

# Long-term follow-up after "ready-made" CAR T cells

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<b>Registration date</b> 10/09/2024	<b>Overall study status</b> Ongoing	<input type="checkbox"/> Statistical analysis plan <input type="checkbox"/> Results
<b>Last Edited</b> 06/06/2025	<b>Condition category</b> Cancer	<input type="checkbox"/> Individual participant data <input checked="" type="checkbox"/> Record updated in last year

## Plain English summary of protocol

### Background and study aims

In this observational study, we are monitoring the long-term patients who previously received 'ready-made' CAR T cells within phase 1 clinical trials. 'Ready-made' CAR T cells are universal cellular products that derive from healthy donor white blood cells. As they are created by using special gene editing techniques, they are called "genome-edited allogeneic CAR-T cells". They aim to fight and reduce leukaemia ahead of bone marrow transplant (BMT) and they are administered to patients with relapsed/refractory acute lymphoblastic or myeloid leukaemia. They are not expected to persist after BMT. This study aims to monitor long-term outcomes and adverse effects after 'ready-made' CART cells and bone marrow transplants. To date, three different 'ready-made' CAR T have been used in phase I trials at Great Ormond Street Hospital, which are PBLTT52CAR19 for B-cell acute lymphoblastic leukaemia, BE-CAR7 for T-cell acute lymphoblastic leukaemia and BE-CAR33 for acute myeloid leukaemia.

### Who can participate?

Children previously administered with one of the mentioned products and who have completed the treatment study will be eligible to participate in this long-term study.

### What does the study involve?

Up to 20 patients will be included in this study which will take place at Great Ormond Street Hospital. The start of this study will be after the treatment study, 12 or 24 months after allogeneic BMT (depending on which treatment study patients were enrolled). As long-term persistence of CAR T cells beyond SCT is not anticipated, only routine blood tests are required. Patients will be monitored with yearly visits up to 15 years after BMT. This study will use arrangements already in place in BMT long-term follow-up. Study visits will match with the planned ones and blood samples will be collected as part of normal routine care.

### What are the possible benefits and risks of participating?

There is no foreseeable risk to participants as this is a follow-up and monitoring study. No IMP administrations are expected. Moreover, the study will align with the already in-place BMT long-term follow-up and will not add extra blood tests, clinical examinations or procedures. Follow-up visits will take place together with standard planned visits.

Where is the study run from?

Great Ormond Street Hospital for Children NHS Foundation Trust

When is the study starting and how long is it expected to run for?

January 2024 to September 2042

Who is funding the study?

Great Ormond Street Hospital for Children NHS Foundation Trust

Who is the main contact?

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## **Additional identifiers**

### **Integrated Research Application System (IRAS)**

1009055

### **Protocol serial number**

23IC21

## **Study information**

### **Scientific Title**

An observational, long-term post-transplant follow-up after allogeneic genome edited lentiviral transduced CAR T cells

### **Acronym**

LTalloCAR

### **Study objectives**

The primary objective is to detect long-term effects after genome edited lentiviral transduced allogeneic universal CAR T cell therapy, including the following Investigational Medicinal Products (IMPs), PBLTT52CAR19, BE-CAR7 and BE-CAR33.

The secondary objectives of the trial are:

- To track mononuclear cell chimerism for the recipient, transplant donor, and IMP (CAR-T) donor signals
- To confirm non-persistence of IMP (by chimerism assay)
- To monitor progression-free survival
- To assess overall survival

### **Ethics approval required**

Ethics approval required

### **Ethics approval(s)**

approved 04/03/2024, London – West London & GTAC Research Ethics Committee (2 Redman Place, Stratford, London, E20 1JQ, United Kingdom; +44 (0)207 104 8241; westlondon.rec@hra.nhs.uk), ref: 24/LO/0005

### **Study design**

Observational long-term post-transplant follow-up study

### **Primary study design**

Observational

### **Study type(s)**

Safety, Efficacy

### **Health condition(s) or problem(s) studied**

Relapsed/refractory acute myeloid leukaemia and relapsed/refractory acute lymphoblastic leukaemia

### **Interventions**

This study is a long-term observational follow-up of patients who received allo-CAR Investigational Medicinal Products (IMPs) after allogeneic stem cell transplantation (allo-SCT). The study aims to assess the long-term safety of these therapies, with follow-up lasting 15 years from the date of IMP administration. Routine blood tests will be conducted annually to monitor safety, with the first visit 24-36 months post-transplant.

Participants: Eligible participants are those who have completed Phase 1 of their treatment with specific CAR T cells:

- PBLTT52CAR19 for B-ALL
- BE-CAR7 for T-ALL
- BE-CAR33 for AML

Assessments:

- Routine blood tests to monitor chimerism, immune recovery, infections, and graft-versus-host disease (GVHD).
- Safety assessments include tracking new hematologic disorders, immune recovery, and disease outcomes.

Follow-up Schedule:

The follow-up will integrate into the standard of care for patients post-SCT, aligning with existing long-term monitoring practices. The first follow-up visit will occur 24-36 months after allo-HSCT, according to the original treatment study schedule. Inclusion status will be confirmed at the time of enrolment. Follow-up visits will be conducted annually from Year 2 or Year 3 until Year 15, with a window of  $\pm 1$  month.

## Intervention Type

Drug

## Phase

Not Applicable

## Drug/device/biological/vaccine name(s)

Allogeneic genome edited lentiviral transduced CAR T cells. [For BE CAR33 T cells: TCR $\alpha\beta$ -CAR33+ T For BE CAR7 T cells: CAR7+TCR $\alpha\beta$ -\_T-cells For PBLTT52CAR19 T cells: CAR19+TCR $\alpha\beta$ -\_T-cells]

## Primary outcome(s)

The identification and documentation of long-term adverse effects following genome-edited allogeneic universal CAR T cell therapy and allogeneic transplant (PBLTT52CAR19, BE-CAR7, and BE-CAR33), including non-persistence of IMP beyond allo-SCT (via chimerism testing), the occurrence of new haematologic disorders (cytopenia, malignancies), autoimmune disorders, immune recovery (T-cell, B-cell aplasia, immunoglobulins), severe viral infections, engraftment, chimerism status, and new or ongoing GVHD grade 3 or higher, assessed annually from year 2 or 3 through year 15 (+/- 1 month)

## Key secondary outcome(s)

The tracking of mononuclear cell chimerism for the recipient, transplant donor, and IMP (CAR-T) donor signals to confirm the non-persistence of IMP (by chimerism assay). Chimerism status in blood will be performed annually from year 2 or 3 through year 15 (+/- 1 month)

## Completion date

30/09/2042

## Eligibility

### Key inclusion criteria

The principal inclusion criteria are:

1. Written informed consent obtained prior to any study-specific procedure (patient or parent(s) or legal representative)
2. Patients affected by advanced lymphoid or myeloid leukemia, who have been administered with allogeneic genome edited lentiviral CAR T cells and had allo-SCT over 12 months ago

### Participant type(s)

Patient

### Healthy volunteers allowed

No

### Age group

Child

### Sex

All

### Total final enrolment

**Key exclusion criteria**

Not meeting the inclusion criteria

**Date of first enrolment**

10/01/2025

**Date of final enrolment**

01/06/2027

## Locations

**Countries of recruitment**

United Kingdom

**Study participating centre**

-

United Kingdom

-

## Sponsor information

**Organisation**

Great Ormond Street Hospital for Children NHS Foundation Trust

**ROR**

<https://ror.org/03zydm450>

## Funder(s)

**Funder type**

Hospital/treatment centre

**Funder Name**

Great Ormond Street Hospital for Children

**Alternative Name(s)**

GOSH

**Funding Body Type**

Government organisation

**Funding Body Subtype**

Local government

**Location**

United Kingdom

## **Results and Publications**

**Individual participant data (IPD) sharing plan**

The datasets generated and/or analysed during the current study will be published as a supplement to the results publication

**IPD sharing plan summary**

Published as a supplement to the results publication