead company, sponsored this study. We believe it is important to share the results with study participants and the general public.

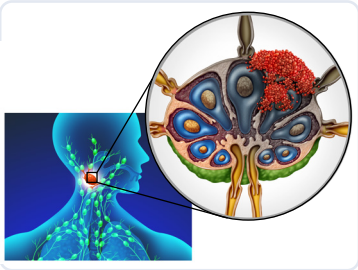
If you participated in the study and have questions about the results, please speak with a doctor or staff member at the study site.

Always talk to a doctor or healthcare provider before making any treatment changes.

This document is a short summary of this study written for a general audience. Links to scientific summaries of this study can be found at the end of this document.

# General information about the study

## What is Indolent Non-Hodgkin Lymphoma?



**Indolent non-Hodgkin lymphoma (iNHL)** is a type of blood cancer that grows slowly in the body. iNHL has many subtypes, with the 2 most common being **follicular lymphoma (FL)** and **marginal zone lymphoma (MZL)**.

**FL** begins in lymphocytes, a type of white blood cell (WBC) found inside the lymph nodes. **MZL** also starts in **lymph nodes**. **Lymph nodes** are small, pea-sized structures are located throughout the body. Together, lymph nodes and lymphocytes are part of the body's immune system—its defense system—which helps fight infections.

In people with iNHL, the lymphocytes and lymph nodes grow larger than the normal size and in an abnormal quantity. These cells don't grow or work like normal lymphocytes. Over time, these defective cells form **tumors**. **Tumors** are abnormal masses of cells. This may lead to severe infections and other health problems that can be life threatening.

**Chemotherapy** has been the standard treatment for people with iNHL. **Chemotherapy** is a combination of medicines that can kill cancer cells. However, these treatments may not be suitable for everyone or may not work for some people. Sometimes, the cancer shrinks or goes away with treatment, but it can come back later. This is called **relapsed** cancer. Other times, the cancer does not respond to treatment at all. This is called **refractory** cancer. Therefore, there is a need for new treatment options for people with relapsed or refractory iNHL (r/r iNHL).

**Axicabtagene ciloleucel (axi-cel)** is a type of **CAR T cell therapy**. It is approved as treatment for different types of blood cancers in people who have failed two or more courses of treatment with chemotherapy.

**CAR T cell therapy: CAR** stands for **chimeric antigen receptor** made in a laboratory and inserted into a type of WBC, called T cells, to better attack cancer cells. To prepare axi-cel, T cells are taken from the patient's blood, modified in a laboratory, and then put back into the patient's body to help destroy the cancer.

In this study, the researchers wanted to see if axi-cel can help people with iNHL of FL or MZL, who failed two or more courses of treatment with chemotherapy.

This is a **Phase 2** clinical study. This means that researchers tested axi-cel in a small number of people with iNHL of FL or MZL at one set dose.

## What was the purpose of the study?

The purpose of the study was to learn how axi-cel works in participants with r/r iNHL of FL or MZL.

### The main question the researchers wanted to answer in this study was:

- How many participants achieved a **complete or partial response** after taking the treatment?
  - **Complete response** means the cancer has completely gone away after taking the treatment.
  - **Partial response** means the cancer has shrunk but hasn't completely gone away. The swollen lymph nodes or other affected areas have gotten smaller, and there are no new signs of cancer.

**Objective response** includes both complete and partial responses, meaning the treatment is working to reduce the cancer.

Researchers also wanted to know if there were any side effects that participants had during the study.

# Who took part in the study?

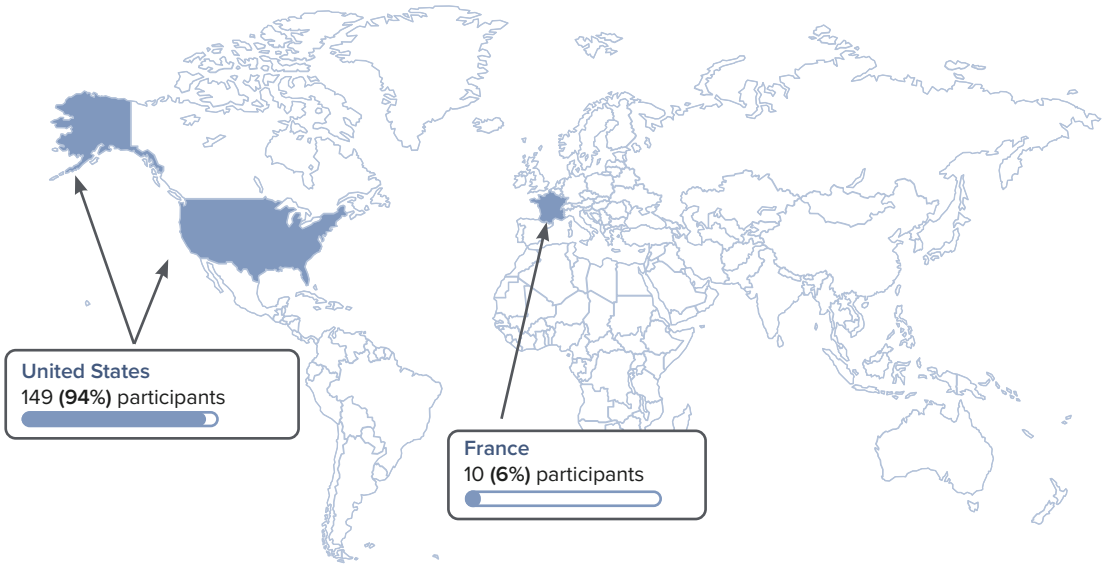
**159** participants living with iNHL in the **United States** and **France** took part in the study.

### People could take part in the study if they:

- Were at least 18 years of age
- Confirmed iNHL of FL or MZL
- Had iNHL relapse within 12 months
- Took two or more courses of treatment for r/r iNHL before, but either had relapsed disease or failed to respond

The study participants were between the ages of **34** and **79** years.

**The participants from each country are shown below (Number (%) of participants).**



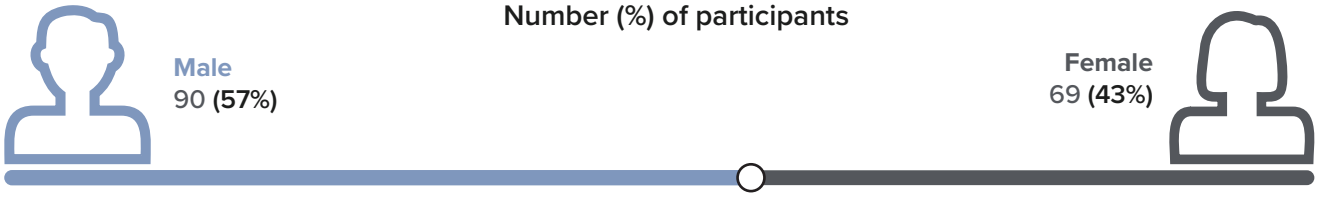

**The race of participants is shown below (Number (%) of participants).**

| | |
|---|---|
| White 146 (92%) | Other or More Than One Race 4 (3%) |
| Black or African American 6 (4%) | Asian 3 (Below 2%) |

**The ethnicity of participants is shown below (Number (%) of participants).**

| | |
|---|---|
| Not Hispanic or Latino 148 (93%) | Hispanic or Latino 10 (6%) |
| Not Collected 1 (below 1%) | |

**Sex of participants is shown below Number (%) of participants**



# What happened during the study?

This was a **single-arm**, **open-label** study.

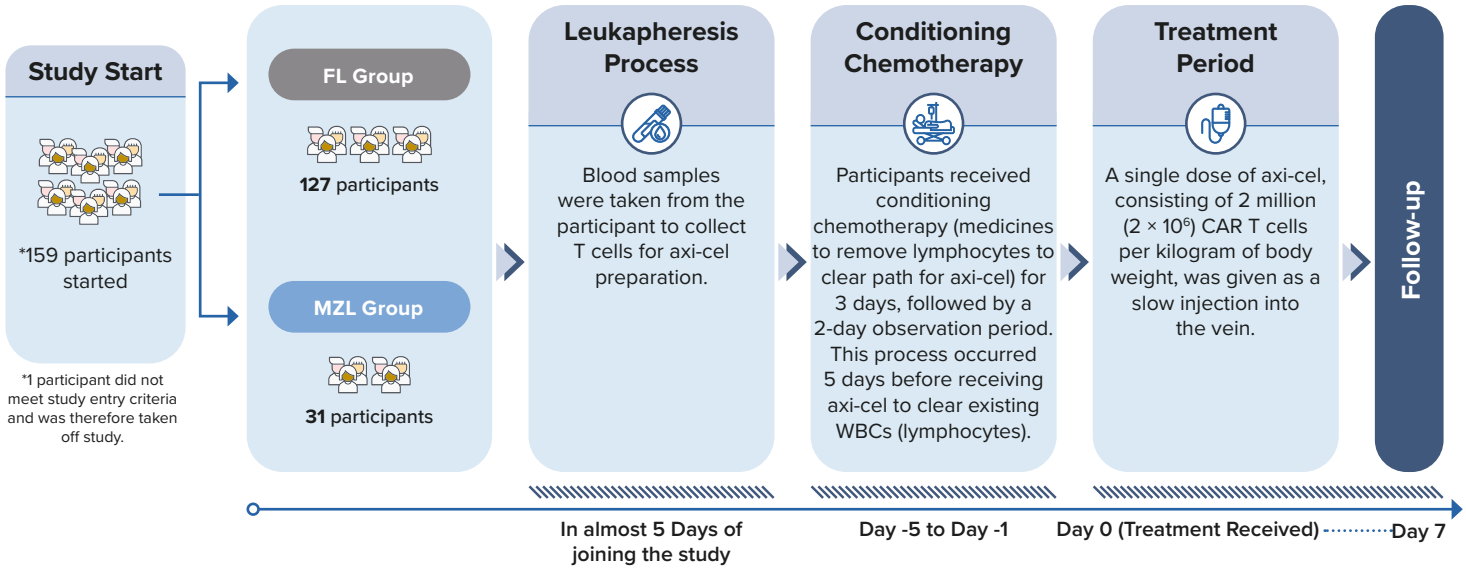
**Single-arm** means all participants received the same drug, axi-cel.

**Open-label** means the participant, the doctors and the study staff knew that they were taking axi-cel.

**Researchers enrolled participants into two groups:**

| | |
|---|---|
| **FL Group** | Included participants with follicular lymphoma |
| **MZL Group** | Included participants with marginal zone lymphoma |

**The graphics below show the study treatment:**




## Axi-cel Retreatment

If a participant responded to treatment but later relapsed, the study doctor allowed them to receive axi-cel again. The doctor only gave the treatment again if the cancer still had a certain marker and the participant didn't have serious side effects from the first axi-cel treatment.

Study doctors monitored participants in the hospital for 7 to 10 days after they received axi-cel. After discharge, they advised participants to stay near the hospital or clinic for at least 4 weeks in case they needed urgent care.

After a minimum of 5 years in FL Group or 2 years in the MZL group, remaining participants were transitioned to a separate long-term follow-up study so their safety could continue to be monitored for a total duration of 15 years.

# What were the results of the study?

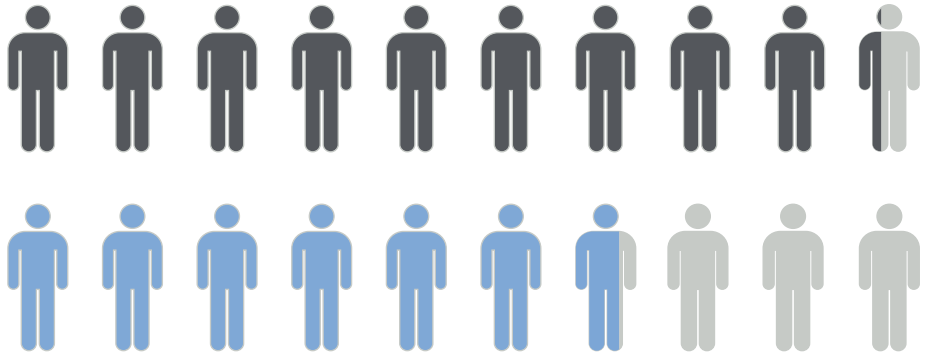
This is a summary of the main results from this study. The individual results of each participant might be different and are not in this summary. A detailed presentation of the results can be found on the websites listed at the end of this summary.

**How many participants achieved a complete or partial response after taking the treatment?**

The researchers wanted to find out how many participants had a complete or partial response to the treatment. To do this, they compared scans and blood tests taken before and after treatment. They looked to see if the lymph nodes and other affected areas had returned to normal and whether any new tumors had appeared. They also checked if the existing tumors had shrunk in size.

Of the 159 participants who started the study, the results for 127 participants in the FL Group and 29 participants in the MZL group were available and are reported below.

**The graphics below show the participants who achieved complete or partial response in each group.**



**92%**
**FL Group**
117 out of 127 participants

**69%**
**MZL Group**
20 out of 29 participants

# What side effects did participants have during the study?

Unwanted medical events can happen to the study participants when they take a study drug. In this summary, "**side effects**" are defined as unwanted medical events that the study doctors thought might be caused by the study drug.

The results from several studies are usually needed to help decide if a study drug actually causes a side effect.

Of the 159 participants, 7 did not receive any study treatment. The side effects results include the remaining 152 participants: 124 in the FL group and 28 in the MZL group. Among them, 14 FL and 2 MZL participants received retreatment with axi-cel.

A side effect is considered "**serious**" if it:

- results in death
- is life-threatening
- is considered by the study doctor to be medically important
- causes lasting problems
- requires hospital care
- causes a birth defect

The table below shows how many participants had side effects during the study.

**Overall Side Effects**

| | FL Group (Out of 124 participants) | MZL Group (Out of 28 participants) | Retreatment FL Group (Out of 14 participants) | Retreatment MZL Group (Out of 2 participants) | Total (Out of 152 participants) |
|---|---|---|---|---|---|
| | Number (%) of participants | | | | |
| How many participants had **any** side effects? | 122 (98%) | 28 (100%) | 13 (93%) | 2 (100%) | 151 (99%) |
| How many participants had **any serious** side effects? | 47 (38%) | 16 (57%) | 3 (21%) | 0 | 66 (43%) |
| How many participants **died** due to side effects during the study? | 3 (2%) | 1 (4%) | 1 (7%) | 0 | 5 (3%) |

**None of the participants stopped the treatment due to any side effects.**

**5 out of 152 (3%) treated participants** died due to serious side effects during the study.

- **FL group**: 3 out of 124 (2%) participants died due to side effects. 1 death was from a type of blood cancer (acute bilineal leukemia), 1 from COVID-19 related lungs infection (COVID-19 pneumonia), and 1 from multiple organ failure (multiple organ dysfunction syndrome).
- **MZL group**: 1 out of 28 (4%) participants died due to a type of blood cancer (acute myeloid leukemia)
- **FL Retreatment group**: 1 out of 14 (7%) participants died due to an infection in brain (progressive multifocal leukoencephalopathy)

## What were the serious side effects?

The table below shows the serious side effects that occurred in at least 2% participants during the study.

**Serious Side Effects**

| | FL Group (Out of 124 participants) | MZL Group (Out of 28 participants) | Retreatment FL Group (Out of 14 participants) | Retreatment MZL Group (Out of 2 participants) | Total (Out of 152 participants) |
|---|---|---|---|---|---|
| | Number (%) of participants | | | | |
| Fever (Pyrexia) | 14 (11%) | 6 (21%) | 0 | 0 | 20 (13%) |
| A brain condition that seriously affect how a person thinks and acts (Encephalopathy) | 8 (6%) | 3 (11%) | 0 | 0 | 11 (7%) |
| Confusional state | 7 (6%) | 0 | 1 (7%) | 0 | 8 (5%) |
| A condition where not enough oxygen reaches the body's tissues (Hypoxia) | 3 (2%) | 2 (7%) | 0 | 0 | 5 (3%) |
| Presence of fluid in the lungs, usually as a result of infection (Pneumonia) | 5 (4%) | 0 | 0 | 0 | 5 (3%) |
| Sleepiness (Somnolence) | 2 (2%) | 3 (11%) | 0 | 0 | 5 (3%) |
| Low blood pressure (Hypotension) | 2 ( 2%) | 2 (7%) | 0 | 0 | 4 (3%) |
| A disorder where the bone marrow produces faulty blood cells (Myelodysplastic syndrome) | 3 (2%) | 1 (4%) | 0 | 0 | 4 (3%) |
| A condition that makes it hard for a person to talk, understand others, read, or write, because of damage to the brain (Aphasia) | 2 (2%) | 1 (4%) | 0 | 0 | 3 (2%) |

### What were the non-serious side effects?

The table below lists the most common non-serious side effects that occurred in more than 35% of the study participants. These side effects were not serious in nature and did not meet the definition of 'serious side effects' mentioned in the section above in this summary.

**The table below shows how many participants had non-serious side effects during the study.**

**Non-Serious Side Effects**

| | FL Group (Out of 124 participants) | MZL Group (Out of 28 participants) | Retreatment FL Group (Out of 14 participants) | Retreatment MZL Group (Out of 2 participants) | Total (Out of 152 participants) |
|---|---|---|---|---|---|
| | Number (%) of participants | | | | |
| Fever (Pyrexia) | 97 ( 78%) | 24 ( 86%) | 8 ( 57%) | 2 (100%) | 125 (82%) |
| Low blood pressure (Hypotension) | 48 ( 39%) | 14 ( 50%) | 2 ( 14%) | 1 (50%) | 63 (41%) |
| Low number of red blood cells (Anemia) | 43 ( 35%) | 14 ( 50%) | 2 ( 14%) | 1 (50%) | 58 (38%) |
| Extreme tiredness (Fatigue) | 43 ( 35%) | 13 ( 46%) | 3 ( 21%) | 2 (100%) | 58 (38%) |
| Feeling sick to the stomach (Nausea) | 41 ( 33%) | 15 ( 54%) | 5 ( 36%) | 2 (100%) | 58 (38%) |
| Low number of white blood cells (Neutropenia) | 47 ( 38%) | 9 ( 32%) | 2 ( 14%) | 0 | 58 (38%) |

There were other serious and non-serious side effects, but those occurred in fewer participants. Some participants may have had more than 1 serious and non-serious side effects.

## ? How has this study helped researchers?

The researchers learned more about the safety of axi-cel and how it works in people with r/r iNHL of FL or MZL.

The results from several studies are needed to help decide which treatments work and are safe. This summary shows only the main results from this one study. Other studies may provide new information or different results.

Gilead Sciences does plan to have further clinical studies with axi-cel.

## Where can I learn more about this study?

You can find more information about this study on the websites listed below.

| Organization (Website) | Study Identifier |
|---|---|
| European Medicines Agency<br>www.euclinicaltrials.eu | EU CT Number: 2023-505169-10 |
| United States National Institutes of Health (NIH)<br>www.clinicaltrials.gov | ClinicalTrials.gov Number: NCT03105336 |
| Gilead Website<br>www.gileadclinicaltrials.com | KTE-C19-105 |

Please note that information on these websites may be presented in a different way from this summary.

**Full Study Title:** A Phase 2 Multicenter Study of Axicabtagene Ciloleucel in Subjects with Relapsed/Refractory Indolent Non-Hodgkin Lymphoma (iNHL)

To learn more about clinical trials in general,
please visit this page on www.clinicaltrials.gov website

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Clinical study participants belong to a large community of people who take part in clinical research around the world. They help researchers answer important health questions and find medical treatments for patients.

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