# PLAIN LANGUAGE SUMMARY OF CLINICAL STUDY RESULTS

**Study Sponsor:** Kite Pharma, a division of Gilead Sciences

**Kite Study Number:** KT-US-486-0201

**Dates of Study:** July 2021 to May 2024 (the study closed earlier than planned)

**Short Study Title:** Study of KITE-222 in People With Relapsed/Refractory Acute Myeloid Leukemia

**Date of this Plain Language Summary:** April 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 **KITE-222.**

Kite Pharma, a division of Gilead Sciences 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.

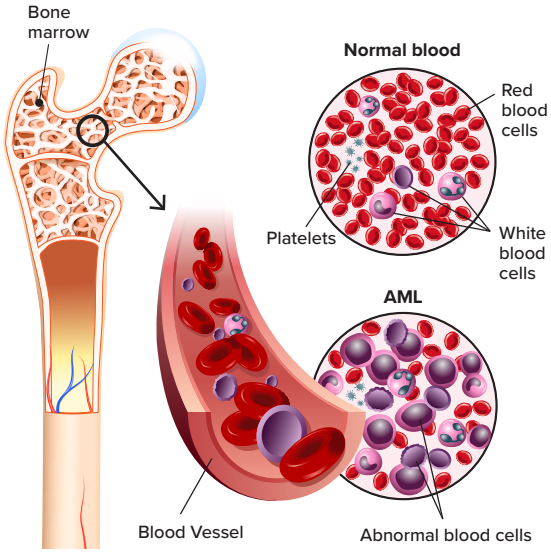
Always talk to a doctor or healthcare provider before making any treatment changes.

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 Acute Myeloid Leukemia (AML)?

**AML** is a type of blood cancer. In healthy people, the blood-forming cells (**stem cells**, also known as myeloid cells) in the bone marrow makes 3 main types of blood cells: red blood cells (RBCs), white blood cells (WBCs), and platelets. The bone marrow is a spongy tissue found inside the bone.




In AML, the bone marrow makes too many defective cells called **blast cells**. These cells don't grow or work like normal blood cells. Over time, these defective cells build up and crowd out healthy blood cells. This may lead to severe infections and other health problems leading to death.

**Chemotherapy** has been the standard treatment for people with AML. Chemotherapy is a combination of medicines that can kill cancer cells. There are other alternative treatment options being studied to treat AML. However, these treatments may not be suitable for everyone or may not work for some people. Sometimes, cancer shrinks or goes away with treatment, but it can come back later this is called **relapsed cancer**. Other times, cancer does not respond to treatment at all, it is called **refractory cancer**.

## What is KITE-222?

**KITE-222** consists of modified chimeric antigen receptor (CAR) T cells. T cells are a type of lymphocyte. Lymphocytes are a type of WBCs, that are a part of the immune system and helps fight infections. KITE-222 is prepared using the patient's own T cells which are taken from the blood, genetically modified in the laboratory, and then given back to the patient as a slow injection into a vein.

In this study, researchers aimed to learn more about a drug called KITE-222 as a treatment for people with relapsed or refractory AML. This included people who developed AML with or without any previous experience of blood disorders or prior cancer treatments.

This is a **Phase 1 clinical study**. This means that researchers looked at how KITE-222 worked in the human body. The researchers tested KITE-222 in the human body for the first time during this study.

## What was the purpose of the study?

The purpose of this study was to learn more about the safety and dosing of KITE-222 in participants with relapsed or refractory AML.

Researchers wanted to find the highest safe dose of KITE-222, known as the maximum tolerated dose (MTD), that did not cause any harmful side effects. They studied different doses of KITE-222 and closely monitored participants for any signs of **dose-limiting toxicity (DLT)**.

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

- How many participants had **dose-limiting toxicities (DLTs)** during the study, if any?

  A **DLT** is a medical event that is severe enough to stop the study doctor from increasing the participant's dose of KITE-222

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

# Who took part in the study?

- **15 participants** living with relapsed or refractory AML from France and the United States took part in this study.
- **3 participants** left the study before taking KITE-222 and were not included in the study results.

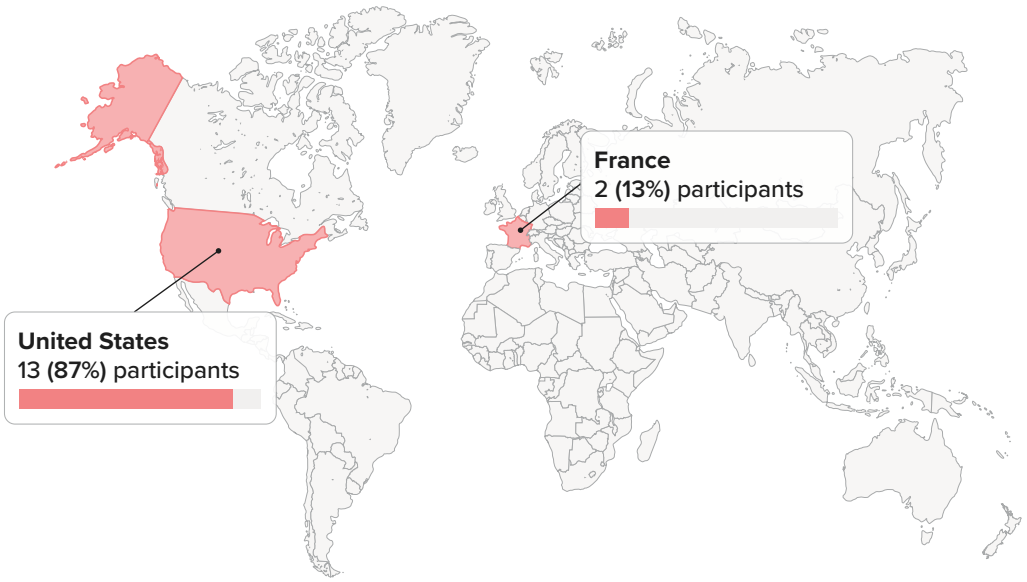
**People could take part in the study if they:**

- Were at least 18 years of age
- Had relapsed or refractory AML
- Had received prior treatments for AML that did not work well or had stopped working
- Were deemed fit for **stem-cell** transplant and had a donor identified before the study started

**Stem-cell** transplant is a procedure that replaces cancer cells with healthy cells in the bone marrow. People could received stem cells from a donor.

Participants were **26 to 76 years** old when they joined the study.

The map below shows how many study participants were from each country.

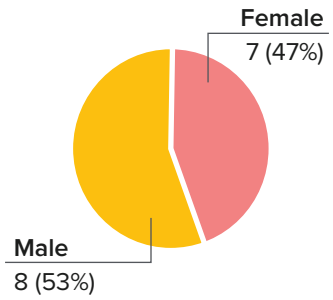



The sex, race, and ethnicity of participants are shown below.

| Race | Number of participants (%) |
|---|---|
| White | 11 (73%) |
| Other or more than one race | 3 (20%) |
| Black or African American | 1 (7%) |

| Ethnicity | Number of participants (%) |
|---|---|
| Not Hispanic or Latino | 13 (87%) |
| Hispanic or Latino | 1 (7%) |
| Not collected | 1 (7%) |

**Sex**



# What happened during the study?

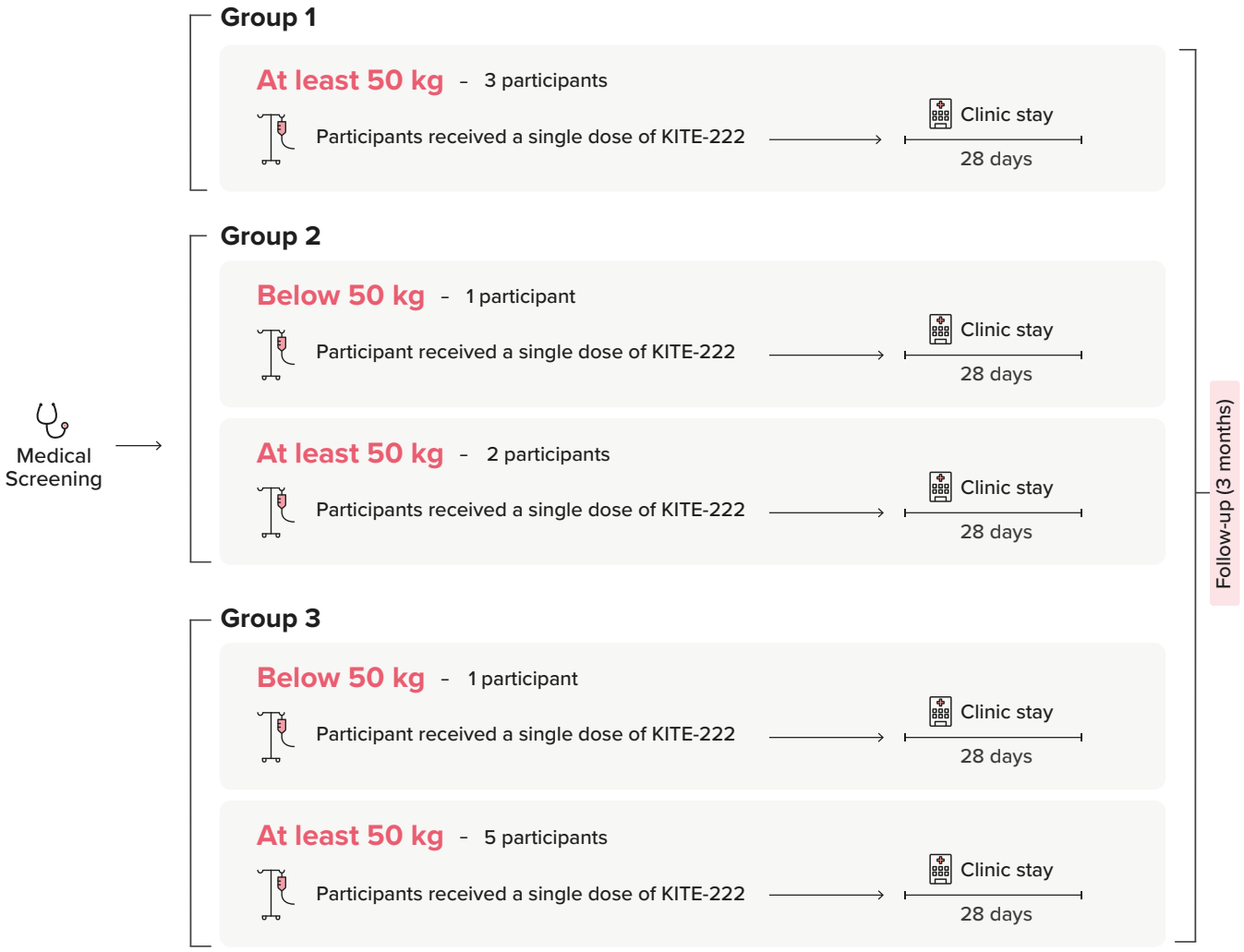
At the start of the study, participants had a medical screening to see if they could participate in the study. Study doctors checked the participants' health and medical history. Each participant had an imaging scan to see how far their cancer had spread. This was an **open-label** study, which means each participant knew what treatment they were taking, and the doctors and study staff also knew.

During the study, participants provided their blood samples. Each sample was used to collect the T-cells, which were then used to produce KITE-222. Participants whose AML got worse during this part of the study could receive standard care of treatment. Standard of care was the best treatment for AML that was available to researchers at the time they were treating each participant.

Before participants took KITE-222, they received chemotherapy for a total of 3 days. This was done 5 days before taking KITE-222. These included:

- **Cyclophosphamide**, given as a slow injection into the vein
- **Fludarabine**, given as a slow injection into the vein

Participants were placed into 1 of 3 treatment groups. KITE-222 was given as a single dose of slow injection into the vein. The doses were based on participants' weight. Participants in Group 1 started with a low dose of KITE-222. The study doctors reviewed the results from these participants. Based on these results, they decided whether to increase the dose for the next group (a process called dose escalation). Researchers use dose escalation studies to learn about the safety of a specific dose before giving a higher dose to participants. In this study, Group 1 received a low dose, Group 2 a higher dose, and Group 3 the highest dose.



After receiving KITE-222, participants stayed at the study clinic for 28 days. During this period, study doctors observed participants for any medical issues. Participants had a follow-up visit up to 3 months after they received KITE-222. Study doctors looked at the results of participants who received KITE-222. If participant's neutrophil levels (a type of white blood cell that fights infections) stayed low for a long time, the doctor might decide they need a stem-cell transplant.

The study planned to enroll more participants once researchers identified a suitable dose of KITE-222. However, after analyzing the data of the participants in Groups 1, 2, and 3, study doctors found that KITE-222 did not benefit the participants. **As a result, Kite decided to discontinue the study. This was not due to any medical issues that participants had during the study. The study was stopped because KITE-222 did not work as expected.**

## 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 had dose-limiting toxicities (DLTs) during the study, if any?

The study doctors checked participants for DLTs. DLTs were KITE-222 related medical events that participants had within the first 28 days after they received KITE-222.

The results included 11 participants who received KITE-222 within Groups 1, 2, or 3, and had a follow-up for a minimum of 28 days after the first dose.

The table below shows the number of participants who had DLTs during the study.

| Dose-limiting Toxicities | | | | |
|---|---|---|---|---|
| | **Group 1** (out of 3 participants) | **Group 2** (out of 3 participants) | **Group 3** (out of 5 participants) | **Total** (out of 11 participants) |
| | **Number of participants (%)** | | | |
| How many participants had DLTs? | 0 | 0 | 1 (20%) | 1 (9%) |

Overall, 1 participant in Group 3 had a DLT during the study. Researchers looked at the results and the KITE-222 doses tested within Groups 1 and 2 were considered safe.

# What side effects did participants have during the study?

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

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

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

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

There were 3 participants who did not take any study treatment. So, the results in this section only include 12 participants.

**No participants died because of side effects.**

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

| Overall side effects | | | |
|---|---|---|---|
| | **Group 1** (out of 3 participants) | **Group 2** (out of 3 participants) | **Group 3** (out of 6 participants) |
| | **Number of participants (%)** | | |
| How many participants had serious side effects? | 0 | 1 (33%) | 2 (33%) |
| How many participants had any side effects? | 1 (33%) | 3 (100%) | 5 (83%) |

The table below shows all the serious side effects that occurred during the study. Some participants may have had more than 1 serious side effect.

| Serious side effects | | | |
|---|---|---|---|
| | **Group 1** (out of 3 participants) | **Group 2** (out of 3 participants) | **Group 3** (out of 6 participants) |
| | **Number of participants (%)** | | |
| **Excess fluid around the heart which prevents it from pumping enough blood** (Cardiac tamponade) | 0 | 1 (33%) | 0 |
| **Disease of the brain that severely alters thinking** (Encephalopathy) | 0 | 0 | 1 (17%) |
| **Immune system is overactive and attacks normal cells** (Haemophagocytic lymphohistiocytosis) | 0 | 0 | 1 (17%) |
| **Low blood pressure** (Hypotension) | 0 | 0 | 1 (17%) |
| **Collection of fluid around the heart** (Pericardial effusion) | 0 | 1 (33%) | 0 |
| **Inflammation of the membrane around the heart** (Pericarditis) | 0 | 1 (33%) | 0 |

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

The table below shows the most common non-serious side effects that occurred in more than 30% of the study participants during the study. 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.

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

| Most common non-serious side effects | | | |
|---|---|---|---|
| | **Group 1** (out of 3 participants) | **Group 2** (out of 3 participants) | **Group 3** (out of 6 participants) |
| | **Number of participants (%)** | | |
| **Fever** (Pyrexia) | 1 (33%) | 2 (67%) | 5 (83%) |
| **Low blood pressure** (Hypotension) | 0 | 2 (67%) | 2 (33%) |
| **Decreased level of oxygen reaching body tissues** (Hypoxia) | 0 | 1 (33%) | 3 (50%) |

## How has this study helped researchers?

The researchers learned more about the safety of KITE-222 in people living with relapsed or refractory AML.

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.

KITE decided to discontinue the study after analyzing the data of 12 participants. The study was stopped because KITE-222 did not work as expected and not due to any medical issues that the participants had during the study.

Gilead Sciences does not plan to have further clinical studies with KITE-222.

## 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: 2023-507748-35-00 |
| **United States National Institutes of Health (NIH)**<br>www.clinicaltrials.gov | ClinicalTrials.gov ID: NCT04789408 |
| **Gilead Clinical Trials**<br>www.gileadclinicaltrials.com | KT-US-486-0201 |

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

**Full Study Title:** A Phase 1 Open-label, Multicenter Study Evaluating the Safety of KITE-222, an Autologous Anti-CLL-1 CAR T-cell Therapy, in Subjects With Relapsed/Refractory Acute Myeloid Leukemia

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

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*Thank you*

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.

GILEAD