Clinical trial results:
Full title of the trial: Allogeneic Stem Cell Transplantation of NiCord®, Umbilical Cord Blood-derived Ex Vivo Expanded Stem and Progenitor Cells, in Adolescents and Adult Patients with Hematological Malignancies

Summary

EudraCT number	2014-000074-19
Trial protocol	ES IT NL
Global end of trial date	29 June 2018

Results information

Result version number	v1 (current)
This version publication date	12 July 2019
First version publication date	12 July 2019
Summary attachment (see zip file)	Synopsis (CSR_SNG01_v1.0_20JUN2019 Synopsis.pdf)

Trial information

Trial identification

Sponsor protocol code	GCP#03.01.020

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01816230
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Gamida Cell Ltd.
Sponsor organisation address	Beit Ofer, 5 Nahum Hafzadi, Jerusalem, Israel, 9548401
Public contact	Kelly Myers, Gamida Cell Ltd, 972 26595631, kelly@gamida-cell.com
Scientific contact	Kelly Myers, Gamida Cell Ltd, 972 26595631, kelly@gamida-cell.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	27 June 2018
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	29 June 2018
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Assess the cumulative incidence of NiCord-derived neutrophil engraftment at 42 days following transplantation.
Assess the incidence of secondary graft failure at 180 days following transplantation of NiCord.

Protection of trial subjects:

The Data Monitoring Committee met to discuss and ensure patient safety throughout the trial. The committee reviewed the accumulated data after 3 patients entered the study and were assessed at day 100 following transplant. At this initial review, the DMC monitored in particular: any occurrence of primary or secondary graft failure, as well as any substantial decrease in donor chimerism (especially myeloid chimerism), or evidence of impending graft failure, as well as all study data in general. A subsequent DMC review took place after 3 patients received the cryopreserved NiCord product and were assessed at day 100 following transplant. Early safety assessment guidelines were used to monitor primary & secondary graft failure, non-relapse mortality at 100 days and alert the DMC.

Background therapy:

1) Myeloablative conditioning regimens: (Each transplant center used the same conditioning regimen for all patients transplanted at their center).
a) Regimen A (Day -11 to -2):
Total Body irradiation (TBI) 1350 cGy, Fludarabine: 160 mg/m2, (optional: Cyclophosphamide: 120 mg/kg or Thiotepa: 10 mg/kg)
b) Regimen B (Day -7 to -3):
Thiotepa 10 mg/kg, Busulfan 9.6 mg/kg, Fludarabine 150 mg/m2
c) Regimen C (Day -5 to -2):
Clofarabine 120 mg/m2, Fludarabine 40 mg/m2, Busulfan AUC 90 mg*h/L

2) GvHD prophylaxis regimen consisted of Mycophenolate Mofetil (MMF) and a calcineurin inhibitor (Tacrolimus or Cyclosporine). Each transplant center used the same calcineurin inhibitor for all patients. Tacrolimus or Cyclosporine from day -3 through day +150 Target tacrolimus trough blood levels of 5-15 ng/ml. Target cyclosporine trough levels of 200-400 ng/mL by TDX method (or 100-250 ng/mL by Tandem MS or equivalent level for other CSA testing methods).
Mycophenolate Mofetil (MMF) 1 g TID IV or PO (15 mg/kg IV TID if patient weighs <50 kg) beginning day -3 to at least day +60.

3) G-CSF therapy was started on day +1 at a dose of 5 µg/kg/day continuing at least until the ANC is >1,000/µl x 2 consecutive days.

Evidence for comparator:

N/A

Actual start date of recruitment	21 May 2013
Long term follow-up planned	Yes
Long term follow-up rationale	Safety, Efficacy, Ethical reason, Regulatory reason, Scientific research
Long term follow-up duration	4 Years
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	United States: 18
Country: Number of subjects enrolled	Singapore: 2
Country: Number of subjects enrolled	Italy: 1
Country: Number of subjects enrolled	Netherlands: 3
Country: Number of subjects enrolled	Spain: 14
Worldwide total number of subjects	38
EEA total number of subjects	18

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)	0
Children (2-11 years)	0
Adolescents (12-17 years)	3
Adults (18-64 years)	35
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Recruitment began in the US in May 2013, followed by Spain, Italy, Netherlands and Singapore.

Pre-assignment

Screening details:

59 subjects assessed for eligibility. 11 ineligible (5 progressive disease, 5 medical deterioration, 1 minimal residual disease). 5 withdrew due to projected waiting time for NiCord production. 5 transplanted with unmanipulated CBU only. 2 transplanted with unmanipulated CBU + NiCord. 36 treated with NiCord.

Period 1

Period 1 title	Overall trial (overall period)
Is this the baseline period?	Yes
Allocation method	Non-randomised - controlled
Blinding used	Not blinded

Arms

Arm title	NiCord

Arm description:

NiCord® is a cryopreserved stem/progenitor cell based product of purified CD133+ cells composed of ex vivo expanded allogeneic UCB cells. NiCord® comprises: 1) cord blood-derived ex vivo expanded CD133+ cells (NiCord® cultured fraction (CF)); and 2) the non-cultured cell fraction (CD133-) of the same CBU (NiCord® Non-cultured Fraction (NF)). Both fractions, i.e. NiCord® CF and NiCord® NF, will be kept frozen until they are infused on the day of transplantation.

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

Dosage and administration details:

Dosage is administered once.

Number of subjects in period 1	NiCord
Started	38
Completed	38

Baseline characteristics

Reporting groups	
Reporting group title	Overall trial

Reporting group description: -

Reporting group values	Overall trial	Total	
Number of subjects	38	38	
Age categorical Units: Subjects			
Adolescents (12-17 years)	3	3	
Adults (18-64 years)	35	35	
Gender categorical Units: Subjects			
Female	16	16	
Male	22	22	

Subject analysis sets	
Subject analysis set title	Efficacy & Safety Analysis
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Any patient that received NiCord as a standalone graft.

Subject analysis set title	Additional Safety Analysis
Subject analysis set type	Safety analysis

Subject analysis set description:

included all patients who received NiCord® as a standalone, or with an unmanipulated CBU.

Reporting group values	Efficacy & Safety Analysis	Additional Safety Analysis	
Number of subjects	36	38	
Age categorical Units: Subjects			
Adolescents (12-17 years)	2	3	
Adults (18-64 years)	34	35	
Gender categorical Units: Subjects			
Female	16	16	
Male	20	22	

End points

End points reporting groups

Reporting group title	NiCord

Reporting group description:

NiCord® is a cryopreserved stem/progenitor cell based product of purified CD133+ cells composed of ex vivo expanded allogeneic UCB cells. NiCord® comprises: 1) cord blood-derived ex vivo expanded CD133+ cells (NiCord® cultured fraction (CF)); and 2) the non-cultured cell fraction (CD133-) of the same CBU (NiCord® Non-cultured Fraction (NF)). Both fractions, i.e. NiCord® CF and NiCord® NF, will be kept frozen until they are infused on the day of transplantation.

Subject analysis set title	Efficacy & Safety Analysis
Subject analysis set type	Sub-group analysis

Subject analysis set description:

Any patient that received NiCord as a standalone graft.

Subject analysis set title	Additional Safety Analysis
Subject analysis set type	Safety analysis

Subject analysis set description:

included all patients who received NiCord® as a standalone, or with an unmanipulated CBU.

Primary: NiCord neutrophil engraftment

End point title	NiCord neutrophil engraftment[1]

End point description:

End point type	Primary

End point timeframe:

42 days following transplantation

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 is a single-arm study; to assess the incidence of neutrophil engraftment post transplant a cumulative incidence curve will be computed along with a 95% confidence interval at 42 days post-transplant. Death prior to engraftment will be considered as a competing risk.

End point values	NiCord	Efficacy & Safety Analysis		
Subject group type	Reporting group	Subject analysis set		
Number of subjects analysed	36	36		
Units: Proportion				
number (not applicable)	94	94		

Statistical analyses

No statistical analyses for this end point

Primary: Secondary graft failure

End point title	Secondary graft failure[2]

End point description:

End point type	Primary

End point timeframe:

180 days

Notes:

[2] - 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 is a single-arm study; the cumulative incidence of secondary graft failure out of those who had initial engraftment will be described using the cumulative incidence estimator, treating death and disease relapse/progression prior to secondary graft failure as a competing event.

End point values	NiCord	Efficacy & Safety Analysis		
Subject group type	Reporting group	Subject analysis set		
Number of subjects analysed	36	36		
Units: percent				
number (confidence interval 95%)	2.8 (0.2 to 12.6)	2.8 (0.2 to 12.6)		

Statistical analyses

No statistical analyses for this end point

Secondary: Time to neutrophil engraftment

End point title	Time to neutrophil engraftment

End point description:

End point type	Secondary

End point timeframe:

42 days

End point values	NiCord	Efficacy & Safety Analysis		
Subject group type	Reporting group	Subject analysis set		
Number of subjects analysed	36	36		
Units: days				
median (confidence interval 95%)	11.5 (9 to 14)	11.5 (9 to 14)		

Statistical analyses

No statistical analyses for this end point

Secondary: Time to platelet engraftment

End point title	Time to platelet engraftment

End point description:

End point type	Secondary

End point timeframe:
180 days

End point values	NiCord	Efficacy & Safety Analysis		
Subject group type	Reporting group	Subject analysis set		
Number of subjects analysed	34[3]	34		
Units: days				
median (confidence interval 95%)	34 (32 to 42)	34 (32 to 42)		

Notes:
[3] - Patients who engrafted neutrophils

Statistical analyses

No statistical analyses for this end point

Secondary: Platelet engraftment

End point title	Platelet engraftment

End point description:

End point type	Secondary

End point timeframe:
100 days

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	34			
Units: percent				
number (not applicable)	81			

Statistical analyses

No statistical analyses for this end point

Secondary: non-relapse mortality

End point title	non-relapse mortality

End point description:

End point type	Secondary

End point timeframe:
100 days

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	8.3 (2.1 to 20.3)			

Statistical analyses

No statistical analyses for this end point

Secondary: Acute GVHD II-IV

End point title	Acute GVHD II-IV

End point description:

End point type	Secondary

End point timeframe:

100 days

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	44.4 (27.7 to 59.9)			

Statistical analyses

No statistical analyses for this end point

Secondary: Acute GVHD III-IV

End point title	Acute GVHD III-IV

End point description:

End point type	Secondary

End point timeframe:

100 days

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	11 (3 to 24)			

Statistical analyses

No statistical analyses for this end point

Secondary: Chronic GVHD

End point title	Chronic GVHD

End point description:

End point type	Secondary

End point timeframe:

180 days

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	30.6 (16.3 to 46.1)			

Statistical analyses

No statistical analyses for this end point

Secondary: Chronic GVHD

End point title	Chronic GVHD

End point description:

End point type	Secondary

End point timeframe:

1 year

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	41.7 (25.1 to 57.4)			

Statistical analyses

No statistical analyses for this end point

Secondary: Secondary graft failure

End point title	Secondary graft failure

End point description:

End point type	Secondary

End point timeframe:

1 year

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	5.6 (1 to 16.6)			

Statistical analyses

No statistical analyses for this end point

Secondary: Overall survival

End point title	Overall survival

End point description:

End point type	Secondary

End point timeframe:

180 days

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	80 (63 to 90)			

Statistical analyses

No statistical analyses for this end point

Secondary: Overall survival

End point title	Overall survival

End point description:

End point type	Secondary

End point timeframe:

1 year

End point values	Efficacy & Safety Analysis			
Subject group type	Subject analysis set			
Number of subjects analysed	36			
Units: percent				
number (confidence interval 95%)	51 (33 to 67)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information

Timeframe for reporting adverse events:

up to 1 year post-transplant

Adverse event reporting additional description:

Infections and GvHD were reported up to 1 year post-transplant.
All common events post-transplant were collected up to day 42 post-transplant. Grade 3-4 non-serious adverse events collected up to one year post-transplant.
Grade 3-5 adverse events and all grades serious adverse events have been reported to the database.

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	21

Reporting groups

Reporting group title	NiCord treated patients

Reporting group description: -

Serious adverse events	NiCord treated patients		
Total subjects affected by serious adverse events			
subjects affected / exposed	35 / 38 (92.11%)		
number of deaths (all causes)	16		
number of deaths resulting from adverse events	8		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Haematological malignancy	Additional description: Includes: Acute myeloid leukaemia; Leukaemia recurrent; Myelodysplastic syndrome		
subjects affected / exposed	9 / 38 (23.68%)		
occurrences causally related to treatment / all	0 / 9		
deaths causally related to treatment / all	0 / 8		
Vascular disorders			
Deep vein thrombosis			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
General disorders and administration site conditions			
Multiple organ dysfunction syndrome			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Asthenia			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
fever			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Immune system disorders			
Graft versus host disease	Additional description: Includes: Acute Graft versus host disease (GvHD), GvHD, GvHD in gastrointestinal tract		
subjects affected / exposed	11 / 38 (28.95%)		
occurrences causally related to treatment / all	17 / 17		
deaths causally related to treatment / all	2 / 2		
Respiratory, thoracic and mediastinal disorders			
Respiratory disorder	Additional description: Includes: Bronchospasm; Dyspnoea; Pneumonitis; Pulmonary oedema; Respiratory failure		
subjects affected / exposed	5 / 38 (13.16%)		
occurrences causally related to treatment / all	0 / 5		
deaths causally related to treatment / all	0 / 0		
pain - non-cardiac			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Investigations			
Transaminases increased			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Injury, poisoning and procedural complications			
Transplant failure			
subjects affected / exposed	4 / 38 (10.53%)		
occurrences causally related to treatment / all	2 / 4		
deaths causally related to treatment / all	0 / 0		
Fall			

subjects affected / exposed	2 / 38 (5.26%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Infusion related reaction			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	1 / 1		
deaths causally related to treatment / all	0 / 0		
Cardiac disorders			
cardiac and vascular disorders	Additional description: Includes cardiogenic shock, atrial fibrilation, pericardial infusion		
subjects affected / exposed	3 / 38 (7.89%)		
occurrences causally related to treatment / all	0 / 3		
deaths causally related to treatment / all	0 / 1		
Nervous system disorders			
Central nervous system events	Additional description: Includes: Cerebrovascular accident; Encephalopathy; Haemorrhage intracranial; Spinal epidural haematoma; Syncope		
subjects affected / exposed	5 / 38 (13.16%)		
occurrences causally related to treatment / all	0 / 5		
deaths causally related to treatment / all	0 / 0		
Blood and lymphatic system disorders			
Febrile neutropenia			
subjects affected / exposed	4 / 38 (10.53%)		
occurrences causally related to treatment / all	0 / 4		
deaths causally related to treatment / all	0 / 1		
Eye disorders			
Optic nerve disorder			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Gastrointestinal disorders			
Gastrointestinal disorder	Additional description: Includes: Colitis; Diarrhoea; Gastrointestinal disorder; Stomatitis		
subjects affected / exposed	5 / 38 (13.16%)		
occurrences causally related to treatment / all	0 / 6		
deaths causally related to treatment / all	0 / 0		
Hepatobiliary disorders			
Venoocclusive liver disease			

subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Adrenal insufficiency			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Acute kidney injury			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Musculoskeletal and connective tissue disorders			
Myopathy			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Infections and infestations			
Infection	Additional description: Includes: Adenovirus; Bacteraemia; Cystitis; Cystitis viral; CMV viraemia; Device related; Enterocolitis infectious; Escherichia bacteraemia; Liver abscess; Lung infection; Pneumonia; Sepsis; URT infection, Viral haemorrhagic cystitis		
subjects affected / exposed	18 / 38 (47.37%)		
occurrences causally related to treatment / all	0 / 26		
deaths causally related to treatment / all	0 / 4		
Metabolism and nutrition disorders			
Fluid overload			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		
Anorexia			
subjects affected / exposed	1 / 38 (2.63%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

Frequency threshold for reporting non-serious adverse events: 3 %

Non-serious adverse events	NiCord treated patients		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	38 / 38 (100.00%)		
Neoplasms benign, malignant and unspecified (incl cysts and polyps)			
Disease recurrence	Additional description: includes serious adverse events		
subjects affected / exposed	9 / 38 (23.68%)		
occurrences (all)	9		
General disorders and administration site conditions			
Mucosal inflammation	Additional description: includes serious adverse events		
subjects affected / exposed	9 / 38 (23.68%)		
occurrences (all)	9		
Pain	Additional description: includes serious adverse events		
subjects affected / exposed	7 / 38 (18.42%)		
occurrences (all)	9		
Oedema			
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	3		
Immune system disorders			
Graft versus host disease	Additional description: includes serious adverse events		
subjects affected / exposed	10 / 38 (26.32%)		
occurrences (all)	16		
Respiratory, thoracic and mediastinal disorders			
Dyspnoea	Additional description: includes serious adverse events		
subjects affected / exposed	3 / 38 (7.89%)		
occurrences (all)	4		
Hypoxia	Additional description: includes serious adverse events		
subjects affected / exposed	4 / 38 (10.53%)		
occurrences (all)	5		
Investigations			
Blood creatine increased	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Hepatic enzyme increased	Additional description: includes serious adverse events		

subjects affected / exposed	3 / 38 (7.89%)		
occurrences (all)	3		
Injury, poisoning and procedural complications			
Fall	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Transplant failure	Additional description: includes serious adverse events		
subjects affected / exposed	4 / 38 (10.53%)		
occurrences (all)	4		
Cardiac disorders			
Cardiac disorder	Additional description: includes arrhythmia, atrial fibrillation, cardiogenic shock, pericardial infusion. includes serious adverse events		
subjects affected / exposed	4 / 38 (10.53%)		
occurrences (all)	5		
Nervous system disorders			
Somnolence	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Syncope	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Blood and lymphatic system disorders			
Febrile neutropenia	Additional description: includes serious adverse events		
subjects affected / exposed	4 / 38 (10.53%)		
occurrences (all)	4		
Gastrointestinal disorders			
Diarrhoea	Additional description: includes serious adverse events		
subjects affected / exposed	3 / 38 (7.89%)		
occurrences (all)	3		
Dysphagia	Additional description: includes serious adverse events		
subjects affected / exposed	4 / 38 (10.53%)		
occurrences (all)	5		
Nausea	Additional description: includes serious adverse events		
subjects affected / exposed	11 / 38 (28.95%)		
occurrences (all)	12		
Hepatobiliary disorders			

hepatobiliary disorder	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Renal and urinary disorders			
Acute kidney injury	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Musculoskeletal and connective tissue disorders			
Muscular weakness	Additional description: includes serious adverse events		
subjects affected / exposed	4 / 38 (10.53%)		
occurrences (all)	4		
Infections and infestations			
Bacteraemia	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	3		
Enterocolitis infectious	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Infection	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Sepsis	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Metabolism and nutrition disorders			
Decreased appetite	Additional description: includes serious adverse events		
subjects affected / exposed	9 / 38 (23.68%)		
occurrences (all)	10		
Hyperglycaemia	Additional description: includes serious adverse events		
subjects affected / exposed	6 / 38 (15.79%)		
occurrences (all)	6		
Hypoalbuminaemia	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	3		
Hyponatraemia	Additional description: includes serious adverse events		

subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		
Hypophosphataemia	Additional description: includes serious adverse events		
subjects affected / exposed	2 / 38 (5.26%)		
occurrences (all)	2		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
20 February 2014	To expand patient population and clarify eligibility criteria
20 March 2014	Change in manufacturing procedure to cryopreserved product. Modify eligibility criteria. Additional option for conditioning regimen.
28 August 2014	Add safety objective. Define childbearing potential and appropriate contraception.
04 December 2014	Modify eligibility criteria. Additional regimen specific stopping guidelines. Additional supportive and viral monitoring care guidelines. Update AE reporting guidelines.
27 March 2015	Increase number of patients for study enrollment. Modify eligibility criteria, COA release criteria, GvHD prophylaxis medication administration and assessment grading criteria.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported

Online references

http://www.ncbi.nlm.nih.gov/pubmed/28392378

http://www.ncbi.nlm.nih.gov/pubmed/30523748

http://www.ncbi.nlm.nih.gov/pubmed/24911148