Clinical trial results:
A Phase 1/2 Combined Dose Ranging and Randomised, Open-label, Comparative Study of the Efficacy and Safety of Plerixafor in Addition to Standard Regimens for Mobilisation of Haematopoietic Stem Cells into Peripheral Blood, and Subsequent Collection by Apheresis, Versus Standard Mobilisation Regimens Alone in Paediatric Patients, Aged 1 to <18 Years, with Solid Tumours Eligible for Autologous Transplants

Summary

EudraCT number	2010-019340-40
Trial protocol	GB DE IT ES BE HU CZ NL DK PL Outside EU/EEA
Global end of trial date	09 May 2017

Results information

Result version number	v1 (current)
This version publication date	24 November 2017
First version publication date	24 November 2017

Trial information

Trial identification

Sponsor protocol code	MOZ15609-DFI12860

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01288573
WHO universal trial number (UTN)	-
Other trial identifiers	Study name: MOZAIC

Notes:

Sponsors

Sponsor organisation name	Genzyme Corporation
Sponsor organisation address	500 Kendall Street , Cambridge, MA, United States, 02142
Public contact	Trial Transparency Team, Sanofi aventis recherche & développement, Contact-US@sanofi.com
Scientific contact	Trial Transparency Team, Sanofi aventis recherche & développement, Contact-US@sanofi.com

Notes:

Paediatric regulatory details

Is trial part of an agreed paediatric investigation plan (PIP)	Yes
EMA paediatric investigation plan number(s)	EMEA-000174-PIP01-07
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?	Yes

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	06 June 2017
Is this the analysis of the primary completion data?	No

Global end of trial reached?	Yes
Global end of trial date	09 May 2017
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The primary objective of this study is to confirm the appropriate dose and efficacy, and to characterise the safety, pharmacokinetics and pharmacodynamics of plerixafor across age and size in paediatric cancer subjects when given in addition to standard mobilisation of hematopoietic stem cells (HSCs) into peripheral blood.

Protection of trial subjects:

The study was conducted by investigators experienced in the treatment of paediatric patients. The parent(s) or guardian(s) as well as the children were fully informed of all pertinent aspects of the clinical trial as well as the possibility to discontinue at any time. In addition to the consent form for the parent(s)/guardian(s), an assent form in child-appropriate language was provided and explained to the child. Repeated invasive procedures were minimized. The number of blood samples as well as the amount of blood drawn were adjusted according to age and weight. A topical anaesthesia may have been used to minimize distress and discomfort.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	18 February 2011
Long term follow-up planned	Yes
Long term follow-up rationale	Safety
Long term follow-up duration	2 Years
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Netherlands: 3
Country: Number of subjects enrolled	Poland: 2
Country: Number of subjects enrolled	Spain: 6
Country: Number of subjects enrolled	United Kingdom: 7
Country: Number of subjects enrolled	Belgium: 1
Country: Number of subjects enrolled	Czech Republic: 10
Country: Number of subjects enrolled	France: 2
Country: Number of subjects enrolled	Germany: 12
Country: Number of subjects enrolled	Hungary: 2
Country: Number of subjects enrolled	Italy: 22

Country: Number of subjects enrolled	Israel: 5
Worldwide total number of subjects	72
EEA total number of subjects	67

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)	4
Children (2-11 years)	52
Adolescents (12-17 years)	16
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

The study was conducted in 11 countries between 18 February 2011 and 09 May 2017. A total of 72 subjects were enrolled and treated in the study. The study was conducted in 2 stages. Stage 1 was the dose escalation study and stage 2 was the comparative study using the appropriate dosing regimen identified in Stage 1.

Pre-assignment

Screening details:

During Stage 1, 27 subjects assigned to plerixafor dose group according to their age & progress with dose escalation within that age group. During Stage 2, 45 subjects randomized in 2:1 ratio to receive either plerixafor + standard mobilization or standard mobilization alone.Standard mobilization was G-CSF ± chemotherapy per site standard practice.

Period 1

Period 1 title	Overall Study (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Are arms mutually exclusive?	Yes
Arm title	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years

Arm description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 160 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: Granulocyte Colony-Stimulating Factor (G-CSF)
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy as per site standard practice.

Arm title	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years

Arm description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental

Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 240 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years

Arm description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 320 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years

Arm description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 160 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years

Arm description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

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Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 240 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years

Arm description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

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Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 320 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years

Arm description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 160 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years

Arm description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

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Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 240 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years

Arm description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 320 mcg/kg was administered 9 to 11 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 2: Standard Mobilization Regimen - G-CSF

Arm description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Treatment G-CSF (alone or in combination with chemotherapy) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Non IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Arm title	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF

Arm description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Arm type	Experimental
Investigational medicinal product name	Plerixafor
Investigational medicinal product code	GZ316455/ SAR316455
Other name	Mozobil®
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

Plerixafor 240 mcg/kg was administered 8 to 12 hours prior to the initiation of apheresis.

Investigational medicinal product name	Non-IMP: G-CSF
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

10 mcg/kg daily alone or in combination with chemotherapy per site standard practice.

Number of subjects in period 1	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years
Started	3	3	3
Completed	2	2	3
Not completed	1	1	0
Consent withdrawn by subject	-	-	-
Death	-	-	-
Other than specified	1	-	-
Lost to follow-up	-	1	-
Progressive disease	-	-	-

Number of subjects in period 1	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years
Started	3	3	3
Completed	2	3	0
Not completed	1	0	3
Consent withdrawn by subject	-	-	-
Death	1	-	1
Other than specified	-	-	-
Lost to follow-up	-	-	1
Progressive disease	-	-	1

Number of subjects in period 1	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years
Started	3	3	3
Completed	2	2	1
Not completed	1	1	2
Consent withdrawn by subject	-	-	-
Death	-	1	1
Other than specified	1	-	1
Lost to follow-up	-	-	-
Progressive disease	-	-	-

Number of subjects in period 1	Stage 2: Standard Mobilization	Stage 2: Plerixafor + Standard

	Regimen - G-CSF	Mobilization Regimen -G-CSF
Started	15	30
Completed	10	25
Not completed	5	5
Consent withdrawn by subject	1	1
Death	3	3
Other than specified	-	1
Lost to follow-up	-	-
Progressive disease	1	-

Baseline characteristics

Reporting groups

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF)

was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 2: Standard Mobilization Regimen - G-CSF

Reporting group description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Treatment G-CSF (alone or in combination with chemotherapy) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF

Reporting group description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group values	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years
Number of subjects	3	3	3
Age categorical Units: Subjects			
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-<12 years)	3	3	3
Adolescents (12-<18 years)	0	0	0
Gender categorical Units: Subjects			
Female	2	2	3
Male	1	1	0

Reporting group values	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years
Number of subjects	3	3	3
Age categorical Units: Subjects			
Infants and toddlers (28 days-23 months)	0	0	0

Children (2-<12 years)	3	3	3
Adolescents (12-<18 years)	0	0	0

Gender categorical Units: Subjects			
Female	1	1	1
Male	2	2	2

Reporting group values	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years
Number of subjects	3	3	3
Age categorical Units: Subjects			
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-<12 years)	0	0	0
Adolescents (12-<18 years)	3	3	3
Gender categorical Units: Subjects			
Female	1	3	2
Male	2	0	1

Reporting group values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF	Total
Number of subjects	15	30	72
Age categorical Units: Subjects			
Infants and toddlers (28 days-23 months)	3	1	4
Children (2-<12 years)	10	24	52
Adolescents (12-<18 years)	2	5	16
Gender categorical Units: Subjects			
Female	8	11	35
Male	7	19	37

End points

End points reporting groups

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF)

was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 2: Standard Mobilization Regimen - G-CSF

Reporting group description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Treatment G-CSF (alone or in combination with chemotherapy) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF

Reporting group description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Primary: Stage 2: Percentage of Subjects Achieving At least a Doubling of Peripheral Blood CD34+ Count

End point title	Stage 2: Percentage of Subjects Achieving At least a Doubling of Peripheral Blood CD34+ Count[1]

End point description:

Percentage of subjects who achieved at least a doubling of peripheral blood CD34+ count (known as successful mobilisation) from the morning of the day preceding the first apheresis day to the morning prior to apheresis was reported in this endpoint. For those subjects who did not have the first apheresis as planned, peripheral CD34+ counts from the morning of the day prior to the planned apheresis day and from the morning of the planned apheresis day itself were included in the analysis. Analysis was performed on full analysis set (FAS) which is comprised of all subjects randomized in Stage 2 according to intent-to-treat (ITT) principle (subjects were analysed according to the treatment group allocated by randomization).

End point type	Primary

End point timeframe:

From the morning of the day preceding the first apheresis day to the morning prior to apheresis

Notes:

[1] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (confidence interval 95%)	28.6 (8.4 to 58.1)	80 (61.4 to 92.3)		

Statistical analyses

Statistical analysis title	G-CSF alone vs. G-CSF + Plerixafor

Statistical analysis description:

The difference of percentage of successful mobilization was relative to standard mobilization alone treatment group. The confidence interval (CI) of the difference is based on the Wald asymptotic CI with continuity correction method.

Comparison groups	Stage 2: Standard Mobilization Regimen - G-CSF v Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF
Number of subjects included in analysis	45
Analysis specification	Pre-specified
Analysis type	superiority
P-value	= 0.0019 [2]
Method	Fisher exact
Parameter estimate	Difference of percentage
Point estimate	51.4
Confidence interval	
level	95 %
sides	2-sided
lower limit	18.5
upper limit	84.3

Notes:

[2] - Threshold level was estimated at 0.05 level.

Secondary: Stage 2: Median Number of Days of Apheresis Required to Reach >=2 *10^6 CD34+ cells/kg

End point title	Stage 2: Median Number of Days of Apheresis Required to Reach >=2 *10^6 CD34+ cells/kg[3]

End point description:

The number of days of apheresis to reach >=2*10^6 CD34+ cells/kg was estimated using the Kaplan-Meier method. A subject was classified as having the event if the cumulative number of CD34+ cells/kg collected reached the target of ≥2*10^6 CD34+ cells/kg. Subjects not reaching target at the end of apheresis period were censored on the last apheresis day (maximum of 5 apheresis in study). Analysis was performed on FAS. Here, 99999 represents data not calculated since majority of subjects reached CD34+ cells target within 1 day.

End point type	Secondary

End point timeframe:

Day 1 up to Day 5

Notes:

[3] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: Days				
median (full range (min-max))	1 (-99999 to 99999)	1 (-99999 to 99999)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Yield of CD34+ (*10^6 cells/kg) Cells for Each Apheresis

End point title	Stage 2: Yield of CD34+ (*10^6 cells/kg) Cells for Each Apheresis[4]

End point description:

Apheresis commenced on the morning following the day when the peripheral blood (PB) CD34+ count reached the target trigger point minimum of 7 CD34+ cells/mcl. Here, 99999 represented that data was not estimable as none of the evaluable subjects had Day 3 apheresis except for 1 subject in the plerixafor + standard mobilization arm. Analysis was performed on FAS population. Here 'n' signifies number of subjects with available data for specified category.

End point type	Secondary

End point timeframe:

Days 1 up to Day 3 following apheresis

Notes:

[4] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: cells (*10^6 cells/kg)				
arithmetic mean (standard deviation)				
Day 1 (n= 14, 29)	17.57 (± 20.79)	19.44 (± 36.7)		
Day 2 (n = 2, 3)	2.8 (± 3.3)	0.69 (± 1.07)		
Day 3 (n = 0, 1)	99999 (± 99999)	0.06 (± 99999)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Total CD34+ Cell Yield

End point title	Stage 2: Total CD34+ Cell Yield[5]

End point description:

The cumulative CD34+ cells/kg yield was calculated by summing the CD34+ yield from each apheresis. Analysis was performed on FAS population. Number of subjects analysed=number of subjects evaluable for this endpoint.

End point type	Secondary

End point timeframe:

Day 1 up to Day 3 following apheresis

Notes:

[5] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	14	29		
Units: cells (*10^6 cells/kg)				
arithmetic mean (standard deviation)	17.61 (± 20.76)	19.44 (± 36.69)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects Proceeding to Autologous Transplant

End point title	Stage 2: Percentage of Subjects Proceeding to Autologous Transplant[6]

End point description:

The percentage of subjects proceeding to autologous transplant was calculated using the total number of subjects in the corresponding analysis set as the denominator. Analysis was performed on FAS population.

End point type	Secondary

End point timeframe:

Within 6 months of last apheresis (up to maximum duration of 2.5 years)

Notes:

[6] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (confidence interval 95%)	66.7 (38.4 to 88.2)	76.7 (57.7 to 90.1)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects Successfully Engrafting

End point title	Stage 2: Percentage of Subjects Successfully Engrafting[7]

End point description:

Successful engrafting was when both absolute neutrophil count (ANC) & platelet were successfully engrafted. Percentage of subjects with successful engraftment was calculated using the total number of subjects in each treatment group who received transplantation as the denominator. Analysis was performed on FAS population. Number of subjects analysed= number of subjects who underwent grafting.

End point type	Secondary

End point timeframe:

24 months after transplant (up to maximum duration of 2.5 years)

Notes:

[7] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	23		
Units: percentage of subjects				
number (confidence interval 95%)	100 (69.2 to 100)	100 (85.2 to 100)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects with Durable Engraftment

End point title	Stage 2: Percentage of Subjects with Durable Engraftment[8]

End point description:

Durable engraftment was defined as subjects with both ANC and platelet engrafted and who remained in the engraftment status at 3, 6, 12, and 24 months post-transplant. The percentage of subjects with durable engraftment at 3, 6, 12, and 24 months post-transplant was calculated using the total number of subjects in each treatment group who received transplant as the denominator. Analysis was performed on FAS population. Number of subjects analysed=number of subjects evaluable for this endpoint.

End point type	Secondary

End point timeframe:

At 3, 6, 12 and 24 months post-transplant (up to maximum duration of 2.5 years)

Notes:

[8] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period. Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	23		
Units: percentage of subjects				
number (confidence interval 95%)				
Month 3	100 (69.2 to 100)	91.3 (72 to 98.9)		
Month 6	90 (55.5 to 99.7)	87 (66.4 to 97.2)		
Month 12	80 (44.4 to 97.5)	87 (66.4 to 97.2)		
Month 24	80 (44.4 to 97.5)	82.6 (61.2 to 95)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 1 and Stage 2: Overview of Safety

End point title	Stage 1 and Stage 2: Overview of Safety

End point description:

Any untoward medical occurrence in a subject who received investigational medicinal product was considered an AE regardless of causal relationship with this treatment. Treatment-Emergent Adverse Events (TEAEs): AEs that developed/worsened/became serious during from the date of enrollment until 30 days after the last dose of subject's study mobilization regimen/until the 1st dose of next anticancer therapy or pre-transplant myeloablative therapy, whichever occurred first. Serious AE (SAE):any untoward medical occurrence that resulted in any of following outcomes: death, life-threatening, required initial/prolonged in-patient hospitalization, persistent or significant disability/incapacity, congenital anomaly/birth defect/considered as medically important event. Any TEAE included both serious & non-serious AEs. Safety set was defined as all subjects who received at least one dose of study drug.

End point type	Secondary

End point timeframe:

From baseline up to the follow up visit (up to 6.2 years)

End point values	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	3	3	3	3
Units: percentage of subjects				
number (not applicable)				
Any TEAE	67	33	33	67
Any treatment-emergent SAE	33.33	0	0	66.67

End point values	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years
Subject group type	Reporting group	Reporting group	Reporting group	Reporting group
Number of subjects analysed	3	3	3	3
Units: percentage of subjects				
number (not applicable)				
Any TEAE	67	67	100	33
Any treatment-emergent SAE	0	33.33	66.67	0

End point values	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	3	15	30	
Units: percentage of subjects				
number (not applicable)				
Any TEAE	67	66.7	76.7	
Any treatment-emergent SAE	33.33	26.7	30	

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects Who Had Hospitalization

End point title	Stage 2: Percentage of Subjects Who Had Hospitalization[9]

End point description:

Analysis was performed on safety set for stage 2 which included all randomized subjects who received at least one study dose (either plerixafor or standard mobilization) in Stage 2. Subjects from Stage 2 were to be analysed for safety according to the treatment they actually received.

End point type	Secondary

End point timeframe:

From randomisation in stage 2 until the end of study (up to maximum duration of 2.5 years)

Notes:

[9] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (not applicable)	86.7	90		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects With Tumor Cell Mobilisation Positive in Peripheral Blood

End point title	Stage 2: Percentage of Subjects With Tumor Cell Mobilisation Positive in Peripheral Blood[10]

End point description:

Tumour cell mobilisation was evaluated by assessment of blood samples for the presence of tumour cells in peripheral blood. Blood samples were collected on the morning of the day preceding the first apheresis day and in the morning prior to G-CSF administration on the first apheresis day itself, as well as in apheresis product after first, second and third apheresis prior to cryopreservation in subjects with neuroblastoma, Ewing’s sarcoma and alveolar rhabdomyosarcoma. Analysis was performed on safety set for stage-2. Here 'n' signifies number of subjects with available data for specified categories for each arm respectively.

End point type	Secondary

End point timeframe:

On morning at Day prior to first planned apheresis, morning of first planned apheresis day, first apheresis, second apheresis, third apheresis additional apheresis days (maximum of 5 apheresis) (up to maximum duration of 2.5 years)

Notes:

[10] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (not applicable)				
on morning prior to 1st apheresis(n=9, 20)	11.1	0		
on morning of 1st apheresis (n=9, 21)	0	0		
at 1st apheresis (n=10, 20)	10	5		
at 2nd apheresis (n=2, 2)	100	0		
at 3rd apheresis (n= 0, 1)	0	0		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Post-Transplant Relapse Rate

End point title	Stage 2: Post-Transplant Relapse Rate[11]

End point description:

Relapse rate was defined as the percentage of subjects with relapse at 3, 6, 12, and 24 months post-transplant. It was summarized using Kaplan-Meier methods for subjects who received transplant. Time to relapse for subjects who received transplant was defined as the time interval from the date of transplant to the date of first recorded recurrent or progressive disease. Subjects with no recurrent or progressive disease recorded were censored at their last visit. The start date was the date of transplant. Analysis was performed on safety set for stage 2. Number of subjects analysed=subjects evaluable for this endpoint.

End point type	Secondary

End point timeframe:

At month 3, 6, 12, and 24 after transplant (up to a maximum duration of 2.5 years)

Notes:

[11] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	23		
Units: Percentage of subjects				
number (confidence interval 95%)				
Month 3 post-transplant	0.1 (0.015 to 0.527)	0.087 (0.022 to 0.305)		
Month 6 post-transplant	0.1 (0.015 to 0.527)	0.087 (0.022 to 0.305)		

Month 12 post-transplant	0.1 (0.015 to 0.527)	0.13 (0.044 to 0.352)		
Month 24 post-transplant	0.55 (0.266 to 0.873)	0.304 (0.158 to 0.534)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Post-Apheresis Relapse Rate

End point title	Stage 2: Post-Apheresis Relapse Rate[12]

End point description:

Relapse rate was defined as the percentage of subjects with relapse at 3, 6, 12, and 24 months post-apheresis. It was summarized using Kaplan-Meier methods for subjects who had apheresis. Time to relapse for subjects who received transplant or not was defined as time interval from the date of last apheresis to the date of the recorded recurrent or progressive disease. Subjects with no recurrent or progressive disease was censored at the last visit. The start date was the date of last apheresis. Analysis was performed on safety set for stage 2. 99999 represents data not calculated as very low number of subject had event (relapse).

End point type	Secondary

End point timeframe:

At month 3, 6, 12, and 24 after apheresis (up to maximum duration of 2.5 years)

Notes:

[12] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (confidence interval 95%)				
Month 3 post-apheresis	0.071 (0.01 to 0.409)	0 (-99999 to 99999)		
Month 6 post-apheresis	0.071 (0.01 to 0.409)	0.036 (0.005 to 0.228)		
Month 12 post-apheresis	0.071 (0.01 to 0.409)	0.071 (0.018 to 0.257)		
Month 24 post-apheresis	0.357 (0.167 to 0.657)	0.253 (0.129 to 0.459)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects With Secondary Malignancies

End point title	Stage 2: Percentage of Subjects With Secondary

End point description:

The occurrence of secondary malignancies during the 24 months after the last planned transplant performed in the 6-month period after last study apheresis (or 24 months after last dose of study mobilisation treatment for subjects who did not undergo transplant within 6 months after last study apheresis) was recorded for all subjects. Analysis was performed on safety set for stage 2. Here, 99999 represents data not calculated as no subject in both arms had any secondary malignancy.

End point type	Secondary

End point timeframe:

Up to 24 months post transplant period (up to a maximum duration of 2.5 years)

Notes:

[13] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (not applicable)	99999	99999		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects with Primary Graft Failure

End point title	Stage 2: Percentage of Subjects with Primary Graft Failure[14]

End point description:

Primary graft failure was defined by the criteria below in the absence of evidence of other causes such as progressive cancer, renal failure, chronic bleeding, severe infection, drug induced cytopaenias, or development of new haematological problems (nutritional or otherwise). Primary neutrophil graft failure was defined as the failure to achieve a sustained ANC of >=0.5*10^9/L (defined by 3 consecutive laboratory
values on 3 different days) or >=1.0*10^9/L for 1 day within 30 days post-transplant. Primary platelet graft failure was defined as the failure to achieve a sustained platelet count >=20*10^9/L (defined by at least 3 consecutive platelet laboratory values obtained over at least 7 days without transfusion) within 100 days post-transplant. Analysis was performed on safety set for stage 2. Number of subjects analysed=subjects
evaluable for this endpoint.

End point type	Secondary

End point timeframe:

Up to 100 days post-transplant or until disease recurrence or progression whichever occurred first (up to a maximum duration of 2.5 years)

Notes:

[14] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and

plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	23		
Units: percentage of subjects				
number (not applicable)				
Primary neutrophil graft failure	0	0		
Primary platelet graft failure	0	4.3		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Percentage of Subjects With Secondary Graft Failure

End point title	Stage 2: Percentage of Subjects With Secondary Graft

End point description:

Secondary graft failure was defined as confirmation of one of the following in the absence of evidence of other causes such as recurrence or progression of cancer, renal failure, chronic bleeding, severe infection, drug-induced cytopaenias, or development of new haematological problems (nutritional or otherwise). Neutrophils: after achieving neutrophil engraftment, there is a subsequent decrease in ANC such that the
ANC falls to <0.5*10^9/L for at least 7 days regardless of growth factor support. Platelets: after achieving primary platelet engraftment, there was a subsequent decrease in platelet counts below 10*10^9/L for 7 days (defined by at least 2 consecutive platelet laboratory values obtained over at least 7 days) or required sustained platelet transfusion support. Analysis was performed on safety set for stage 2. Number of subjects analysed=subjects evaluable for this endpoint.

End point type	Secondary

End point timeframe:

Up to 24 months post-transplant or until disease recurrence or progression whichever occurred first (up to a maximum duration of 2.5 years)

Notes:

[15] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	23		
Units: percentage of subjects				
number (not applicable)				
Secondary neutrophil graft failure	0	0		

Secondary platelet graft failure	0	4.3		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Median Time to Secondary Graft Failure

End point title	Stage 2: Median Time to Secondary Graft Failure[16]

End point description:

Median time to secondary graft failure was defined as the time interval from the date of successful engraftment to the date of documented secondary graft failure for those subjects who had achieved successful engraftment. Subjects without graft failure at the end of the follow-up period were to be censored at the last visit. Analysis was performed using Kaplan-Meier method. Analysis was performed on safety set for stage 2. Here, 99999 represents data not calculated as no subjects had event (secondary graft failure).

End point type	Secondary

End point timeframe:

From date of engraftment till the graft failure (up to a maximum duration of 2.5 years)

Notes:

[16] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: months				
number (confidence interval 95%)	99999 (99999 to 99999)	99999 (99999 to 99999)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Overall Survival at Month 3, 6, 12 and 24 Post- Transplant

End point title	Stage 2: Overall Survival at Month 3, 6, 12 and 24 Post-Transplant[17]

End point description:

Overall survival for subjects who received transplant(s) was defined as the time interval from the date of transplantation to the date of death. Subjects alive at the end of the follow-up period were to be censored at the last follow-up visit with known alive status. Kaplan-Meier method was used to estimate the confidence intervals (CIs), using a log transformation. Percentage of subjects with OS were estimated. Analysis was performed on safety set for stage 2. Number of subjects analysed=subjects

evaluable for this endpoint. Here, 99999 represents data (95% CI) not calculated as very low number of subjects had event.

End point type	Secondary

End point timeframe:

At 3, 6, 12 and 24 Months Post Transplant (up to a maximum duration of 2.5 years)

Notes:

[17] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	10	23		
Units: percentage of subjects				
number (confidence interval 95%)				
Month 3	1 (-99999 to 99999)	0.957 (0.729 to 0.994)		
Month 6	0.9 (0.473 to 0.985)	0.957 (0.729 to 0.994)		
Month 12	0.9 (0.473 to 0.985)	0.957 (0.729 to 0.994)		
Month 24	0.675 (0.291 to 0.882)	0.87 (0.648 to 0.956)		

Statistical analyses

No statistical analyses for this end point

Secondary: Stage 2: Overall Survival Rate for Subjects at 3, 6, 12 and 24 Months Post Apheresis

End point title	Stage 2: Overall Survival Rate for Subjects at 3, 6, 12 and 24 Months Post Apheresis[18]

End point description:

Overall survival for all subjects was calculated as the time interval from the date of last apheresis to the date of death. Subjects alive at the end of the follow-up period were to be censored at the last follow-up visit with known alive status. Kaplan-Meier method was used to estimate the CIs, using a log transformation Percentage of subjects with OS were estimated. Analysis was performed on safety set for stage 2. Here, 99999 represents data (95% CI) not calculated as very low number of subjects had event.

End point type	Secondary

End point timeframe:

At 3, 6, 12 and 24 Months Post Apheresis (up to a maximum duration of 2.5 years)

Notes:

[18] - The end point is not reporting statistics for all the arms in the baseline period. It is expected all the baseline period arms will be reported on when providing values for an end point on the baseline period.
Justification: The endpoint is reporting results only for stage 2 in which standard mobilization alone and plerixafor + standard mobilization was administered.

End point values	Stage 2: Standard Mobilization Regimen - G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	15	30		
Units: percentage of subjects				
number (confidence interval 95%)				
Month 3	1 (-99999 to 99999)	1 (-99999 to 99999)		
Month 6	1 (-99999 to 99999)	1 (-99999 to 99999)		
Month 12	0.929 (0.591 to 0.99)	0.964 (0.772 to 0.995)		
Month 24	0.766 (0.433 to 0.919)	0.893 (0.704 to 0.964)		

Statistical analyses

No statistical analyses for this end point

Adverse events information

Timeframe for reporting adverse events:

All Adverse Events (AEs) were collected from signature of the informed consent form up to the final visit (up to post-transplant follow up visit of 2 years) regardless of seriousness or relationship to investigational product.

Adverse event reporting additional description:

Reported AEs are TEAEs that is AEs that developed/worsened during the on treatment period (from the date of randomization until 30 days after the last dose of subject's study mobilization regimen, or until the first dose of their next anticancer therapy or pre-transplant myeloablative therapy or 2-year follow-up period).

Assessment type	Systematic

Dictionary used

Dictionary name	MedDRA
Dictionary version	20.0

Reporting groups

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years

Reporting group description:

Subjects aged 2-<6 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg /kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5

aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years

Reporting group description:

Subjects aged 6-<12 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 160 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years

Reporting group description:

Subjects aged 12-<18 years started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 320 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 2: Standard Mobilization Regimen- G-CSF

Reporting group description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Treatment G-CSF (alone or in combination with chemotherapy) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Reporting group title	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF

Reporting group description:

Subjects started with standard mobilization with G-CSF 10 mcg/kg/day in the morning, alone or in combination with chemotherapy. Once a "trigger point" of 7 CD34+ cells/mcL in peripheral blood was reached treatment with plerixafor was initiated. Plerixafor 240 mcg/kg was administered in the evening and the daily dose of G-CSF (as part of the standard mobilization regimen) the following morning approximately 1 hour before each apheresis. Treatment (plerixafor and G-CSF) was continued until a minimum of 2*10^6 CD34+ cells/kg were collected or for up to a maximum of 5 aphereses.

Serious adverse events	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years
Total subjects affected by serious adverse events			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
number of deaths (all causes)	0	0	0

number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Blood Stem Cell Harvest Failure			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Surgical and medical procedures			
Bone Marrow Harvest			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Nervous system disorders			
Hydrocephalus			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Blood and lymphatic system disorders			
Bone Marrow Failure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Febrile Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Leukopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Pancytopenia			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Gastrointestinal Inflammation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Stomatitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Respiratory, thoracic and mediastinal disorders			
Pulmonary Embolism			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Infections and infestations			
Abdominal Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Enterobacter Bacteraemia			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Hyponatraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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

Serious adverse events	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 3 (66.67%)	0 / 3 (0.00%)	1 / 3 (33.33%)
number of deaths (all causes)	1	0	1
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Blood Stem Cell Harvest Failure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Surgical and medical procedures			
Bone Marrow Harvest			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Nervous system disorders			
Hydrocephalus			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Blood and lymphatic system disorders			

Bone Marrow Failure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Febrile Neutropenia			
subjects affected / exposed	2 / 3 (66.67%)	0 / 3 (0.00%)	0 / 3 (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
Leukopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Pancytopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Gastrointestinal Inflammation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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

Stomatitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Respiratory, thoracic and mediastinal disorders			
Pulmonary Embolism			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Infections and infestations			
Abdominal Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Enterobacter Bacteraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Hyponatraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0

Serious adverse events	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 3 (66.67%)	0 / 3 (0.00%)	1 / 3 (33.33%)
number of deaths (all causes)	0	1	1
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			

Blood Stem Cell Harvest Failure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Surgical and medical procedures			
Bone Marrow Harvest			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Nervous system disorders			
Hydrocephalus			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Blood and lymphatic system disorders			
Bone Marrow Failure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Febrile Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences causally related to treatment / all	0 / 0	0 / 0	0 / 1
deaths causally related to treatment / all	0 / 0	0 / 0	0 / 0
Leukopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Neutropenia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Pancytopenia			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Gastrointestinal Inflammation			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Stomatitis			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Respiratory, thoracic and mediastinal disorders			
Pulmonary Embolism			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Infections and infestations			
Abdominal Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Enterobacter Bacteraemia			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (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
Hyponatraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (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

Serious adverse events	Stage 2: Standard Mobilization Regimen- G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF	
Total subjects affected by serious adverse events			
subjects affected / exposed	9 / 30 (30.00%)	4 / 15 (26.67%)	
number of deaths (all causes)	3	3	
number of deaths resulting from adverse events			
Injury, poisoning and procedural complications			
Blood Stem Cell Harvest Failure			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Surgical and medical procedures			
Bone Marrow Harvest			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Nervous system disorders			
Hydrocephalus			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Blood and lymphatic system disorders			

Bone Marrow Failure			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 1	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Febrile Neutropenia			
subjects affected / exposed	3 / 30 (10.00%)	2 / 15 (13.33%)	
occurrences causally related to treatment / all	0 / 3	0 / 2	
deaths causally related to treatment / all	0 / 0	0 / 0	
Leukopenia			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Neutropenia			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Pancytopenia			
subjects affected / exposed	2 / 30 (6.67%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
General disorders and administration site conditions			
Pyrexia			
subjects affected / exposed	2 / 30 (6.67%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 2	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal disorders			
Diarrhoea			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Gastrointestinal Inflammation			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Stomatitis			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Respiratory, thoracic and mediastinal disorders			
Pulmonary Embolism			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Infections and infestations			
Abdominal Infection			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Enterobacter Bacteraemia			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences causally related to treatment / all	0 / 0	0 / 1	
deaths causally related to treatment / all	0 / 0	0 / 0	
Metabolism and nutrition disorders			
Dehydration			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	
Hyponatraemia			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences causally related to treatment / all	0 / 0	0 / 0	
deaths causally related to treatment / all	0 / 0	0 / 0	

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Stage 1: Plerixafor 160 mcg /kg: 2-<6 years	Stage 1: Plerixafor 240 mcg /kg: 2-<6 years	Stage 1: Plerixafor 320 mcg /kg: 2-<6 years
Total subjects affected by non-serious adverse events			
subjects affected / exposed	2 / 3 (66.67%)	1 / 3 (33.33%)	2 / 3 (66.67%)

Vascular disorders			
Hyperaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypertension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypotension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Catheter Site Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Chest Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Extravasation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Fatigue			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Injection Site Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Malaise			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Mucosal Inflammation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pyrexia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	1	0	1
Immune system disorders			

Drug Hypersensitivity			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Increased Viscosity Of Upper Respiratory Secretion			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rhinorrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Psychiatric disorders			
Anxiety			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Investigations			
Alanine Aminotransferase Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Antithrombin Iii Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Aspartate Aminotransferase Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Blood Bicarbonate Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Echocardiogram Abnormal			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Platelet Count Decreased			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Weight Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Allergic Transfusion Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Back Injury			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Brain Contusion			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Infusion Related Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Procedural Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Thermal Burn			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Intracranial Venous Sinus Thrombosis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Seizure			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Febrile Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypofibrinogenaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Leukopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Thrombocytopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Ear and labyrinth disorders			
Deafness			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Optic Atrophy			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pupils Unequal			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Vision Blurred			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Gastrointestinal disorders			

Abdominal Distension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Abdominal Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Abdominal Pain Upper			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Constipation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Dental Caries			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Dyspepsia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Lip Dry			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Proctalgia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Stomatitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Vomiting			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rash Papular			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Urticaria			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Endocrine disorders			
Cushingoid			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Back Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pain In Extremity			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Infections and infestations			
Bronchitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Conjunctivitis Viral			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Device Related Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Enterobacter Infection			

subjects affected / exposed	0 / 3 (0.00%)	1 / 3 (33.33%)	0 / 3 (0.00%)
occurrences (all)	0	1	0
Escherichia Urinary Tract Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Febrile Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Otitis Media			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rhinitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Staphylococcal Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Viral Upper Respiratory Tract Infection			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Metabolism and nutrition disorders			
Decreased Appetite			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hyperglycaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypoalbuminaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypocalcaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypokalaemia			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypomagnesaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hyponatraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypophosphataemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Non-serious adverse events	Stage 1: Plerixafor 160 mcg /kg: 6-<12 years	Stage 1: Plerixafor 240 mcg /kg: 6-<12 years	Stage 1: Plerixafor 320 mcg /kg: 6-<12 years
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 3 (100.00%)	2 / 3 (66.67%)	2 / 3 (66.67%)
Vascular disorders			
Hyperaemia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Hypertension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypotension			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
General disorders and administration site conditions			
Catheter Site Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Chest Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Extravasation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Fatigue			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Injection Site Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Malaise			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Mucosal Inflammation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pyrexia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Immune system disorders			
Drug Hypersensitivity			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Increased Viscosity Of Upper Respiratory Secretion			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rhinorrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Psychiatric disorders			
Anxiety			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Investigations			
Alanine Aminotransferase Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Antithrombin Iii Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Aspartate Aminotransferase Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Blood Bicarbonate Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Echocardiogram Abnormal			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Platelet Count Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Weight Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Allergic Transfusion Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Back Injury			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Brain Contusion			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Infusion Related Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Procedural Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Thermal Burn			

subjects affected / exposed	0 / 3 (0.00%)	1 / 3 (33.33%)	0 / 3 (0.00%)
occurrences (all)	0	1	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Intracranial Venous Sinus Thrombosis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Seizure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Febrile Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypofibrinogenaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Leukopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Thrombocytopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Ear and labyrinth disorders			

Deafness			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Optic Atrophy			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pupils Unequal			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Vision Blurred			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Gastrointestinal disorders			
Abdominal Distension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Abdominal Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Abdominal Pain Upper			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Constipation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Dental Caries			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Dyspepsia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Lip Dry			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	1 / 3 (33.33%)	1 / 3 (33.33%)	0 / 3 (0.00%)
occurrences (all)	1	1	0
Proctalgia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Stomatitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Vomiting			
subjects affected / exposed	1 / 3 (33.33%)	1 / 3 (33.33%)	1 / 3 (33.33%)
occurrences (all)	1	1	1
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rash Papular			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Urticaria			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Endocrine disorders			
Cushingoid			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Back Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Pain In Extremity			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Infections and infestations			
Bronchitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Conjunctivitis Viral			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Device Related Infection			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Enterobacter Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Escherichia Urinary Tract Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Febrile Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Otitis Media			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rhinitis			
subjects affected / exposed	0 / 3 (0.00%)	1 / 3 (33.33%)	0 / 3 (0.00%)
occurrences (all)	0	1	0
Staphylococcal Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Viral Upper Respiratory Tract Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Metabolism and nutrition disorders			

Decreased Appetite			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hyperglycaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypoalbuminaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypocalcaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypokalaemia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Hypomagnesaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hyponatraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypophosphataemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Non-serious adverse events	Stage 1: Plerixafor 160 mcg /kg: 12-<18 years	Stage 1: Plerixafor 240 mcg/kg: 12-<18 years	Stage 1: Plerixafor 320 mcg/kg: 12-<18 years
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 3 (100.00%)	1 / 3 (33.33%)	1 / 3 (33.33%)
Vascular disorders			
Hyperaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypertension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypotension			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
General disorders and administration site conditions			
Catheter Site Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Chest Pain			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Extravasation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Fatigue			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Injection Site Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Malaise			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Mucosal Inflammation			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Pyrexia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Immune system disorders			
Drug Hypersensitivity			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Increased Viscosity Of Upper			

Respiratory Secretion			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rhinorrhoea			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Psychiatric disorders			
Anxiety			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Investigations			
Alanine Aminotransferase Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Antithrombin Iii Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Aspartate Aminotransferase Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Blood Bicarbonate Increased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Echocardiogram Abnormal			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Platelet Count Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Weight Decreased			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Injury, poisoning and procedural complications			
Allergic Transfusion Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Back Injury			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Brain Contusion			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Infusion Related Reaction			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Procedural Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Thermal Burn			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Cardiac disorders			
Tachycardia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Nervous system disorders			
Headache			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	2	0	3
Intracranial Venous Sinus Thrombosis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Seizure			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	1 / 3 (33.33%)
occurrences (all)	0	0	1
Febrile Neutropenia			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0

Hypofibrinogenaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Leukopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Neutropenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Thrombocytopenia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Ear and labyrinth disorders			
Deafness			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Eye disorders			
Optic Atrophy			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pupils Unequal			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Vision Blurred			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Gastrointestinal disorders			
Abdominal Distension			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Abdominal Pain			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Abdominal Pain Upper			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Constipation			

subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Dental Caries			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Diarrhoea			
subjects affected / exposed	2 / 3 (66.67%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	3	0	0
Dyspepsia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Lip Dry			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Nausea			
subjects affected / exposed	0 / 3 (0.00%)	1 / 3 (33.33%)	1 / 3 (33.33%)
occurrences (all)	0	1	1
Proctalgia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Stomatitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Vomiting			
subjects affected / exposed	2 / 3 (66.67%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	2	0	0
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	1 / 3 (33.33%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	1	0	0
Rash Papular			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Urticaria			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Renal and urinary disorders			
Dysuria			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Endocrine disorders			
Cushingoid			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Musculoskeletal and connective tissue disorders			
Back Pain			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Pain In Extremity			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Infections and infestations			
Bronchitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Conjunctivitis Viral			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Device Related Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Enterobacter Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Escherichia Urinary Tract Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Febrile Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Otitis Media			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Rhinitis			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Staphylococcal Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Viral Upper Respiratory Tract Infection			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Metabolism and nutrition disorders			
Decreased Appetite			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hyperglycaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypoalbuminaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypocalcaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypokalaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypomagnesaemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hyponatraemia			
subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0
Hypophosphataemia			

subjects affected / exposed	0 / 3 (0.00%)	0 / 3 (0.00%)	0 / 3 (0.00%)
occurrences (all)	0	0	0

Non-serious adverse events	Stage 2: Standard Mobilization Regimen- G-CSF	Stage 2: Plerixafor + Standard Mobilization Regimen -G-CSF	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	20 / 30 (66.67%)	10 / 15 (66.67%)	
Vascular disorders			
Hyperaemia			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Hypertension			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Hypotension			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
General disorders and administration site conditions			
Catheter Site Pain			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Chest Pain			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Extravasation			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Fatigue			
subjects affected / exposed	0 / 30 (0.00%)	3 / 15 (20.00%)	
occurrences (all)	0	4	
Injection Site Reaction			
subjects affected / exposed	2 / 30 (6.67%)	0 / 15 (0.00%)	
occurrences (all)	2	0	
Malaise			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	

Mucosal Inflammation			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Pyrexia			
subjects affected / exposed	1 / 30 (3.33%)	1 / 15 (6.67%)	
occurrences (all)	1	1	
Immune system disorders			
Drug Hypersensitivity			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Respiratory, thoracic and mediastinal disorders			
Cough			
subjects affected / exposed	2 / 30 (6.67%)	1 / 15 (6.67%)	
occurrences (all)	2	1	
Increased Viscosity Of Upper Respiratory Secretion			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Rhinorrhoea			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Psychiatric disorders			
Anxiety			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Investigations			
Alanine Aminotransferase Increased			
subjects affected / exposed	2 / 30 (6.67%)	2 / 15 (13.33%)	
occurrences (all)	2	2	
Antithrombin Iii Decreased			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Aspartate Aminotransferase Increased			
subjects affected / exposed	2 / 30 (6.67%)	1 / 15 (6.67%)	
occurrences (all)	2	1	
Blood Bicarbonate Increased			

subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Echocardiogram Abnormal			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Platelet Count Decreased			
subjects affected / exposed	6 / 30 (20.00%)	2 / 15 (13.33%)	
occurrences (all)	6	2	
Weight Decreased			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Injury, poisoning and procedural complications			
Allergic Transfusion Reaction			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Back Injury			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Brain Contusion			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Infusion Related Reaction			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Procedural Pain			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Thermal Burn			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Cardiac disorders			
Tachycardia			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Nervous system disorders			

Headache			
subjects affected / exposed	2 / 30 (6.67%)	0 / 15 (0.00%)	
occurrences (all)	2	0	
Intracranial Venous Sinus Thrombosis			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Seizure			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Blood and lymphatic system disorders			
Anaemia			
subjects affected / exposed	9 / 30 (30.00%)	3 / 15 (20.00%)	
occurrences (all)	9	3	
Febrile Neutropenia			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Hypofibrinogenaemia			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Leukopenia			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Neutropenia			
subjects affected / exposed	1 / 30 (3.33%)	1 / 15 (6.67%)	
occurrences (all)	1	1	
Thrombocytopenia			
subjects affected / exposed	1 / 30 (3.33%)	1 / 15 (6.67%)	
occurrences (all)	3	1	
Ear and labyrinth disorders			
Deafness			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Eye disorders			
Optic Atrophy			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	

Pupils Unequal			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Vision Blurred			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Gastrointestinal disorders			
Abdominal Distension			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Abdominal Pain			
subjects affected / exposed	3 / 30 (10.00%)	0 / 15 (0.00%)	
occurrences (all)	3	0	
Abdominal Pain Upper			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Constipation			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Dental Caries			
subjects affected / exposed	1 / 30 (3.33%)	1 / 15 (6.67%)	
occurrences (all)	1	1	
Diarrhoea			
subjects affected / exposed	3 / 30 (10.00%)	1 / 15 (6.67%)	
occurrences (all)	4	1	
Dyspepsia			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Lip Dry			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Nausea			
subjects affected / exposed	2 / 30 (6.67%)	3 / 15 (20.00%)	
occurrences (all)	3	4	
Proctalgia			

subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Stomatitis			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Vomiting			
subjects affected / exposed	3 / 30 (10.00%)	2 / 15 (13.33%)	
occurrences (all)	3	3	
Skin and subcutaneous tissue disorders			
Pruritus			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Rash Papular			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Urticaria			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Renal and urinary disorders			
Dysuria			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Endocrine disorders			
Cushingoid			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Musculoskeletal and connective tissue disorders			
Back Pain			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Pain In Extremity			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Infections and infestations			
Bronchitis			

subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Conjunctivitis Viral			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Device Related Infection			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Enterobacter Infection			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Escherichia Urinary Tract Infection			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Febrile Infection			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Otitis Media			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Rhinitis			
subjects affected / exposed	3 / 30 (10.00%)	1 / 15 (6.67%)	
occurrences (all)	3	1	
Staphylococcal Infection			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Viral Upper Respiratory Tract Infection			
subjects affected / exposed	0 / 30 (0.00%)	0 / 15 (0.00%)	
occurrences (all)	0	0	
Metabolism and nutrition disorders			
Decreased Appetite			
subjects affected / exposed	0 / 30 (0.00%)	1 / 15 (6.67%)	
occurrences (all)	0	1	
Hyperglycaemia			

subjects affected / exposed	1 / 30 (3.33%)	1 / 15 (6.67%)	
occurrences (all)	1	1	
Hypoalbuminaemia			
subjects affected / exposed	3 / 30 (10.00%)	1 / 15 (6.67%)	
occurrences (all)	3	1	
Hypocalcaemia			
subjects affected / exposed	2 / 30 (6.67%)	1 / 15 (6.67%)	
occurrences (all)	2	1	
Hypokalaemia			
subjects affected / exposed	1 / 30 (3.33%)	2 / 15 (13.33%)	
occurrences (all)	2	2	
Hypomagnesaemia			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Hyponatraemia			
subjects affected / exposed	1 / 30 (3.33%)	0 / 15 (0.00%)	
occurrences (all)	1	0	
Hypophosphataemia			
subjects affected / exposed	1 / 30 (3.33%)	1 / 15 (6.67%)	
occurrences (all)	1	1	

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
04 November 2013	Following amendments were made: - Dose from phase I (stage 1 of the study) was incorporated for phase II use (stage 2). - Sub-grouping for stratification was changed based on information from stage 1 population recruited: Ewing's and other STS; Lymphoma; Neuroblastoma; Other malignancies including brain tumours. - The age range for the study was modified to 1 to <18 years to allow potential inclusion of 1 to 2 year olds if feasible (no minimal number of 1-2 y old subjects required), as requested by the Paediatrics Development Committee of the EMA (PIP). - Modifications arising from discussions with the DMC and the Investigators following Stage 1 were incorporated, such as: • Timing of G CSF administration • Limited PK sampling timing • Selected Lab Value minimums for ANC and platelets. - Administrative details were updated from original Genzyme protocol to Sanofi standards and procedures (study management transferred to Sanofi).

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported