



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) | EMA-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.

| | |
|--|------------------------|
| 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: 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.

| | |
|--|------------------------|
| 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: 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.

| | |
|--|------------------------|
| 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: 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 $\geq 2 \times 10^6$ CD34+ cells/kg

| | |
|---|--|
| End point title | Stage 2: Median Number of Days of Apheresis Required to Reach $\geq 2 \times 10^6$ CD34+ cells/kg ^[3] |
| End point description: The number of days of apheresis to reach $\geq 2 \times 10^6$ 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 $\geq 2 \times 10^6$ 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 $\geq 0.5 \times 10^9/L$ (defined by 3 consecutive laboratory

values on 3 different days) or $\geq 1.0 \times 10^9/L$ for 1 day within 30 days post-transplant. Primary platelet graft failure was defined as the failure to achieve a sustained platelet count $\geq 20 \times 10^9/L$ (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 cytopenias, 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 $<0.5 \times 10^9/L$ 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 $10 \times 10^9/L$ 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

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.

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

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

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

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

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

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

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

| | | | |
|---|--------------------|--------------------|--------------------|
| subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 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 occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Rhinorrhoea subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Psychiatric disorders Anxiety subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Investigations Alanine Aminotransferase Increased subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Antithrombin Iii Decreased subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Aspartate Aminotransferase Increased subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Blood Bicarbonate Increased subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Echocardiogram Abnormal subjects affected / exposed occurrences (all) | 1 / 3 (33.33%) 1 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Platelet Count Decreased subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Weight Decreased subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Injury, poisoning and procedural complications Allergic Transfusion Reaction subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 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 occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Leukopenia subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Neutropenia subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Thrombocytopenia subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Ear and labyrinth disorders Deafness subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Eye disorders Optic Atrophy subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Pupils Unequal subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Vision Blurred subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Gastrointestinal disorders Abdominal Distension subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Abdominal Pain subjects affected / exposed occurrences (all) | 1 / 3 (33.33%) 1 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Abdominal Pain Upper subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 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 occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Endocrine disorders Cushingoid subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 |
| Musculoskeletal and connective tissue disorders Back Pain subjects affected / exposed occurrences (all) Pain In Extremity subjects affected / exposed occurrences (all) | 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 |
| Infections and infestations Bronchitis subjects affected / exposed occurrences (all) Conjunctivitis Viral subjects affected / exposed occurrences (all) Device Related Infection subjects affected / exposed occurrences (all) Enterobacter Infection subjects affected / exposed occurrences (all) Escherichia Urinary Tract Infection subjects affected / exposed occurrences (all) Febrile Infection subjects affected / exposed occurrences (all) Otitis Media | 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 | 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 0 / 3 (0.00%) 0 |

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

| | | | |
|---|----------------------|----------------------|--|
| subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Echocardiogram Abnormal subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 0 / 15 (0.00%) 0 | |
| Platelet Count Decreased subjects affected / exposed occurrences (all) | 6 / 30 (20.00%) 6 | 2 / 15 (13.33%) 2 | |
| Weight Decreased subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Injury, poisoning and procedural complications Allergic Transfusion Reaction subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Back Injury subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Brain Contusion subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Infusion Related Reaction subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Procedural Pain subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Thermal Burn subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 0 / 15 (0.00%) 0 | |
| Cardiac disorders Tachycardia subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 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 occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Vision Blurred subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 0 / 15 (0.00%) 0 | |
| Gastrointestinal disorders | | | |
| Abdominal Distension subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Abdominal Pain subjects affected / exposed occurrences (all) | 3 / 30 (10.00%) 3 | 0 / 15 (0.00%) 0 | |
| Abdominal Pain Upper subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Constipation subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Dental Caries subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 1 / 15 (6.67%) 1 | |
| Diarrhoea subjects affected / exposed occurrences (all) | 3 / 30 (10.00%) 4 | 1 / 15 (6.67%) 1 | |
| Dyspepsia subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Lip Dry subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Nausea subjects affected / exposed occurrences (all) | 2 / 30 (6.67%) 3 | 3 / 15 (20.00%) 4 | |
| Proctalgia | | | |

| | | | |
|--|----------------------|----------------------|--|
| subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Stomatitis subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Vomiting subjects affected / exposed occurrences (all) | 3 / 30 (10.00%) 3 | 2 / 15 (13.33%) 3 | |
| Skin and subcutaneous tissue disorders Pruritus subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 0 / 15 (0.00%) 0 | |
| Rash Papular subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Urticaria subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Renal and urinary disorders Dysuria subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Endocrine disorders Cushingoid subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 0 | |
| Musculoskeletal and connective tissue disorders Back Pain subjects affected / exposed occurrences (all) | 0 / 30 (0.00%) 0 | 1 / 15 (6.67%) 1 | |
| Pain In Extremity subjects affected / exposed occurrences (all) | 1 / 30 (3.33%) 1 | 0 / 15 (0.00%) 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 | <p>Following amendments were made:</p> <ul style="list-style-type: none">- 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:<ul style="list-style-type: none">• 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