Clinical trial results:
Phase 3, Randomized, Double-Blind, Placebo-Controlled Trial, with Cross-Over, of Posoleucel (ALVR105) for the Treatment of Adenovirus Infection in Pediatric and Adult Participants Receiving Standard of Care Following Allogeneic Hematopoietic Cell Transplantation

Summary

EudraCT number	2021-003450-22
Trial protocol	SE IT ES
Global end of trial date	31 January 2024

Results information

Result version number	v1 (current)
This version publication date	01 May 2024
First version publication date	01 May 2024

Trial information

Trial identification

Sponsor protocol code	P-105-303

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT05179057
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	AlloVir, Inc.
Sponsor organisation address	1100 Winter Street, Waltham, United States, MA 02451
Public contact	Clinical Trials Information Line, AlloVir, Inc., +1 617-433-2605, ClinicalTrials@allovir.com
Scientific contact	Clinical Trials Information Line, AlloVir, Inc., +1 617-433-2605, ClinicalTrials@allovir.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	No
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	31 January 2024
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	31 January 2024
Was the trial ended prematurely?	Yes

Notes:

General information about the trial

Main objective of the trial:

The main objectives of the study were:
- To compare the percent of participants who have clearance of adenovirus viremia at Day 29 in participants receiving posoleucel and standard of care (SoC) to that in participants receiving placebo and SoC.
- To determine the safety and tolerability of posoleucel by analyzing the incidence and severity of treatment-emergent adverse events (TEAEs), including individual adverse events of special interest (AESIs).

Protection of trial subjects:

This study was performed in compliance with the principles of Good Clinical Practice, including the archiving of essential documents.

Background therapy:

Participants in both treatment arms continued to receive SoC per their treating physician.

Evidence for comparator: -

Actual start date of recruitment	26 April 2022
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Canada: 6
Country: Number of subjects enrolled	United Kingdom: 21
Country: Number of subjects enrolled	United States: 17
Country: Number of subjects enrolled	Italy: 9
Country: Number of subjects enrolled	Spain: 3
Country: Number of subjects enrolled	Sweden: 1
Worldwide total number of subjects	57
EEA total number of subjects	13

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)	6
Children (2-11 years)	28
Adolescents (12-17 years)	13
Adults (18-64 years)	8
From 65 to 84 years	2
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants were enrolled at 47 study centers in the United States, Canada, Italy, Spain, Sweden, and the United Kingdom, and participated from April 2022 to January 2024.

Pre-assignment

Screening details:

Participants with adenovirus infection receiving standard of care following allogeneic hematopoietic stem cell transplant (allo-HCT) were randomized in a 1:1 ratio to receive either posoleucel or placebo. Randomization was stratified by level of viremia (≥10,000 copies/mL or <10,000 copies/mL adenovirus DNA) and age (≥12 years or <12 years).

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Investigator, Subject

Arms

Are arms mutually exclusive?	Yes
Arm title	Posoleucel, Then Placebo

Arm description:

Participants were randomized to receive 2 sequential infusions of posoleucel, separated by 14 ± 3 days. The Primary Study Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Eligible participants who experienced progression to active target organ disease or progression of existing target organ disease could cross-over to placebo treatment between Day 29 and Week 10. In the Cross-Over Period, participants received 2 sequential infusions of placebo, separated by 14 ± 3 days. The Cross-over Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Arm type	Experimental
Investigational medicinal product name	Posoleucel
Investigational medicinal product code	ALVR105
Other name	PSL, ALVR-105, Viralym-M
Pharmaceutical forms	Dispersion for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administered as 2-4 milliliter infusion, visually identical to placebo.

Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Dispersion for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administered as 2-4 milliliter infusion, visually identical to Posoleucel.

Arm title	Placebo, Then Posoleucel

Arm description:

Participants were randomized to receive 2 sequential infusions of placebo, separated by 14 ± 3 days. The Primary Study Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Eligible participants who experienced progression to active target organ disease or progression of existing target organ disease could cross-over to posoleucel treatment between Day 29 and Week 10. In the Cross- Over Period, participants received 2 sequential infusions of placebo, separated by 14 ± 3

days. The Cross-over Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Arm type	Experimental
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Investigational medicinal product name	Placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Dispersion for infusion
Routes of administration	Intravenous use

Dosage and administration details:

Administered as 2-4 milliliter infusion, visually identical to Posoleucel.

Number of subjects in period 1	Posoleucel, Then Placebo	Placebo, Then Posoleucel
Started	30	27
Received Primary Study Treatment	28	23
Met Crossover Eligibility Criteria	4 [1]	5 [2]
Received Cross-over Treatment	4 [3]	5 [4]
Completed	10	13
Not completed	20	14
Study terminated	13	7
Discontinuation or withdrawal	2	2
Adverse event, non-fatal	3	2
Never received study treatment	1	3
Noncompliance with protocol	1	-

Notes:

[1] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: The milestone represents participants who met the cross-over eligibility criteria.

[2] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: The milestone represents participants who received any cross-over treatment.

[3] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: The milestone represents participants who received any cross-over treatment.

[4] - The number of subjects at this milestone seems inconsistent with the number of subjects in the arm. It is expected that the number of subjects will be greater than, or equal to the number that completed, minus those who left.

Justification: The milestone represents participants who met the cross-over eligibility criteria.

Baseline characteristics

Reporting groups	
Reporting group title	Posoleucel, Then Placebo

Reporting group description:

Participants were randomized to receive 2 sequential infusions of posoleucel, separated by 14 ± 3 days. The Primary Study Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Eligible participants who experienced progression to active target organ disease or progression of existing target organ disease could cross-over to placebo treatment between Day 29 and Week 10. In the Cross-Over Period, participants received 2 sequential infusions of placebo, separated by 14 ± 3 days. The Cross-over Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Reporting group title	Placebo, Then Posoleucel

Reporting group description:

Participants were randomized to receive 2 sequential infusions of placebo, separated by 14 ± 3 days. The Primary Study Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Eligible participants who experienced progression to active target organ disease or progression of existing target organ disease could cross-over to posoleucel treatment between Day 29 and Week 10. In the Cross- Over Period, participants received 2 sequential infusions of placebo, separated by 14 ± 3 days. The Cross-over Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Reporting group values	Posoleucel, Then Placebo	Placebo, Then Posoleucel	Total
Number of subjects	30	27	57
Age categorical Units: Subjects			
Age continuous Units: years			
arithmetic mean	16.2	14.9	
standard deviation	± 17.10	± 15.80	-
Gender categorical Units: Subjects			
Female	9	11	20
Male	21	16	37
Ethnicity Units: Subjects			
Hispanic or Latino	5	2	7
Not Hispanic or Latino	22	23	45
Unknown or Not Reported	3	2	5
Race Units: Subjects			
Asian	3	2	5
Black or African American	3	1	4
White	19	20	39
Not Reported	3	1	4
Other	2	3	5

End points

End points reporting groups

Reporting group title	Posoleucel, Then Placebo

Reporting group description:

Participants were randomized to receive 2 sequential infusions of posoleucel, separated by 14 ± 3 days. The Primary Study Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Eligible participants who experienced progression to active target organ disease or progression of existing target organ disease could cross-over to placebo treatment between Day 29 and Week 10. In the Cross-Over Period, participants received 2 sequential infusions of placebo, separated by 14 ± 3 days. The Cross-over Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Reporting group title	Placebo, Then Posoleucel

Reporting group description:

Participants were randomized to receive 2 sequential infusions of placebo, separated by 14 ± 3 days. The Primary Study Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Eligible participants who experienced progression to active target organ disease or progression of existing target organ disease could cross-over to posoleucel treatment between Day 29 and Week 10. In the Cross- Over Period, participants received 2 sequential infusions of placebo, separated by 14 ± 3 days. The Cross-over Period included a 4-week efficacy evaluation followed by a 20-week safety follow-up.

Subject analysis set title	Posoleucel (Primary Study Period)
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants were randomized to receive 2 sequential infusions of posoleucel, separated by 14 ± 3 days, during the Primary Study Period.

Subject analysis set title	Placebo (Primary Study Period)
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants were randomized to receive 2 sequential infusions of placebo, separated by 14 ± 3 days, during the Primary Study Period.

Subject analysis set title	Posoleucel (Cross-over Period)
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants were randomized to receive 2 sequential infusions of placebo in the Primary Study Period and were eligible to cross-over between Day 29 and Week 10. Participants received posoleucel during the Crossover Period.

Subject analysis set title	Placebo (Cross-over Period)
Subject analysis set type	Safety analysis

Subject analysis set description:

Participants were randomized to receive 2 sequential infusions of posoleucel in the Primary Study Period and were eligible to cross-over between Day 29 and Week 10. Participants received placebo during the Cross-over Period.

Primary: Number of Participants With Undetectable Adenovirus Infection

End point title	Number of Participants With Undetectable Adenovirus Infection

End point description:

Viral load of adenovirus was measured at the central laboratory using quantitative polymerase chain reaction (qPCR) from blood and stool samples at each study visit and on Day 29 from a nasopharyngeal swab. There was a 14-day window for participants who crossed over from posoleucel to placebo; and for participants who crossed over from placebo to posoleucel, the pre-dose cross-over Day 1 viral load was used. Participants missing the primary endpoint but having undetectable viremia before Day 29 and after Day 43 were imputed as successes. Undetectable adenovirus viremia was less than the lower limit of quantification (LLOQ).

The modified intent-to-treat (mITT) population included all randomized participants who received at

least one dose of posoleucel or placebo. Only participants in the mITT population who completed through Day 29 or discontinued early were included.

End point type	Primary

End point timeframe:

Day 29 through Day 43 (Day 29 + 14 days; up to 43 days post-first infusion)

End point values	Posoleucel, Then Placebo	Placebo, Then Posoleucel		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	25	21		
Units: participants	11	9		

Statistical analyses

Statistical analysis title	Posoleucel versus Placebo

Statistical analysis description:

A logistic regression model included treatment, level of viremia at baseline (≥10,000 copies/mL or <10,000 copies/mL adenovirus DNA), age (≥12 years or <12 years), and absolute lymphocyte counts at baseline. The null hypothesis is that the true percentage for posoleucel plus SoC is less than or equal to the true percentage for placebo plus SoC, and the alternative hypothesis is that it is greater.

Comparison groups	Posoleucel, Then Placebo v Placebo, Then Posoleucel
Number of subjects included in analysis	46
Analysis specification	Pre-specified
Analysis type	superiority
Parameter estimate	Odds ratio (OR)
Point estimate	0.98
Confidence interval	
level	95 %
sides	2-sided
lower limit	0.26
upper limit	3.69

Primary: Number of Participants Who Experienced TEAEs

End point title	Number of Participants Who Experienced TEAEs[1]

End point description:

A TEAE was defined as an adverse event (AE) with a start date and time on or after the first dose of study treatment. A serious AE (SAE) was an AE that met at least one of the following serious criteria: fatal, life-threatening, required inpatient hospitalization or prolongation of existing hospitalization; resulted in persistent or significant disability/ incapacity; was a congenital anomaly/birth defect; or other important medical event. Treatment-emergent AESI included acute or chronic graft versus host disease, cytokine release syndrome, infusion-related reactions, and graft failure or rejection. Treatment-related refers to the assessment of a relationship between study treatment and the event by the investigator.

The safety population included all participants who received any amount of posoleucel or placebo and had at least one post-treatment safety assessment.

End point type	Primary

End point timeframe:

Up to 34 weeks

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: This was a pre-specified endpoint in which descriptive statistics were planned, with no hypothesis testing.

End point values	Posoleucel (Primary Study Period)	Placebo (Primary Study Period)	Posoleucel (Cross-over Period)	Placebo (Cross-over Period)
Subject group type	Subject analysis set	Subject analysis set	Subject analysis set	Subject analysis set
Number of subjects analysed	28	23	5	4
Units: participants				
Any TEAE	27	23	5	3
Any TEAE related to study treatment	7	9	1	1
Any AESI	6	9	1	2
Any SAE	16	16	3	1
Any SAE related to study treatment	1	1	1	1
TEAE leading to study treatment discontinuation	2	1	0	2
Any TEAE leading to study discontinuation	1	2	0	1
Any TEAE leading to death	2	1	0	1

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants With Overall Disease Progression

End point title	Number of Participants With Overall Disease Progression

End point description:

Data not collected due to early termination after Data and Safety Monitoring Board (DSMB) futility analysis concluded the study was unlikely to meet its primary endpoint.

End point type	Secondary

End point timeframe:

From Day 29 up to Week 10

End point values	Posoleucel, Then Placebo	Placebo, Then Posoleucel		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0[2]	0[3]		
Units: participants				

Notes:

[2] - Data not collected due to early termination after DSMB futility analysis.

[3] - Data not collected due to early termination after DSMB futility analysis.

Statistical analyses

No statistical analyses for this end point

Secondary: Area Under the Curve (AUC) Adenovirus Viral Load

End point title	Area Under the Curve (AUC) Adenovirus Viral Load

End point description:

Data not collected due to early termination after DSMB futility analysis concluded the study was unlikely to meet its primary endpoint.

End point type	Secondary

End point timeframe:

Pre-dose and Day 29

End point values	Posoleucel, Then Placebo	Placebo, Then Posoleucel		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0[4]	0[5]		
Units: copies/mL*h				
arithmetic mean (standard deviation)	()	()		

Notes:

[4] - Data not collected due to early termination after DSMB futility analysis.

[5] - Data not collected due to early termination after DSMB futility analysis.

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants Who Achieved Adenovirus Viremia <400 Copies/mL at Day 29

End point title	Number of Participants Who Achieved Adenovirus Viremia <400 Copies/mL at Day 29

End point description:

Data not collected due to early termination after DSMB futility analysis concluded the study was unlikely to meet its primary endpoint.

End point type	Secondary

End point timeframe:

Day 29

End point values	Posoleucel, Then Placebo	Placebo, Then Posoleucel		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0[6]	0[7]		
Units: participants				

Notes:

[6] - Data not collected due to early termination after DSMB futility analysis.

[7] - Data not collected due to early termination after DSMB futility analysis.

Statistical analyses

No statistical analyses for this end point

Secondary: Time to Undetectable Adenovirus Viremia (Less Than LLOQ)

End point title	Time to Undetectable Adenovirus Viremia (Less Than LLOQ)

End point description:

Data not collected due to early termination after DSMB futility analysis concluded the study was unlikely to meet its primary endpoint.

End point type	Secondary

End point timeframe:

Pre-dose to 34 weeks

End point values	Posoleucel, Then Placebo	Placebo, Then Posoleucel		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0[8]	0[9]		
Units: hours				
median (full range (min-max))	(to)	(to)		

Notes:

[8] - Data not collected due to early termination after DSMB futility analysis.

[9] - Data not collected due to early termination after DSMB futility analysis.

Statistical analyses

No statistical analyses for this end point

Secondary: Number of Participants With Adenovirus Disease Recurrence

End point title	Number of Participants With Adenovirus Disease Recurrence

End point description:

Data not collected due to early termination after DSMB futility analysis concluded the study was unlikely to meet its primary endpoint.

End point type	Secondary

End point timeframe:

34 weeks

End point values	Posoleucel, Then Placebo	Placebo, Then Posoleucel		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	0[10]	0[11]		
Units: participants				

Notes:

[10] - Data not collected due to early termination after DSMB futility analysis.

[11] - Data not collected due to early termination after DSMB futility analysis.

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: Up to 34 weeks	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	25.1

Reporting groups	
Reporting group title	Posoleucel (Primary Study Period)
Reporting group description: Participants were randomized to receive 2 sequential infusions of posoleucel, separated by 14 ± 3 days, during the Primary Study Period.	
Reporting group title	Placebo (Primary Study Period)
Reporting group description: Participants were randomized to receive 2 sequential infusions of placebo, separated by 14 ± 3 days, during the Primary Study Period.	
Reporting group title	Posoleucel (Cross-over Period)
Reporting group description: Participants were randomized to receive 2 sequential infusions of placebo in the Primary Study Period and were eligible to cross-over between Day 29 and Week 10. Participants received posoleucel during the Crossover Period.	
Reporting group title	Placebo (Cross-over Period)
Reporting group description: Participants were randomized to receive 2 sequential infusions of posoleucel in the Primary Study Period and were eligible to cross-over between Day 29 and Week 10. Participants received placebo during the Cross-over Period.	

Serious adverse events	Posoleucel (Primary Study Period)	Placebo (Primary Study Period)	Posoleucel (Cross-over Period)
Total subjects affected by serious adverse events			
subjects affected / exposed	16 / 28 (57.14%)	16 / 23 (69.57%)	3 / 5 (60.00%)
number of deaths (all causes)	2	1	0
number of deaths resulting from adverse events			
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Leukaemia recurrent			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Vascular disorders			
Capillary leak syndrome			

subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Disease progression			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 1	0 / 0
Influenza like illness			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Pyrexia			
subjects affected / exposed	4 / 28 (14.29%)	3 / 23 (13.04%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 6	1 / 4	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Immune system disorders			
Acute graft versus host disease			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Acute graft versus host disease in intestine			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Acute graft versus host disease in skin			

subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (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
Chronic graft versus host disease			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Chronic graft versus host disease in intestine			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 3	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Chronic graft versus host disease in lung			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Chronic graft versus host disease oral			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Graft versus host disease			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	1 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Graft versus host disease in gastrointestinal tract			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	1 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Haemophagocytic lymphohistiocytosis			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Respiratory, thoracic and mediastinal disorders			
Deep vein thrombosis			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Hypoxia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	3 / 3	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pharyngeal inflammation			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pleural effusion			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumomediastinum			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumothorax			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Investigations			
Weight decreased			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Injury, poisoning and procedural complications			
Lower limb fracture			

subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nervous system disorders			
Cerebral haemorrhage			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Seizure			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Blood and lymphatic system disorders			
Autoimmune haemolytic anaemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Haemolytic anaemia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Thrombotic microangiopathy			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Immune-mediated cytopenia			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Ascites			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Diarrhoea			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal haemorrhage			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Nausea			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Pancreatitis			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	1 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hepatobiliary disorders			
Acute hepatic failure			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 1	0 / 0	0 / 0
Hepatic failure			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Renal and urinary disorders			
Chronic kidney disease			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Haematuria			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Nephrolithiasis			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Renal tubular disorder			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Infections and infestations			
Bacteraemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 2
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
COVID-19			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 0	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Candida infection			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Clostridium difficile colitis			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Clostridium difficile infection			
subjects affected / exposed	0 / 28 (0.00%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cryptosporidiosis infection			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cytomegalovirus colitis			

subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Cytomegalovirus infection reactivation			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Device related infection			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Enterocolitis infectious			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Escherichia pyelonephritis			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 2	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 1	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastroenteritis rotavirus			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Human herpesvirus 6 infection			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Localised infection			

subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Pancreatitis viral			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Parainfluenzae virus infection			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Parotitis			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Phlebitis infective			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Pneumococcal bacteraemia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 2	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Respiratory syncytial virus infection			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sepsis			

subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Sinusitis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Metabolism and nutrition disorders			
Electrolyte imbalance			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Hypokalaemia			
subjects affected / exposed	0 / 28 (0.00%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences causally related to treatment / all	0 / 0	0 / 1	0 / 0
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Hypophosphataemia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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
Metabolic acidosis			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (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

Serious adverse events	Placebo (Cross-over Period)		
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 4 (25.00%)		
number of deaths (all causes)	1		
number of deaths resulting from adverse events			
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Leukaemia recurrent			

subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Vascular disorders			
Capillary leak syndrome			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Disease progression			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Influenza like illness			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pyrexia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Immune system disorders			
Acute graft versus host disease			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Acute graft versus host disease in intestine			

subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Acute graft versus host disease in skin				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Chronic graft versus host disease				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Chronic graft versus host disease in intestine				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Chronic graft versus host disease in lung				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Chronic graft versus host disease oral				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Graft versus host disease				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Graft versus host disease in gastrointestinal tract				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			

Haemophagocytic lymphohistiocytosis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Respiratory, thoracic and mediastinal disorders				
Deep vein thrombosis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Hypoxia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pharyngeal inflammation				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pleural effusion				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pneumomediastinum				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pneumothorax				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Investigations				
Weight decreased				

subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Lower limb fracture			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nervous system disorders			
Cerebral haemorrhage			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 1		
Seizure			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Autoimmune haemolytic anaemia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Haemolytic anaemia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Thrombotic microangiopathy			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Immune-mediated cytopenia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		

Gastrointestinal disorders			
Ascites			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Diarrhoea			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal haemorrhage			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Nausea			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Pancreatitis			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Acute hepatic failure			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences causally related to treatment / all	0 / 0		
deaths causally related to treatment / all	0 / 0		
Hepatic failure			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Chronic kidney disease			

subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Haematuria				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Nephrolithiasis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Renal tubular disorder				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Infections and infestations				
Bacteraemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
COVID-19				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Candida infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Clostridium difficile colitis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Clostridium difficile infection				

subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Cryptosporidiosis infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Cytomegalovirus colitis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Cytomegalovirus infection reactivation				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Device related infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Enterocolitis infectious				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Escherichia pyelonephritis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Gastroenteritis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Gastroenteritis rotavirus				

subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Human herpesvirus 6 infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Localised infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pancreatitis viral				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Parainfluenzae virus infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Parotitis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Phlebitis infective				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pneumococcal bacteraemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Pneumonia				

subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Respiratory syncytial virus infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Sepsis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Sinusitis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Metabolism and nutrition disorders				
Electrolyte imbalance				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Hypokalaemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Hypophosphataemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			
Metabolic acidosis				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences causally related to treatment / all	0 / 0			
deaths causally related to treatment / all	0 / 0			

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

Non-serious adverse events	Posoleucel (Primary Study Period)	Placebo (Primary Study Period)	Posoleucel (Cross-over Period)
Total subjects affected by non-serious adverse events			
subjects affected / exposed	27 / 28 (96.43%)	23 / 23 (100.00%)	5 / 5 (100.00%)
Vascular disorders			
Hypertension			
subjects affected / exposed	4 / 28 (14.29%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	4	1	0
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences (all)	1	1	1
Gait disturbance			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Pyrexia			
subjects affected / exposed	10 / 28 (35.71%)	8 / 23 (34.78%)	1 / 5 (20.00%)
occurrences (all)	10	8	1
Immune system disorders			
Acute graft versus host disease in intestine			
subjects affected / exposed	1 / 28 (3.57%)	3 / 23 (13.04%)	0 / 5 (0.00%)
occurrences (all)	1	3	0
Acute graft versus host disease in skin			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Cytokine release syndrome			
subjects affected / exposed	1 / 28 (3.57%)	3 / 23 (13.04%)	0 / 5 (0.00%)
occurrences (all)	1	3	0
Graft versus host disease			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Graft versus host disease in liver			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0

Hypogammaglobulinaemia			
subjects affected / exposed	3 / 28 (10.71%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	3	0	0
Reproductive system and breast disorders			
Pelvic pain			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	1	0	1
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	3 / 28 (10.71%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	3	2	0
Dyspnoea			
subjects affected / exposed	3 / 28 (10.71%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	3	0	0
Epistaxis			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences (all)	2	1	1
Hypoxia			
subjects affected / exposed	2 / 28 (7.14%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	2	2	0
Pleural effusion			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	1	0	0
Tachypnoea			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Investigations			
Activated partial thromboplastin time prolonged			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Alanine aminotransferase increased			
subjects affected / exposed	2 / 28 (7.14%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	2	2	0
Blood bicarbonate decreased			

subjects affected / exposed	1 / 28 (3.57%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	1	2	0
Blood bilirubin increased			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Blood creatinine increased			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences (all)	2	1	1
Enterobacter test positive			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Gamma-glutamyltransferase increased			
subjects affected / exposed	1 / 28 (3.57%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	1	2	0
Lipase increased			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Neutrophil count decreased			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Platelet count decreased			
subjects affected / exposed	2 / 28 (7.14%)	3 / 23 (13.04%)	1 / 5 (20.00%)
occurrences (all)	2	3	1
Weight decreased			
subjects affected / exposed	2 / 28 (7.14%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	2	2	0
Injury, poisoning and procedural complications			
Traumatic haematoma			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Congenital, familial and genetic disorders			
Phimosis			
subjects affected / exposed	4 / 28 (14.29%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	4	2	0
Cardiac disorders			

Pericardial effusion			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Sinus tachycardia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	1	0	0
Nervous system disorders			
Somnolence			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	6 / 28 (21.43%)	5 / 23 (21.74%)	2 / 5 (40.00%)
occurrences (all)	6	5	2
Febrile neutropenia			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Thrombocytopenia			
subjects affected / exposed	3 / 28 (10.71%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	3	1	0
Eye disorders			
Orbital haematoma			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Vision blurred			
subjects affected / exposed	0 / 28 (0.00%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	0	2	0
Gastrointestinal disorders			
Abdominal distension			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Abdominal pain			
subjects affected / exposed	6 / 28 (21.43%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	6	2	0
Ascites			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0

Constipation			
subjects affected / exposed	3 / 28 (10.71%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	3	2	0
Diarrhoea			
subjects affected / exposed	5 / 28 (17.86%)	4 / 23 (17.39%)	1 / 5 (20.00%)
occurrences (all)	5	4	1
Gastritis			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Gastrointestinal haemorrhage			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Nausea			
subjects affected / exposed	6 / 28 (21.43%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	6	1	0
Stomatitis			
subjects affected / exposed	0 / 28 (0.00%)	3 / 23 (13.04%)	0 / 5 (0.00%)
occurrences (all)	0	3	0
Vomiting			
subjects affected / exposed	8 / 28 (28.57%)	4 / 23 (17.39%)	0 / 5 (0.00%)
occurrences (all)	8	4	0
Hepatobiliary disorders			
Hypertransaminasaemia			
subjects affected / exposed	2 / 28 (7.14%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	2	2	0
Skin and subcutaneous tissue disorders			
Dry skin			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Rash			
subjects affected / exposed	3 / 28 (10.71%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	3	1	0
Rash maculo-papular			
subjects affected / exposed	3 / 28 (10.71%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences (all)	3	1	1
Renal and urinary disorders			

Dysuria			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Glycosuria			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Proteinuria			
subjects affected / exposed	3 / 28 (10.71%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	3	0	0
Renal failure			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Endocrine disorders			
Adrenal insufficiency			
subjects affected / exposed	0 / 28 (0.00%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	0	2	0
Musculoskeletal and connective tissue disorders			
Back pain			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Pain in extremity			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences (all)	1	1	1
Infections and infestations			
Adenoviral hepatitis			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Adenovirus infection			
subjects affected / exposed	3 / 28 (10.71%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	3	0	0
BK virus infection			
subjects affected / exposed	3 / 28 (10.71%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	3	1	0
Bacteraemia			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0

COVID-19			
subjects affected / exposed	3 / 28 (10.71%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	3	1	0
Candida infection			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0
Cytomegalovirus infection reactivation			
subjects affected / exposed	0 / 28 (0.00%)	3 / 23 (13.04%)	1 / 5 (20.00%)
occurrences (all)	0	3	1
Cytomegalovirus viraemia			
subjects affected / exposed	3 / 28 (10.71%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	3	0	0
Enterovirus infection			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Epstein-Barr viraemia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	1	0	1
Epstein-Barr virus infection reactivation			
subjects affected / exposed	1 / 28 (3.57%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	1	1	0
Gastroenteritis adenovirus			
subjects affected / exposed	1 / 28 (3.57%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	1	2	0
Klebsiella bacteraemia			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Klebsiella infection			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	1	0	1
Pneumonia			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Rhinovirus infection			

subjects affected / exposed	2 / 28 (7.14%)	3 / 23 (13.04%)	0 / 5 (0.00%)
occurrences (all)	2	3	0
Sepsis			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Upper respiratory tract infection			
subjects affected / exposed	3 / 28 (10.71%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	3	0	0
Viral rhinitis			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	0	0	1
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	1 / 28 (3.57%)	2 / 23 (8.70%)	1 / 5 (20.00%)
occurrences (all)	1	2	1
Hyperammonaemia			
subjects affected / exposed	0 / 28 (0.00%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	0	0	0
Hypercalcaemia			
subjects affected / exposed	1 / 28 (3.57%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	1	0	1
Hyperkalaemia			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	0 / 5 (0.00%)
occurrences (all)	2	0	0
Hypermagnesaemia			
subjects affected / exposed	2 / 28 (7.14%)	0 / 23 (0.00%)	1 / 5 (20.00%)
occurrences (all)	2	0	1
Hypervolaemia			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	1 / 5 (20.00%)
occurrences (all)	2	1	1
Hypoalbuminaemia			
subjects affected / exposed	2 / 28 (7.14%)	2 / 23 (8.70%)	1 / 5 (20.00%)
occurrences (all)	2	2	1
Hypokalaemia			
subjects affected / exposed	9 / 28 (32.14%)	6 / 23 (26.09%)	1 / 5 (20.00%)
occurrences (all)	9	6	1

Hypomagnesaemia			
subjects affected / exposed	5 / 28 (17.86%)	3 / 23 (13.04%)	0 / 5 (0.00%)
occurrences (all)	5	3	0
Hypophosphataemia			
subjects affected / exposed	3 / 28 (10.71%)	2 / 23 (8.70%)	0 / 5 (0.00%)
occurrences (all)	3	2	0
Iron overload			
subjects affected / exposed	2 / 28 (7.14%)	1 / 23 (4.35%)	0 / 5 (0.00%)
occurrences (all)	2	1	0

Non-serious adverse events	Placebo (Cross-over Period)		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 4 (75.00%)		
Vascular disorders			
Hypertension			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
General disorders and administration site conditions			
Asthenia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Gait disturbance			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Pyrexia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Immune system disorders			
Acute graft versus host disease in intestine			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Acute graft versus host disease in skin			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Cytokine release syndrome			

subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Graft versus host disease			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
Graft versus host disease in liver			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
Hypogammaglobulinaemia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Reproductive system and breast disorders			
Pelvic pain			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Dyspnoea			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Epistaxis			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Hypoxia			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
Pleural effusion			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
Tachypnoea			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
Investigations			

Activated partial thromboplastin time prolonged subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Alanine aminotransferase increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Blood bicarbonate decreased subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Blood bilirubin increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Blood creatinine increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Enterobacter test positive subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Gamma-glutamyltransferase increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Lipase increased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Neutrophil count decreased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Platelet count decreased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Weight decreased subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Injury, poisoning and procedural complications			

Traumatic haematoma subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Congenital, familial and genetic disorders Phimosis subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Cardiac disorders Pericardial effusion subjects affected / exposed occurrences (all) Sinus tachycardia subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1 1 / 4 (25.00%) 1		
Nervous system disorders Somnolence subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Blood and lymphatic system disorders Anaemia subjects affected / exposed occurrences (all) Febrile neutropenia subjects affected / exposed occurrences (all) Thrombocytopenia subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1 0 / 4 (0.00%) 0 0 / 4 (0.00%) 0		
Eye disorders Orbital haematoma subjects affected / exposed occurrences (all) Vision blurred subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1 0 / 4 (0.00%) 0		
Gastrointestinal disorders			

Abdominal distension subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Abdominal pain subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Ascites subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Constipation subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Diarrhoea subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Gastritis subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Gastrointestinal haemorrhage subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Nausea subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Stomatitis subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Vomiting subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Hepatobiliary disorders Hypertransaminasaemia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Skin and subcutaneous tissue disorders			

Dry skin subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Rash subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Rash maculo-papular subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Renal and urinary disorders Dysuria subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Glycosuria subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Proteinuria subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Renal failure subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Endocrine disorders Adrenal insufficiency subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Musculoskeletal and connective tissue disorders Back pain subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Pain in extremity subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Infections and infestations Adenoviral hepatitis			

subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Adenovirus infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
BK virus infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Bacteraemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
COVID-19				
subjects affected / exposed	1 / 4 (25.00%)			
occurrences (all)	1			
Candida infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Cytomegalovirus infection reactivation				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Cytomegalovirus viraemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Enterovirus infection				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Epstein-Barr viraemia				
subjects affected / exposed	0 / 4 (0.00%)			
occurrences (all)	0			
Epstein-Barr virus infection reactivation				
subjects affected / exposed	1 / 4 (25.00%)			
occurrences (all)	1			
Gastroenteritis adenovirus				

subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Klebsiella bacteraemia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Klebsiella infection			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Pneumonia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Rhinovirus infection			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Sepsis			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Upper respiratory tract infection			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Viral rhinitis			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Metabolism and nutrition disorders			
Decreased appetite			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Hyperammonaemia			
subjects affected / exposed	1 / 4 (25.00%)		
occurrences (all)	1		
Hypercalcaemia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		
Hyperkalaemia			
subjects affected / exposed	0 / 4 (0.00%)		
occurrences (all)	0		

Hypermagnesaemia subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Hypervolaemia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Hypoalbuminaemia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Hypokalaemia subjects affected / exposed occurrences (all)	1 / 4 (25.00%) 1		
Hypomagnesaemia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Hypophosphataemia subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		
Iron overload subjects affected / exposed occurrences (all)	0 / 4 (0.00%) 0		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
17 December 2021	Major revisions include: - Labeled safety objectives and endpoints as primary. - Added a requirement for participants who receive a third dose for recurrence to undergo safety follow-up for 20 weeks after the third dose of study treatment. - Added a statement that participating sites will adhere to relevant safety standards. - Added a statement that participants will be approached for enrollment in a long-term registry study. - Provided additional guidance on permitted and prohibited medications and the recording of concomitant medications. - Updated text surrounding study treatment discontinuation. - Added the Intent-to-Treat Population. - Deleted the Modified Intent-to-Treat Population. - Updated the planned analysis of the primary efficacy endpoint to be analyzed based on logistic regression instead of Fisher's exact test. - Provided justification for analysis of the primary efficacy endpoint at Day 29 and for durability of response assessments. - Revised schedule of assessments. - Provided details on interim analyses. - Added additional guidance on post-infusion monitoring. - Added a statement that all protocol amendments will be submitted to regulatory authorities and ethics committees/Institutional Review Boards as appropriate. - Added a statement that in some jurisdictions, legally effective informed consent must be obtained from all legal guardians for minor participants. - Provided guidance on estimating fraction of inspired oxygen.
23 August 2022	Major Revisions include: - Revised the order and timing of Study Objectives and Endpoints. - Revised Eligibility Criteria. - Added flexibility in blood specimen testing to allow blood specimen results from local laboratory results to be used for confirmation of eligibility and dosing if Adenovirus viral load results from the central laboratory are not available. - Expanded the screening window. - Added clarifying information regarding premature Crossover. - Added Data Safety Monitoring Board review and recommendation of preliminary safety data from 5 participants ≥1 and ≤6 years of age prior to enrolling participants under age 1. - Statistical Updates. - Added language regarding premedication with an antihistamine. - Streamlined study drug administration and dosing instructions. - Updated text to clarify that no subsequent infusion of blinded study treatment should occur in participants who develop new onset graft versus host disease (Grade >2) or worsening of graft versus host disease. - Added Clarifying information regarding Target Disease Endpoint Definitions.
21 September 2023	Major Revisions include: - Revised Eligibility Criteria. - Revised Concomitant Medications. - Analysis updates.
30 November 2023	Major Revisions include: - Addition of Interim analysis (sample size re-estimation and futility analysis). - Changes in Sample Size Determination. - Changes in Primary Analysis.

Notes:

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.

The study was discontinued following a pre-planned DSMB futility analysis concluding that the study was unlikely to meet its primary endpoint, no safety concerns were identified.

Notes: