



PLAIN LANGUAGE SUMMARY OF CLINICAL STUDY RESULTS

Study Sponsor: Kite, a Gilead Company

Kite Protocol Number: KTE-C19-104

Date of Study: February 2016 to March 2026

Plain Language Study Title: Study of KTE-X19 in Children and Teenagers With B-precursor Acute Lymphoblastic Leukemia or B-Cell Non-Hodgkin Lymphoma

Study Nickname: ZUMA-4

Date of this Plain Language Summary: September 2026

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 **KTE-X19**, also known as **brexucabtagene autoleucel**. In addition, thank you to the parents and caregivers of the participants.

Kite, a Gilead company sponsored this study. This summary is prepared for 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.

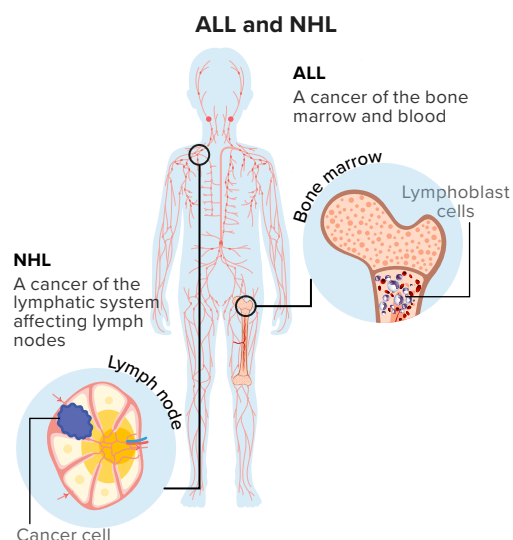
Always talk to a doctor or healthcare provider before making any treatment changes.

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 are B-precursor Acute lymphoblastic leukemia and B-cell non-Hodgkin Lymphoma?



B-precursor acute lymphoblastic leukemia (B-precursor ALL) is a blood cancer that starts in the bone marrow, the soft tissue inside bones that makes blood cells. In B-precursor ALL, the body makes too many abnormal young B cells (lymphoblasts), which do not work properly.

B-cell non-Hodgkin lymphoma (B-cell NHL) is a group of blood cancers that affects mature B cells. These abnormal B cells can grow out of control and form masses in the lymph nodes or other parts of the body. Lymph nodes are small structures that help the body fight infections.

Both ALL and NHL can affect people of any age. However, B-precursor ALL is more common in children and young people. In both diseases, cancer cells can crowd out healthy blood cells and cause symptoms such as fever, tiredness, frequent infections, bruising or bleeding, and swollen lymph nodes.

Doctors use **chemotherapy** (medicines to kill cancer cells) to treat these cancers, but sometimes the cancer comes back called, cancer relapse (r) or does not respond to treatment called as refractory (r) disease.

What is KTE-X19?

KTE-X19 also called as brexucabtagene autoleucel (brexu-cel), is a type of **CAR T-cell therapy**. It is an approved treatment for adults with B-precursor ALL and mantle cell lymphoma, a type of B-cell NHL. This study looked at whether brexu-cel could help children, teenagers, and young adults with relapsed or refractory (r/r) B-precursor ALL or r/r B-cell NHL.



CAR T-cell therapy is a form of cancer treatment that uses the patient's own immune cells. **CAR** stands for **chimeric antigen receptor**, a special protein made in a laboratory. To prepare brexu-cel, T-cell lymphocytes—a type of white blood cell (WBC) that helps fight infection—are collected from the patient's blood through a process called **leukapheresis**. These cells are then modified in the laboratory to express CAR protein. Once ready, the modified cells (called CAR T cells, brexu-cel) are infused back into patient's bloodstream through an injection into a vein, where they work to identify and destroy cancer cells.



What was the purpose of the study?

The purpose of this study was to identify a suitable dose of brexu-cel and learn how safe and effective it was in children, teenagers, and young adults with r/r ALL or r/r NHL.

The main questions the researchers wanted to answer in this study were:

- How many participants had **dose-limiting toxicities (DLTs)** with a higher dose of brexu-cel?
- How many participants responded to brexu-cel treatment?

Researchers also wanted to know if participants had any **side effects** during the study.



A **DLT** is a medical event that is severe enough to stop the study doctor from increasing the participant's dose of brexu-cel. This is done to find a safe dose of the drug.



Who took part in the study?

A total of **95** children, teenagers, and young adults took part in this study. Of these, **79** had r/r ALL and **16** had r/r NHL.

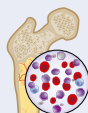
People could take part in the study if they:



Were up to 21 years of age



Were 6 kg or more in weight



Had confirmed r/r ALL or r/r NHL



Had 2 or more previous courses of failed chemotherapy treatment

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

United States	66 (70%)	Italy	3 (3%)
France	10 (11%)	Poland	3 (3%)
Canada	6 (6%)	Czech Republic	1 (1%)
Spain	5 (5%)	Germany	1 (1%)

The race of participants is shown below (number of participants (%)).

White	61 (64%)	Black or African American	5 (5%)
Other or More Than One Race	25 (26%)	Asian	4 (4%)

The ethnicity of participants is shown below (number of participants (%)).

Not Hispanic or Latino	45 (47%)	Hispanic or Latino	43 (45%)
Not Collected	7 (7%)		

The age of participants is shown below (number of participants (%)).



The sex of participants is shown below (number of participants (%)).



Male
61 participants (64%)



Female
34 participants (36%)

? What happened during the study?

Before joining the study, researchers did tests to see whether people met the study requirements. They also did blood tests, bone marrow tests, and scans to see how far their cancer has spread.

This was an **open-label** and **single-arm** study.

i **Open-label** means the participant, the doctors and the study staff knew that the participants were receiving brexu-cel. **Single-arm** means all participants received the same drug, brexu-cel.

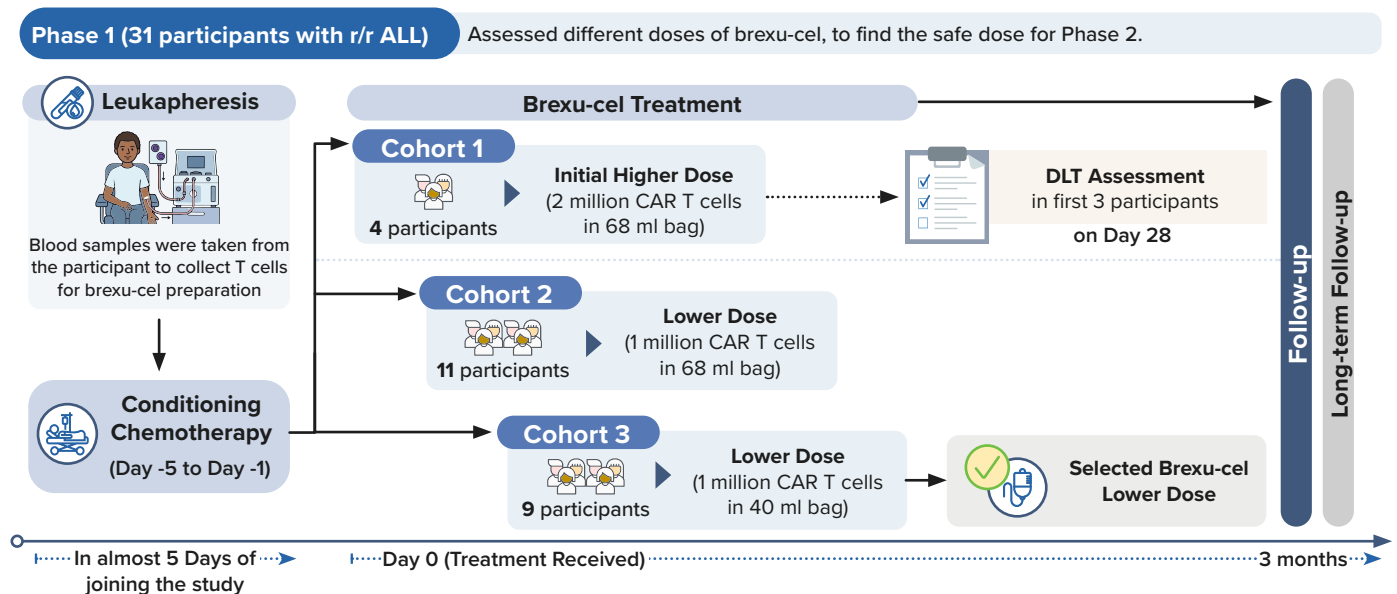
At the start of the study, 95 participants underwent leukapheresis. Of these, 81 received **conditioning chemotherapy** and brexu-cel treatment. **Conditioning chemotherapy** reduces lymphocytes and help make space for the CAR T cells to grow and work effectively. Participants received this therapy for 3 days to prepare their bodies for treatment. All participants received a single dose of brexu-cel per kilogram (kg) of their body weight, as a slow injection into a vein.

The study had 2 parts: **Phase 1** and **Phase 2**. Phase 1 included 31 participants with r/r ALL. Phase 2 included 64 participants: 48 with r/r ALL and 16 with r/r NHL.

In **Phase 1**, researchers tested different doses of brexu-cel in 3 small **cohorts** of participants with r/r ALL. A **cohort** is a group of participants who received the same dose of brexu-cel. Researchers first tested a higher dose and monitored participants for DLTs. They then tested a lower dose in other 2 cohorts to identify a safe dose for Phase 2.

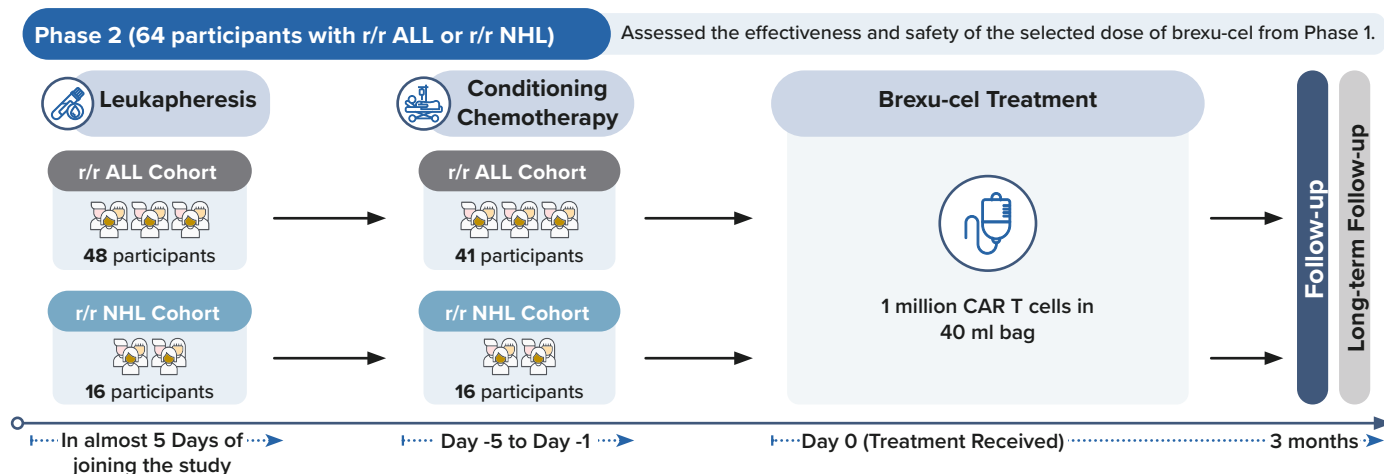
- **Cohort 1:** 5 participants had leukapheresis and 4 received conditioning chemotherapy and brexu-cel at a dose of **2 million CAR T cells per kg in a 68 ml bag**.
- **Cohort 2:** 13 participants had leukapheresis and 11 received conditioning chemotherapy and brexu-cel at a dose of **1 million CAR T cells per kg in a 68 ml bag**.
- **Cohort 3:** 13 participants had leukapheresis and 9 received conditioning chemotherapy and brexu-cel at a dose of **1 million CAR T cells per kg in a 40 ml bag**.

The graphic below shows treatment plan of Phase 1 of the study:



In **Phase 2**, researchers assessed the safety and effectiveness of the selected dose of brexu-cel, **1 million CAR T cells per kg in a 40 ml bag**, in 2 additional cohorts including 64 participants with **r/r ALL** or **r/r NHL**.

The graphic below shows treatment plan of Phase 2 of the study:



Follow-up: Participants received brexu-cel in the hospital and stayed there for 7 to 10 days for monitoring. After leaving the hospital, they returned for follow-up visits 14 days, 28 days, 8 weeks, and 3 months after treatment.

Long-term Follow-up: All participants were moved to a separate long-term follow-up study (Study KT-US-982-5968; NCT number: NCT05041309; EU CT number: 2023-507041-28), so their safety could continue to be monitored for a total duration of 15 years.

The study was completed as planned. However, enrollment of additional participants with r/r ALL was stopped during Phase 2 of the study, due to safety concerns, resulting in fewer participants with r/r ALL than originally planned.

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 mentioned in this summary. A detailed presentation of the results can be found on the websites listed at the end of this summary.

The results of participants who received brexu-cel treatment are reported below.

How many participants had dose-limiting toxicities (DLTs) with a higher dose of brexu-cel?

The researchers closely monitored the first 3 participants with r/r ALL in Cohort 1 for DLTs during Phase 1. DLTs were side effects related to brexu-cel that occurred within the first 28 days after participants received the treatment.

None of the participants had any DLTs after receiving brexu-cel at a dose of 2 million CAR T cells per kg of body weight.

The sponsor tested a lower dose of brexu-cel containing 1 million CAR T cells and found it to be safer for participants in Phase 2.

How many participants responded to brexu-cel treatment?

Researchers did blood tests, bone marrow tests, and scans to see whether the selected dose of 1 million CAR T cells of brexu-cel was helping participants in Phase 2. Researchers defined a response as **complete remission, with or without normal blood counts**, or **partial remission**.

Complete remission means no signs of cancer were found after treatment. **Partial remission** means the cancer became smaller or less severe after treatment, but it did not completely go away.

Researchers looked at how many participants with r/r ALL achieved complete remission and how many participants with r/r NHL achieved complete or partial remission.

The graphics below show how many participants responded to brexu-cel treatment.



More than half of the participants with r/r ALL achieved complete remission. In participants with r/r NHL, some had strong and long-lasting responses, but others did not respond well and their disease got worse.

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

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

Overall Side Effects					
	Phase 1 (Participants with r/r ALL)			Phase 2	
	Cohort 1	Cohort 2	Cohort 3	r/r ALL Cohort	r/r NHL Cohort
	4 participants	11 participants	9 participants	41 participants	16 participants
	Total				
	81 participants				
	Number of participants (%)				
How many participants had any side effects?	4 (100%)	10 (91%)	9 (100%)	41 (100%)	12 (75%)
How many participants had any serious side effects?	4 (100%)	6 (55%)	4 (44%)	28 (68%)	7 (44%)
	Total				
	76 (94%)				
	49 (60%)				

One participant in Phase 2 r/r ALL Cohort died due to a build-up of extra fluid in the brain that caused the brain to swell (brain edema).

What were the serious side effects?

The table below shows the serious side effects that occurred in **at least 6%** of the total number of study participants.

Serious Side Effects						
	Phase 1 (Participants with r/r ALL)			Phase 2		Total
	Cohort 1	Cohort 2	Cohort 3	r/r ALL Cohort	r/r NHL Cohort	
	4 participants	11 participants	9 participants	41 participants	16 participants	
	Number of participants (%)					
Fever (Pyrexia)	1 (25%)	4 (36%)	4 (44%)	9 (22%)	2 (13%)	20 (25%)
Low blood pressure (Hypotension)	3 (75%)	5 (45%)	2 (22%)	9 (22%)	0	19 (23%)
Fast heart rate (Tachycardia)	0	2 (18%)	4 (44%)	4 (10%)	0	10 (12%)
A condition where a person feels confused and less alert or aware than usual (Confusional state)	0	3 (27%)	0	3 (7%)	2 (13%)	8 (10%)
A brain condition that seriously affects how a person thinks and acts (Encephalopathy)	1 (25%)	1 (9%)	2 (22%)	3 (7%)	1 (6%)	8 (10%)
Headache	0	2 (18%)	2 (22%)	2 (5%)	1 (6%)	7 (9%)
A condition where not enough oxygen reaches the body's tissues (Hypoxia)	1 (25%)	0	0	5 (12%)	1 (6%)	7 (9%)
A condition that makes it hard for a person to talk, understand others, read, or write, because of damage to the brain (Aphasia)	0	2 (18%)	0	3 (7%)	1 (6%)	6 (7%)
A sudden change in how the brain works that can cause shaking, stiffness, unusual movements, or changes in awareness (Seizure)	1 (25%)	0	0	4 (10%)	1 (6%)	6 (7%)
A build-up of extra fluid in the brain that causes the brain to swell (Brain edema)	0	1 (9%)	0	4 (10%)	0	5 (6%)
Fever with a low number of WBCs called neutrophils (Febrile neutropenia)	0	1 (9%)	0	2 (5%)	2 (13%)	5 (6%)

What were the non-serious side effects?

The table below lists the most common non-serious side effects that occurred in **at least 30%** of the total number of study participants. These side effects were not serious in nature and did not meet the definition of 'serious side effects' mentioned in this summary.

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

Non-Serious Side Effects						
	Phase 1 (Participants with r/r ALL)			Phase 2		Total
	Cohort 1	Cohort 2	Cohort 3	r/r ALL Cohort	r/r NHL Cohort	
	4 participants	11 participants	9 participants	41 participants	16 participants	
	Number of participants (%)					
Fever (Pyrexia)	3 (75%)	5 (45%)	3 (33%)	31 (76%)	7 (44%)	49 (60%)
Headache	2 (50%)	1 (9%)	3 (33%)	17 (41%)	5 (31%)	28 (35%)
Low number of red blood cells (Anemia)	0	2 (18%)	5 (56%)	14 (34%)	5 (31%)	26 (32%)
Low blood pressure (Hypotension)	2 (50%)	4 (36%)	2 (22%)	13 (32%)	3 (19%)	24 (30%)

? How has this study helped researchers?

The researchers learned more about the safety of brexu-cel and how it works in children, teenagers, and young adults with r/r ALL or r/r NHL.

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 not plan to have further clinical studies with brexu-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 www.clinicaltrialsregister.eu	EU CTIS number: 2023-509440-97-00
United States National Institutes of Health (NIH) www.clinicaltrials.gov	ClinicalTrials.gov Number: NCT02625480
Gilead Website www.gileadclinicaltrials.com	KTE-C19-104

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

Full Study Title: A Phase 1/2 Multi-Center Study Evaluating the Safety and Efficacy of KTE-X19 in Pediatric and Adolescent Subjects with Relapsed/Refractory B-precursor Acute Lymphoblastic Leukemia or Relapsed/Refractory B-Cell Non-Hodgkin Lymphoma

To learn more about clinical trials in general,
please visit this [page](#) on www.clinicaltrials.gov website

Kite, a Gilead Company
2400 Broadway, Santa Monica, CA 90404, USA
Email: medinfo@kitepharma.com



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.

