



## Clinical trial results:

**A phase 1 / 2, open-label, single arm, multicohort, multicenter trial to evaluate the safety and efficacy of JCAR017 in pediatric subjects with relapsed/refractory B-ALL and B-NHL (TRANSCEND PEDALL)**

### Summary

EudraCT number	2018-001246-34
Trial protocol	FR DE ES IT BE NL
Global end of trial date	26 January 2024

### Results information

Result version number	v1 (current)
This version publication date	07 August 2024
First version publication date	07 August 2024

### Trial information

#### Trial identification

Sponsor protocol code	JCAR017-BCM-004
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#### Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03743246
WHO universal trial number (UTN)	-

Notes:

### Sponsors

Sponsor organisation name	Bristol-Myers Squibb
Sponsor organisation address	Chaussee de la Hulpe 185, Brussels, Belgium, 1170
Public contact	EU Study Start-Up Unit, Bristol-Myers Squibb International Corporation, Clinical.Trials@bms.com
Scientific contact	Bristol-Myers Squibb Study Director, Bristol-Myers Squibb, Clinical.Trials@bms.com

Notes:

### Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMA-001995-PIP01-02
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

## Results analysis stage

Analysis stage	Final
Date of interim/final analysis	26 January 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	26 January 2024
Was the trial ended prematurely?	Yes

Notes:

## General information about the trial

Main objective of the trial:

The primary objective of Phase 1 is to identify the recommended Phase 2 dose (RP2D) in pediatric subjects with relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL).

Protection of trial subjects:

Patient Confidentiality, Personal Data Protection and Biomarker Consent

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	17 October 2018
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

## Population of trial subjects

### Subjects enrolled per country

Country: Number of subjects enrolled	Netherlands: 1
Country: Number of subjects enrolled	Germany: 1
Country: Number of subjects enrolled	Spain: 10
Country: Number of subjects enrolled	Italy: 9
Worldwide total number of subjects	21
EEA total number of subjects	21

Notes:

### Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	3
Children (2-11 years)	15
Adolescents (12-17 years)	3
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details: -

### Pre-assignment

Screening details:

The study was terminated and hence participants were not enrolled in Phase 2 cohorts (r/r B-ALL; MRD+ B-ALL; r/r B-NHL).

### Period 1

Period 1 title	Pre-Treatment Period
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Not blinded

### Arms

Are arms mutually exclusive?	Yes
<b>Arm title</b>	0.05 x 10 <sup>6</sup> CAR+ T cells/kg

Arm description:

Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) with 0.05 x 10<sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.

Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

JCAR017 Infusion was administered on Day 1 intravenously

<b>Arm title</b>	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
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Arm description:

Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10<sup>6</sup> CAR+ T cells/kg post leukapheresis.

Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

JCAR017 Infusion was administered on Day 1 intravenously

<b>Arm title</b>	0.50 x 10 <sup>6</sup> CAR+T cells/kg
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Arm description:

Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) with 0.50 x 10<sup>6</sup> CAR+T cells/kg post leukapheresis.

Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

**Dosage and administration details:**

JCAR017 Infusion was administered on Day 1 intravenously

<b>Arm title</b>	Not Assigned
Arm description: Participants only underwent leukapheresis.	
Arm type	No intervention
No investigational medicinal product assigned in this arm	

<b>Number of subjects in period 1</b>	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Started	9	8	3
Completed	8	7	1
Not completed	1	1	2
Adverse event, serious fatal	-	1	-
Failure to meet treatment criteria	-	-	2
Death	-	-	-
Study Drug Manufacturing Failure	1	-	-

<b>Number of subjects in period 1</b>	Not Assigned
Started	1
Completed	0
Not completed	1
Adverse event, serious fatal	-
Failure to meet treatment criteria	-
Death	1
Study Drug Manufacturing Failure	-

**Period 2**

Period 2 title	Lymphodepleting chemotherapy
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Not blinded

**Arms**

Are arms mutually exclusive?	Yes
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<b>Arm title</b>	0.05 x 10 <sup>6</sup> CAR+ T cells/kg
Arm description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.05 x 10 <sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.	
Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use
Dosage and administration details: JCAR017 Infusion was administered on Day 1 intravenously	
<b>Arm title</b>	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Arm description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10 <sup>6</sup> CAR+ T cells/kg post leukapheresis.	
Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use
Dosage and administration details: JCAR017 Infusion was administered on Day 1 intravenously	
<b>Arm title</b>	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Arm description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.50 x 10 <sup>6</sup> CAR+T cells/kg post leukapheresis.	
Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use
Dosage and administration details: JCAR017 Infusion was administered on Day 1 intravenously	

<b>Number of subjects in period 2</b>	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Started	8	7	1
Completed	7	7	1
Not completed	1	0	0
Progressive Disease	1	-	-

**Period 3**

Period 3 title	Treatment Period (JCAR017 Infusion)
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Not blinded

**Arms**

Are arms mutually exclusive?	No
<b>Arm title</b>	0.05 x 10 <sup>6</sup> CAR+ T cells/kg

Arm description:

Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.05 x 10<sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.

Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

JCAR017 Infusion was administered on Day 1 intravenously

<b>Arm title</b>	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
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Arm description:

Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10<sup>6</sup> CAR+ T cells/kg post leukapheresis.

Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

JCAR017 Infusion was administered on Day 1 intravenously

<b>Arm title</b>	0.50 x 10 <sup>6</sup> CAR+T cells/kg
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Arm description:

Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.50 x 10<sup>6</sup> CAR+T cells/kg post leukapheresis.

Arm type	Experimental
Investigational medicinal product name	JCAR017 Infusion
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Injection
Routes of administration	Intravenous use

Dosage and administration details:

JCAR017 Infusion was administered on Day 1 intravenously

<b>Number of subjects in period 3</b>	<b>0.05 x 10<sup>6</sup> CAR+ T cells/kg</b>	<b>0.15 x 10<sup>6</sup> CAR+ T cells/kg</b>	<b>0.50 x 10<sup>6</sup> CAR+T cells/kg</b>
Started	7	7	1
Completed	3	6	1
Not completed	4	1	0
Adverse event, serious fatal	1	-	-
Study Terminated by sponsor	1	-	-
Progressive Disease	1	-	-
Other Reason	1	-	-
Lost to follow-up	-	1	-

## Baseline characteristics

### Reporting groups

Reporting group title	0.05 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) with 0.05 x 10 <sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.	
Reporting group title	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10 <sup>6</sup> CAR+ T cells/kg post leukapheresis.	
Reporting group title	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) with 0.50 x 10 <sup>6</sup> CAR+T cells/kg post leukapheresis.	
Reporting group title	Not Assigned
Reporting group description: Participants only underwent leukapheresis.	

Reporting group values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Number of subjects	9	8	3
Age Categorical Units: participants			
< 6 years	3	4	2
>= 6 to < 12 years	5	3	1
>= 12 to < 18 years	1	1	0
Sex: Female, Male Units: participants			
Female	5	4	2
Male	4	4	1
Race (NIH/OMB) Units: Subjects			
American Indian or Alaska Native	1	1	0
Asian	0	0	0
Native Hawaiian or Other Pacific Islander	0	0	0
Black or African American	0	0	0
White	6	5	3
More than one race	0	0	0
Unknown or Not Reported	2	2	0
Ethnicity (NIH/OMB) Units: Subjects			
Hispanic or Latino	4	3	1
Not Hispanic or Latino	4	4	2
Unknown or Not Reported	1	1	0

Reporting group values	Not Assigned	Total	
Number of subjects	1	21	

Age Categorical			
Units: participants			
< 6 years	0	9	
>= 6 to < 12 years	0	9	
>= 12 to < 18 years	1	3	
Sex: Female, Male			
Units: participants			
Female	0	11	
Male	1	10	
Race (NIH/OMB)			
Units: Subjects			
American Indian or Alaska Native	1	3	
Asian	0	0	
Native Hawaiian or Other Pacific Islander	0	0	
Black or African American	0	0	
White	0	14	
More than one race	0	0	
Unknown or Not Reported	0	4	
Ethnicity (NIH/OMB)			
Units: Subjects			
Hispanic or Latino	1	9	
Not Hispanic or Latino	0	10	
Unknown or Not Reported	0	2	

## End points

### End points reporting groups

Reporting group title	0.05 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) with 0.05 x 10 <sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.	
Reporting group title	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10 <sup>6</sup> CAR+ T cells/kg post leukapheresis.	
Reporting group title	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) with 0.50 x 10 <sup>6</sup> CAR+T cells/kg post leukapheresis.	
Reporting group title	Not Assigned
Reporting group description: Participants only underwent leukapheresis.	
Reporting group title	0.05 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.05 x 10 <sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.	
Reporting group title	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10 <sup>6</sup> CAR+ T cells/kg post leukapheresis.	
Reporting group title	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.50 x 10 <sup>6</sup> CAR+T cells/kg post leukapheresis.	
Reporting group title	0.05 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with with 0.05 x 10 <sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.	
Reporting group title	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.15 x 10 <sup>6</sup> CAR+ T cells/kg post leukapheresis.	
Reporting group title	0.50 x 10 <sup>6</sup> CAR+T cells/kg
Reporting group description: Participants with CD19+ relapsed/refractory (r/r) B-cell acute lymphoblastic leukemia (B-ALL) were infused with 0.50 x 10 <sup>6</sup> CAR+T cells/kg post leukapheresis.	

### Primary: Number of Participants with Treatment Emergent Adverse Events up to 30 Days Post JCAR017 Infusion

End point title	Number of Participants with Treatment Emergent Adverse Events up to 30 Days Post JCAR017 Infusion <sup>[1][2]</sup>
End point description: An AE is defined as any noxious, unintended, or untoward medical occurrence that may appear or worsen in a participant during the course of a study. Treatment Emergent Adverse Events (TEAEs) are defined as any AE occurring or worsening on the day of the JCAR017 infusion until 30 days post-treatment (JCAR017 infusion) using National Cancer Institute (NCI) Common Terminology Criteria for Adverse Events (CTCAE) version 4.03 or greater. Safety Analysis Set included all participants who	

received conforming JCAR017 infusion.

End point type	Primary
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End point timeframe:

From first JCAR017 infusion to 30 days after JCAR017 infusion

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: Statistical Analysis Comparison was not planned as per protocol.

[2] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	7	6	1	
Units: participants	7	6	1	

## Statistical analyses

No statistical analyses for this end point

## Primary: Number of Participants with Dose Limiting Toxicities

End point title	Number of Participants with Dose Limiting Toxicities <sup>[3]</sup> <sup>[4]</sup>
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End point description:

A DLT was defined as below:

- Death not related to PD
- Grade (Gr) 4 (Life-threatening) neurotoxicity
- Gr 3 (Severe) neurotoxicity of greater than 7 days
- Gr 3 neurotoxicity does not revert to baseline within 28 days of the start date of the Grade 3 event
- Seizures of grade that do not resolve within 7 days
- Gr 4 cytokine release syndrome (CRS) that does not resolve to Grade  $\leq 3$  within 3 days
- Gr 3 CRS that does not resolve to Grade  $\leq 2$  within 7 days
- Any increase in aspartate aminotransferase (AST) or ALT  $> 3 \times$  ULN and concurrent increase in total bilirubin  $> 2 \times$  ULN that is unrelated to CRS and has no other probable reason to explain the combination of increases
- Any cardiac, dermatologic, gastrointestinal, hepatic, pulmonary, renal/genitourinary, or neurologic Gr 3 or 4 event not pre-existing or not due to the underlying malignancy
- Any other Gr 3 or 4 event deemed unexpected by the Investigator and considered a DLT upon evaluation by the safety review committee

End point type	Primary
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End point timeframe:

From first JCAR017 infusion to 28 days after JCAR017 infusion

Notes:

[3] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: Statistical Analysis Comparison was not planned as per protocol.

[4] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	5	5	1	
Units: participants	0	2	1	

## Statistical analyses

No statistical analyses for this end point

## Primary: Concentration of JCAR017 in Peripheral Blood at Day 28 Post JCAR017 Infusion

End point title	Concentration of JCAR017 in Peripheral Blood at Day 28 Post JCAR017 Infusion <sup>[5][6]</sup>
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End point description:

Concentration of JCAR017 in Peripheral Blood was assessed by droplet digital polymerase chain reaction. The PK Analysis Set include all subjects who received JCAR017 infusion and have at least one measurable JCAR017 concentration. Participants available at specific timepoints have been analyzed.

End point type	Primary
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End point timeframe:

From first JCAR017 infusion to 28 days after JCAR017 infusion

Notes:

[5] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.

Justification: Statistical Analysis Comparison was not planned as per protocol.

[6] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	3	3	1 <sup>[7]</sup>	
Units: copies per microgram				
geometric mean (geometric coefficient of variation)	1131.821 (± 64.658)	1457.591 (± 75.558)	2847.910 (± 99999)	

Notes:

[7] - Not calculable due to insufficient number of participants

## Statistical analyses

No statistical analyses for this end point

## Secondary: Number of Participants with Treatment-Emergent Adverse Events up to 90 Days Post JCAR017 Infusion

End point title	Number of Participants with Treatment-Emergent Adverse Events up to 90 Days Post JCAR017 Infusion <sup>[8]</sup>
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End point description:

An AE is defined as any noxious, unintended, or untoward medical occurrence that may appear or worsen in a participant during the course of a study. Treatment Emergent Adverse Events (TEAEs) are defined as any AE occurring or worsening on the day of the JCAR017 infusion until 90 days post-

treatment (JCAR017 infusion) using National Cancer Institute (NCI) Common Terminology Criteria for Adverse Events (CTCAE) version 4.03 or greater. The following scale for grading was used - Grade 3 = Severe, Grade 4 = Life-Threatening. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
End point timeframe:	
From first JCAR017 infusion to 90 days after JCAR017 infusion	

Notes:

[8] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period. Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	7	6	1	
Units: participants				
At least one TEAE	7	6	1	
At least one grade 3/4 TEAE	6	5	1	
At Least One TEAE Related To JCAR017	5	5	1	
At Least One grade 3/4 TEAE Related To JCAR017	3	4	1	

## Statistical analyses

No statistical analyses for this end point

## Secondary: Number of Participants with Serious Treatment-Emergent Adverse Events up to 90 Days Post JCAR017 Infusion

End point title	Number of Participants with Serious Treatment-Emergent Adverse Events up to 90 Days Post JCAR017 Infusion <sup>[9]</sup>
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End point description:

Serious adverse events (SAE) are defined as any AE which results in death; is life-threatening; requires inpatient hospitalization or prolongation of existing hospitalization; results in persistent or significant disability/incapacity; is a congenital anomaly/ birth defect; constitutes an important medical event. Treatment Emergent Adverse Events (TEAEs) are defined as any AE occurring or worsening on the day of the JCAR017 infusion until 90 days post-treatment (JCAR017 infusion) using National Cancer Institute (NCI) Common Terminology Criteria for Adverse Events (CTCAE) version 4.03 or greater. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
End point timeframe:	
From first JCAR017 infusion to 90 days after JCAR017 infusion	

Notes:

[9] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period. Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	7	6	1	
Units: participants				
AT Least one Serious TEAE	5	3	1	
At Least One Serious TEAE Related To JCAR017	1	3	1	

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline at Day 28 in Hematology Laboratory Parameters- Basophils, Eosinophils, Leukocytes, Lymphocytes, Monocytes, Neutrophils, Platelets

End point title	Change from Baseline at Day 28 in Hematology Laboratory Parameters- Basophils, Eosinophils, Leukocytes, Lymphocytes, Monocytes, Neutrophils, Platelets <sup>[10]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[10] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[11]</sup>	
Units: 10 <sup>9</sup> cells/L				
arithmetic mean (standard deviation)				
Basophils	0.00 (± 0.025)	-0.01 (± 0.023)	-0.01 (± 99999)	
Eosinophils	0.03 (± 0.048)	0.08 (± 0.114)	-0.08 (± 99999)	
Leukocytes	4.01 (± 8.338)	0.59 (± 1.753)	-2.00 (± 99999)	
Lymphocytes	-0.22 (± 0.879)	0.27 (± 1.286)	-0.57 (± 99999)	
Monocytes	0.11 (± 0.163)	0.09 (± 0.173)	-0.22 (± 99999)	
Neutrophils	0.41 (± 0.782)	-0.18 (± 0.735)	-1.50 (± 99999)	
Platelets	27.33 (± 132.672)	-68.60 (± 168.583)	-163.00 (± 99999)	

Notes:

[11] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline at Day 28 in Hematology Laboratory Parameters- Basophils/Leukocytes; Eosinophils/Leukocytes; Lymphocytes/Leukocytes; Neutrophils/Leukocytes; Monocytes/Leukocytes

End point title	Change from Baseline at Day 28 in Hematology Laboratory Parameters- Basophils/Leukocytes; Eosinophils/Leukocytes; Lymphocytes/Leukocytes; Neutrophils/Leukocytes; Monocytes/Leukocytes <sup>[12]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[12] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[13]</sup>	
Units: Ratio				
arithmetic mean (standard deviation)				
Basophils/Leukocytes	0.25 (± 0.885)	-0.24 (± 0.654)	-0.30 (± 99999)	
Eosinophils/Leukocytes	0.85 (± 1.402)	4.28 (± 5.389)	-2.90 (± 99999)	
Lymphocytes/Leukocytes	-29.57 (± 21.690)	-16.30 (± 25.140)	33.80 (± 99999)	
Neutrophils/Leukocytes	26.45 (± 26.815)	10.70 (± 18.797)	-33.90 (± 99999)	
Monocytes/Leukocytes	4.35 (± 10.123)	1.74 (± 4.515)	0.20 (± 99999)	

Notes:

[13] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline at Day 28 in Hematology Laboratory Parameters-

## Erythrocytes

End point title	Change from Baseline at Day 28 in Hematology Laboratory Parameters- Erythrocytes <sup>[14]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[14] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[15]</sup>	
Units: 10 <sup>12</sup> cells/L				
arithmetic mean (standard deviation)	0.04 (± 0.781)	0.08 (± 0.268)	-0.70 (± 99999)	

Notes:

[15] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline at Day 28 in Hematology Laboratory Parameters- Hematocrit

End point title	Change from Baseline at Day 28 in Hematology Laboratory Parameters- Hematocrit <sup>[16]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[16] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[17]</sup>	
Units: proportion of red blood cells in blood				
arithmetic mean (standard deviation)	0.00 (± 0.068)	0.02 (± 0.016)	-0.03 (±	

Notes:

[17] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline at Day 28 in Hematology Laboratory Parameters- Hemoglobin

End point title	Change from Baseline at Day 28 in Hematology Laboratory Parameters- Hemoglobin <sup>[18]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[18] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[19]</sup>	
Units: grams per liter				
arithmetic mean (standard deviation)	2.33 (± 24.312)	6.20 (± 6.834)	-13.00 (± 99999)	

Notes:

[19] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Alanine aminotransferase; Alkaline phosphatase; Aspartate aminotransferase; Lactate dehydrogenase

End point title	Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Alanine aminotransferase; Alkaline phosphatase; Aspartate aminotransferase; Lactate dehydrogenase <sup>[20]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[20] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[21]</sup>	
Units: units per liter				
arithmetic mean (standard deviation)				
Alanine aminotransferase	5.33 (± 19.439)	4.50 (± 16.299)	-19.00 (± 99999)	
Alkaline phosphatase	53.50 (± 158.322)	78.75 (± 40.950)	-121.00 (± 99999)	
Aspartate aminotransferase	29.17 (± 72.204)	11.50 (± 13.675)	-1.00 (± 99999)	
Lactate dehydrogenase	992.33 (± 2238.888)	22.25 (± 43.684)	-36.00 (± 99999)	

Notes:

[21] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

## Secondary: Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Albumin; Protein

End point title	Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Albumin; Protein <sup>[22]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[22] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	4	1 <sup>[23]</sup>	
Units: grams per liter				
arithmetic mean (standard deviation)				
Albumin	5.83 (± 9.663)	6.75 (± 2.062)	-3.00 (± 99999)	
Protein	4.00 (± 12.602)	5.00 (± 6.683)	-6.00 (± 99999)	

Notes:

[23] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Bicarbonate; Blood Urea Nitrogen; Calcium; Chloride; Glucose; Magnesium; Phosphate; Potassium; Sodium, Triglyceride

End point title	Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Bicarbonate; Blood Urea Nitrogen; Calcium; Chloride; Glucose; Magnesium; Phosphate; Potassium; Sodium, Triglyceride <sup>[24]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[24] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	4	1 <sup>[25]</sup>	
Units: mmol/L				
arithmetic mean (standard deviation)				
Bicarbonate	2.53 (± 4.453)	-2.65 (± 1.919)	4.10 (± 99999)	
Blood Urea Nitrogen	3.60 (± 3.542)	2.43 (± 0.826)	0.20 (± 99999)	
Calcium	0.13 (± 0.157)	0.04 (± 0.058)	-0.17 (± 99999)	
Chloride	-1.50 (± 4.324)	1.50 (± 1.291)	1.00 (± 99999)	
Glucose	0.32 (± 0.641)	-0.08 (± 0.472)	14.90 (± 99999)	
Magnesium	0.06 (± 0.107)	0.06 (± 0.109)	0.00 (± 99999)	
Phosphate	0.02 (± 0.281)	-0.02 (± 0.336)	-0.16 (± 99999)	
Potassium	-0.05 (± 0.672)	0.20 (± 0.535)	1.50 (± 99999)	
Sodium	1.00 (± 2.608)	2.25 (± 2.217)	2.00 (± 99999)	
Triglyceride	0.45 (± 1.652)	0.29 (± 0.320)	0.33 (± 99999)	

Notes:

[25] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Bilirubin; Creatinine; Direct Bilirubin; Urate

End point title	Change from Baseline at Day 28 in Chemistry Laboratory Parameters- Bilirubin; Creatinine; Direct Bilirubin; Urate <sup>[26]</sup>
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End point description:

Baseline is defined as the last assessment with non-missing value on or prior to the first date of the lymphodepletion chemotherapy. Safety Analysis Set included all participants who received conforming JCAR017 infusion.

End point type	Secondary
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End point timeframe:

Baseline and at Day 28

Notes:

[26] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	5	1 <sup>[27]</sup>	
Units: umol/L				
arithmetic mean (standard error)				
Bilirubin	20.26 (± 41.498)	-1.00 (± 6.928)	2.00 (± 99999)	
Creatinine	9.84 (± 14.218)	1.50 (± 9.000)	-2.00 (± 99999)	
Direct Bilirubin	13.44 (± 33.601)	-0.25 (± 0.500)	0.00 (± 99999)	
Urate	43.51 (± 94.081)	85.00 (± 71.615)	9.00 (± 99999)	

Notes:

[27] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

### Secondary: Number of Participants with Manufacturing Success of JCAR017 product

End point title	Number of Participants with Manufacturing Success of JCAR017 product
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End point description:

Successful product was defined as JCAR017 product was generated and able to be QC released (including nonconforming product) for infusion. Unsuccessful product is defined as no JCAR017 product could be generated after two manufacturing attempts using a single apheresis product for starting material or product was unable to be QC released for infusion. Pre-Treatment Set included all participants who have screened successfully into the study and underwent leukapheresis.

End point type	Secondary
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End point timeframe:

From screening to JCAR017 infusion (day -35 to day 1)

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	Not Assigned
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	9	8	3	1
Units: participants	7	7	2	1

## Statistical analyses

No statistical analyses for this end point

### Secondary: Overall Response Rate (ORR)

End point title	Overall Response Rate (ORR) <sup>[28]</sup>
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End point description:

ORR is defined as the percentage of participants who achieved either a complete response (CR) or complete response with incomplete blood recovery (CRi) on Day 28 that is confirmed on Day 56. Response assessment was performed according to the 2019 Comprehensive Cancer Network (NCCN) response criteria guidelines for pediatric acute lymphoblastic leukemia (ALL). CR is defined as absence of circulating blasts, extramedullary disease, lymphadenopathy, splenomegaly, skin/gum infiltration/testicular mass/CNS involvement, Trilineage hematopoiesis (TLH) and < 5%, Absolute neutrophil count (ANC) > 1000 per microliter, Platelets > 100,000 per microliter and no recurrence for 4 weeks. CRi is defined as meeting all criteria for CR except platelets < 100,000/μL or ANC is < 1000/μL. Efficacy Analysis Set.

End point type	Secondary
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End point timeframe:

Up to Day 56

Notes:

[28] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	4	1	
Units: percentage of participants				
number (confidence interval 95%)	50.0 (11.8 to 88.2)	25.0 (0.6 to 80.6)	100.0 (2.5 to 100.0)	

## Statistical analyses

No statistical analyses for this end point

### Secondary: Duration of Response (DOR)

End point title	Duration of Response (DOR) <sup>[29]</sup>
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**End point description:**

DOR is defined as time from first response (either CR or CRi) until progressive disease (PD), disease relapse, or death from any cause, whichever occurs first. Response assessment was performed according to the 2019 Comprehensive Cancer Network (NCCN) response criteria guidelines for pediatric ALL. CR is defined as absence of circulating blasts, extramedullary disease, lymphadenopathy, splenomegaly, skin/gum infiltration/testicular mass/CNS involvement, Trilineage hematopoiesis (TLH) and < 5%, Absolute neutrophil count (ANC) > 1000 per microliter, Platelets > 100,000 per microliter and no recurrence for 4 weeks. CRi is defined as meeting all criteria for CR except platelets < 100,000/ $\mu$ L or ANC is < 1000/ $\mu$ L. Disease progression is defined as increase of at least 25% in the absolute number of circulating or bone marrow blasts or development of extramedullary disease. Efficacy Analysis Set.

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End point type	Secondary
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**End point timeframe:**

From first response (either CR or CRi) until progressive disease (PD), disease relapse, or death from any cause, whichever occurs first (Up to approximately 14 months)

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**Notes:**

[29] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	3	1 <sup>[30]</sup>	1 <sup>[31]</sup>	
Units: months				
median (full range (min-max))	13.70 (2.46 to 13.70)	99999 (99999 to 99999)	99999 (99999 to 99999)	

**Notes:**

[30] - 99999 = Data not calculable (insufficient number of participants with events)

[31] - 99999 = Data not calculable (insufficient number of participants with events)

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**Statistical analyses**

No statistical analyses for this end point

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**Secondary: Relapse Free Survival (RFS)**

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End point title	Relapse Free Survival (RFS) <sup>[32]</sup>
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**End point description:**

RFS is defined as time from conforming JCAR017 infusion to the first progressive disease (PD), relapsed disease or death from any cause, whichever occurs first. Disease progression is defined as increase of at least 25% in the absolute number of circulating or bone marrow blasts or development of extramedullary disease. Relapsed disease is defined as Reappearance of blasts in the blood or bone marrow (> 5%) or > 1% with previous/supportive molecular findings or in any extramedullary site after a CR. CR is defined as absence of circulating blasts, extramedullary disease, lymphadenopathy, splenomegaly, skin/gum infiltration/testicular mass/CNS involvement, Trilineage hematopoiesis (TLH) and < 5%, Absolute neutrophil count (ANC) > 1000 per microliter, Platelets > 100,000 per microliter and no recurrence for 4 weeks. Efficacy Analysis Set.

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End point type	Secondary
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**End point timeframe:**

Up to approximately 15 months

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**Notes:**

[32] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	4	1 <sup>[33]</sup>	
Units: months				
median (full range (min-max))	7.77 (0.46 to 14.62)	6.98 (1.15 to 8.97)	99999 (99999 to 99999)	

Notes:

[33] - 99999 stands for Not Applicable

## Statistical analyses

No statistical analyses for this end point

## Secondary: Event Free Survival (EFS)

End point title	Event Free Survival (EFS) <sup>[34]</sup>
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End point description:

EFS is defined as time from conforming JCAR017 infusion to progressive disease (PD), relapsed disease, start of a new anticancer therapy including HSCT or death from any cause, whichever occurs first. Disease progression is defined as increase of at least 25% in the absolute number of circulating or bone marrow blasts or development of extramedullary disease. Relapsed disease is defined as Reappearance of blasts in the blood or bone marrow (> 5%) or > 1% with previous/supportive molecular findings or in any extramedullary site after a CR. CR is defined as absence of circulating blasts, extramedullary disease, lymphadenopathy, splenomegaly, skin/gum infiltration/testicular mass/CNS involvement, Trilineage hematopoiesis (TLH) and < 5%, Absolute neutrophil count (ANC) > 1000 per microliter, Platelets > 100,000 per microliter and no recurrence for 4 weeks. Only responders are included in the analysis. Censored participants were also analyzed. Efficacy Analysis Set.

End point type	Secondary
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End point timeframe:

From conforming JCAR017 infusion to PD, relapsed disease, start of a new anticancer therapy including HSCT or death from any cause, whichever occurs first (Up to approximately 15 months)

Notes:

[34] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	5	4	1	
Units: months				
median (full range (min-max))	3.24 (0.46 to 14.62)	5.42 (1.15 to 8.97)	3.12 (3.12 to 3.12)	

## Statistical analyses

No statistical analyses for this end point

## Secondary: Overall Survival (OS)

End point title	Overall Survival (OS) <sup>[35]</sup>
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**End point description:**

OS is defined as the interval from the date of first confirming JCAR017 infusion to the date of death due to any reason. Efficacy Analysis Set. The Efficacy Analysis Set included all participants who fulfill all study eligibility criteria (prospectively and retrospectively) and receive JCAR017 infusion in accordance with drug product release specifications (i.e., conforming JCAR017 product).

End point type	Secondary
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**End point timeframe:**

From the date of first confirming JCAR017 infusion to the date of death due to any reason (Up to approximately 63 months)

**Notes:**

[35] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6 <sup>[36]</sup>	4 <sup>[37]</sup>	1 <sup>[38]</sup>	
Units: months				
median (confidence interval 95%)	7.13 (0.76 to 99999)	7.08 (4.99 to 99999)	99999 (99999 to 99999)	

**Notes:**

[36] - 99999 stands for Not Applicable

[37] - 99999 stands for Not Applicable

[38] - 99999 stands for Not Applicable (Due to low number of events)

**Statistical analyses**

No statistical analyses for this end point

**Secondary: Minimal Residual Response (MRD) Negative Response Rate**

End point title	Minimal Residual Response (MRD) Negative Response Rate <sup>[39]</sup>
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**End point description:**

Minimal Residual Disease (MRD) Negative Response Rate is defined as the percentage of participants achieving either a CR or CRi with a MRD negative bone marrow on Day 28, confirmed on Day 56. CR is defined as absence of circulating blasts, extramedullary disease, lymphadenopathy, splenomegaly, skin/gum infiltration/testicular mass/CNS involvement, Trilineage hematopoiesis (TLH) and < 5%, Absolute neutrophil count (ANC) > 1000 per microliter, Platelets > 100,000 per microliter and no recurrence for 4 weeks. CRi is defined as meeting all criteria for CR except platelets < 100,000/μL or ANC is < 1000/μL. Disease progression is defined as increase of at least 25% in the absolute number of circulating or bone marrow blasts or development of extramedullary disease. Disease assessments recorded on or after start of a new anticancer therapy, including HSCT, will not be considered, nor will disease assessments reported after a PD or relapse has been observed. Efficacy Analysis Set.

End point type	Secondary
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**End point timeframe:**

Up to Day 56

**Notes:**

[39] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	6	4	1	
Units: percentage of participants				
number (confidence interval 95%)	16.7 (0.4 to 64.1)	25.0 (0.6 to 80.6)	100.0 (2.5 to 100.0)	

## Statistical analyses

No statistical analyses for this end point

## Secondary: Number of Participants who Achieved a Response after JCAR017 Infusion and then Proceeded to Hematopoietic Stem Cell Transplant

End point title	Number of Participants who Achieved a Response after JCAR017 Infusion and then Proceeded to Hematopoietic Stem Cell Transplant <sup>[40]</sup>
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End point description:

Number of participants who undergo HSCT after receiving a JCAR017 infusion and achieving a response are presented. The time of proceeding to HSCT is defined as the time of commencing the conditioning regimen as required for HSCT. CR is defined as absence of circulating blasts, extramedullary disease, lymphadenopathy, splenomegaly, skin/gum infiltration/testicular mass/CNS involvement, Trilineage hematopoiesis (TLH) and < 5%, Absolute neutrophil count (ANC) > 1000 per microliter, Platelets > 100,000 per microliter and no recurrence for 4 weeks. CRi is defined as meeting all criteria for CR except platelets < 100,000/μL or ANC is < 1000/μL. Disease progression is defined as increase of at least 25% in the absolute number of circulating or bone marrow blasts or development of extramedullary disease.

End point type	Secondary
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End point timeframe:

Up to approximately 24 months

Notes:

[40] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.

Justification: Statistical Analysis Comparison was not planned as per protocol.

End point values	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	7	6	1	
Units: participants	1	1	1	

## Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

All-cause mortality was collected from JCAR017 infusion until their study completion (up to approximately 63 months). Serious and non-serious adverse events were collected from first JCAR017 infusion to 90 days post JCAR017 infusion

Adverse event reporting additional description:

All-cause mortality was collected for all participants that underwent leukapheresis and were assigned to dose level. Serious and non-serious adverse events were collected for all participants who received conforming JCAR017 infusion.

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	23.1
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### Reporting groups

Reporting group title	0.05 x 10 <sup>6</sup> CAR+ T cells/kg
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Reporting group description:

Participants with Relapsed and Refractory Multiple Myeloma were infused with 0.05 x 10<sup>6</sup> CAR+ T cells per kilogram (kg) post leukapheresis.

Reporting group title	0.50 x 10 <sup>6</sup> CAR+T cells/kg
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Reporting group description:

Participants with RRMM were infused with 0.50 x 10<sup>6</sup> CAR+T cells/kg post leukapheresis.

Reporting group title	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
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Reporting group description:

Participants with RRMM were infused with 0.15 x 10<sup>6</sup> CAR+ T cells/kg post leukapheresis.

Serious adverse events	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Total subjects affected by serious adverse events			
subjects affected / exposed	5 / 7 (71.43%)	1 / 1 (100.00%)	3 / 6 (50.00%)
number of deaths (all causes)	4	1	3
number of deaths resulting from adverse events	0	0	0
Injury, poisoning and procedural complications			
Extradural haematoma			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infusion related reaction			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			

Neurotoxicity			
subjects affected / exposed	1 / 7 (14.29%)	1 / 1 (100.00%)	1 / 6 (16.67%)
occurrences causally related to treatment / all	1 / 1	1 / 1	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Brain oedema			
subjects affected / exposed	0 / 7 (0.00%)	1 / 1 (100.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 0	1 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Immune system disorders			
Cytokine release syndrome			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	3 / 6 (50.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	3 / 3
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Infections and infestations			
Sepsis			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumonia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Viraemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	0.05 x 10 <sup>6</sup> CAR+ T cells/kg	0.50 x 10 <sup>6</sup> CAR+T cells/kg	0.15 x 10 <sup>6</sup> CAR+ T cells/kg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 7 (100.00%)	1 / 1 (100.00%)	6 / 6 (100.00%)
Vascular disorders			

Cyanosis subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Hypertension subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	1 / 1 (100.00%) 1	0 / 6 (0.00%) 0
General disorders and administration site conditions Pyrexia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 3	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Immune system disorders Cytokine release syndrome subjects affected / exposed occurrences (all)	4 / 7 (57.14%) 4	1 / 1 (100.00%) 1	1 / 6 (16.67%) 1
Haemophagocytic lymphohistiocytosis subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Hypogammaglobulinaemia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	1 / 1 (100.00%) 1	1 / 6 (16.67%) 1
Respiratory, thoracic and mediastinal disorders Pleural effusion subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Cough subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Psychiatric disorders Insomnia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Investigations Alanine aminotransferase increased subjects affected / exposed occurrences (all)	4 / 7 (57.14%) 4	1 / 1 (100.00%) 1	0 / 6 (0.00%) 0
Aspartate aminotransferase increased			

subjects affected / exposed	4 / 7 (57.14%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	12	0	0
Blood fibrinogen decreased			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Gamma-glutamyltransferase increased			
subjects affected / exposed	4 / 7 (57.14%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	5	0	0
Interleukin level increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
International normalised ratio increased			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Oxygen saturation decreased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	2 / 6 (33.33%)
occurrences (all)	0	0	2
Serum ferritin increased			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Blood uric acid increased			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Blood triglycerides increased			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Bilirubin conjugated increased			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Blood bilirubin increased			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	3	0	0
Blood creatinine increased			

subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 5	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Injury, poisoning and procedural complications Refractoriness to platelet transfusion subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Cardiac disorders Tachycardia subjects affected / exposed occurrences (all)  Bradycardia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0  0 / 7 (0.00%) 0	1 / 1 (100.00%) 1  1 / 1 (100.00%) 1	0 / 6 (0.00%) 0  1 / 6 (16.67%) 2
Nervous system disorders Dizziness subjects affected / exposed occurrences (all)  Hemiplegia subjects affected / exposed occurrences (all)  Neurotoxicity subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0  1 / 7 (14.29%) 1  0 / 7 (0.00%) 0	0 / 1 (0.00%) 0  0 / 1 (0.00%) 0  1 / 1 (100.00%) 1	1 / 6 (16.67%) 1  0 / 6 (0.00%) 0  1 / 6 (16.67%) 1
Blood and lymphatic system disorders Anaemia subjects affected / exposed occurrences (all)  Thrombocytopenia subjects affected / exposed occurrences (all)  Neutropenia subjects affected / exposed occurrences (all)  Leukopenia subjects affected / exposed occurrences (all)	5 / 7 (71.43%) 10  4 / 7 (57.14%) 4  3 / 7 (42.86%) 9  4 / 7 (57.14%) 6	1 / 1 (100.00%) 2  1 / 1 (100.00%) 2  1 / 1 (100.00%) 2  1 / 1 (100.00%) 2	5 / 6 (83.33%) 14  3 / 6 (50.00%) 6  2 / 6 (33.33%) 2  1 / 6 (16.67%) 1

Febrile neutropenia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Coagulopathy subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 3	0 / 6 (0.00%) 0
Ear and labyrinth disorders Ear pain subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Gastrointestinal disorders Colitis subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Anal fissure subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Anaesthesia oral subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Abdominal pain subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Constipation subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Diarrhoea subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 2	1 / 6 (16.67%) 1
Intestinal haemorrhage subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Lip dry subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Nausea			

subjects affected / exposed	2 / 7 (28.57%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	2	0	3
Oral mucosal erythema			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Vomiting			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	3 / 6 (50.00%)
occurrences (all)	1	0	4
Hepatobiliary disorders			
Hyperbilirubinaemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Cholecystitis			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Skin and subcutaneous tissue disorders			
Skin erosion			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Dermatitis atopic			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Erythema			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Night sweats			
subjects affected / exposed	0 / 7 (0.00%)	1 / 1 (100.00%)	0 / 6 (0.00%)
occurrences (all)	0	1	0
Rash			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Rash maculo-papular			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Skin exfoliation			

subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Renal and urinary disorders Haematuria subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Musculoskeletal and connective tissue disorders Myalgia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 1	0 / 6 (0.00%) 0
Infections and infestations Clostridium difficile infection subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 2	0 / 6 (0.00%) 0
Bacteraemia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1
Staphylococcal bacteraemia subjects affected / exposed occurrences (all)	0 / 7 (0.00%) 0	1 / 1 (100.00%) 1	0 / 6 (0.00%) 0
Cytomegalovirus infection subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Paronychia subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Pneumonia fungal subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Upper respiratory tract infection subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	0 / 6 (0.00%) 0
Metabolism and nutrition disorders Decreased appetite subjects affected / exposed occurrences (all)	1 / 7 (14.29%) 1	0 / 1 (0.00%) 0	1 / 6 (16.67%) 1

Fluid retention			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Hyperamylasaemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Hyperlipidaemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Hypernatraemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Hyperphosphataemia			
subjects affected / exposed	1 / 7 (14.29%)	1 / 1 (100.00%)	0 / 6 (0.00%)
occurrences (all)	1	1	0
Hypertriglyceridaemia			
subjects affected / exposed	3 / 7 (42.86%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	3	0	0
Hypoglycaemia			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Hypophosphataemia			
subjects affected / exposed	1 / 7 (14.29%)	1 / 1 (100.00%)	0 / 6 (0.00%)
occurrences (all)	1	2	0
Tumour lysis syndrome			
subjects affected / exposed	0 / 7 (0.00%)	0 / 1 (0.00%)	1 / 6 (16.67%)
occurrences (all)	0	0	1
Hypoalbuminaemia			
subjects affected / exposed	2 / 7 (28.57%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	3	0	0
Hypermagnesaemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	1	0	0
Hypomagnesaemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	2	0	0

Hypokalaemia			
subjects affected / exposed	1 / 7 (14.29%)	0 / 1 (0.00%)	0 / 6 (0.00%)
occurrences (all)	3	0	0

## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
07 January 2019	PA01 was implemented to amend inclusion and exclusion criteria following an Urgent Safety Measure on another liso-cel clinical trial.
10 September 2019	PA 02 was implemented to amend the initial starting dose of liso-cel and Phase 1 study design.

Notes:

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### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

Limitations of the trial such as small numbers of subjects analysed or technical problems leading to unreliable data.

Study was terminated early on the grounds that liso-cel did not represent a significant therapeutic benefit over existing therapies for the treatment of pediatric B-cell malignancies.

Notes: