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

Published: 06-01-2014
Last updated: 23-04-2024

In this study, plerixafor, the study drug, will be given in addition to GCSF to see if it works the same way in children as in adults.

Ethical review	Approved WMO
Status	Recruitment stopped
Health condition type	Other condition
Study type	Interventional

Summary

ID

NL-OMON40176

Source
ToetsingOnline

Brief title

MOZAIC

Condition

- Other condition

Synonym
solid tumor

Health condition

solide tumoren

Research involving
Human

Sponsors and support

Primary sponsor: Sanofi-aventis
Source(s) of monetary or material Support: Genzyme

Intervention

Keyword: open-label, paediatric patients, plerixafor, solid tumor

Outcome measures

Primary outcome

The primary efficacy endpoint will be the difference between the 2 treatment

arms in the proportion of patients achieving at least a doubling of peripheral

blood CD34+ count from the morning of the day preceding the first apheresis day

to the morning prior to apheresis.

Secondary outcome

• Number of days of apheresis required to reach >=2 × 106 CD34+ cells/kg

• CD34+ yield for each apheresis

• Total CD34+ yield

• Percentage of patients proceeding to transplant

• Percentage of patients successfully engrafting

• Percentage of patients with durable engraftment at 3, 6, 12 and 24 months

post-transplant

Study description

Background summary

The purpose of this study is to find out if the drug, plerixafor, is safe and effective in children.

Plerixafor is a drug that has already been tested and approved for use in adults. It is used to help collect blood stem cells for transplantation (after chemotherapy) in patients with cancer. Blood stem cells are the original cells from which all of the other blood cells develop. They are made in the bone marrow (the spongy structure in the centre of large bones).

Study objective

In this study, plerixafor, the study drug, will be given in addition to GCSF to see if it works the same way in children as in adults.

Study design

This is a open-label, comparative, randomised trial

Intervention

Group 1: standard therapy with GCSF and plerixafor
Group 2: standard therapy with GCSF

In a ratio of 2:1

Study burden and risks

The side effects listed below are seen in adults taking part in clinical trials of plerixafor. It is likely that similar side effects may be seen in children but we cannot be certain.

Very common side effects seen in more than 1 in 10 patients:
Diarrhoea, nausea (feeling sick), injection site redness or irritation

Common side effects seen in between 1 and 10 of every 100 patients:
Headache, dizziness, feeling tired or unwell, difficulty in sleeping, flatulence, constipation, indigestion, vomiting, stomach symptoms such as pain, swelling or discomfort, dry mouth, numbness around the mouth, sweating, generalized redness of the skin, joint pains, pains in muscles and bones.

Uncommon side effects seen in between 1 and 10 of every 1,000 patients:
Allergic reactions such as skin rash, swelling around the eyes, shortness of breath and loss of consciousness.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Children (2-11 years)

Inclusion criteria

1. Age 1 to < 18 years
2. Ewing*s sarcoma, soft tissue sarcoma, lymphoma, neuroblastoma, or other malignancy including brain tumours (excluding any form of leukaemia) requiring treatment with high dose chemotherapy and autologous transplant as rescue therapy
3. Eligible for autologous transplantation
4. Recovered from all acute significant toxic effects of prior chemotherapy
5. Adequate performance status
- for patients >=16 years of age, defined as Karnofsky score >60
- for patients <16 years of age, defined as Lansky score > 60
6. Absolute neutrophil count >0.75 × 10P9/L
7. Platelet count > 50 × 10P9/L
8. Calculated creatinine clearance (using the Schwartz method):
- during study Stage 2, >;60 mL/min/1.73mP2
9. Liver functions < 3 × upper limit of normal
10. The patient and/or their parent/legal guardian is willing and able to provide signed informed consent

Exclusion criteria

1. Any form of leukaemia
2. A co-morbid condition, such as ventricular arrhythmias, which, in the view of the Investigator, renders the patient at high-risk from treatment complications
3. Previous stem cell transplantation
4. Patients with persistent high percentage marrow involvement prior to mobilisation will be prohibited.

Study design

Design

Study phase:	2
Study type:	Interventional
Intervention model:	Parallel
Allocation:	Randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Treatment

Recruitment

NL

Recruitment status:	Recruitment stopped
Start date (anticipated):	10-06-2014
Enrollment:	3
Type:	Actual

Medical products/devices used

Product type:	Medicine
Brand name:	mozobil
Generic name:	plerixafor
Registration:	Yes - NL outside intended use

Ethics review

Approved WMO Date:	06-01-2014
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	14-04-2014
Application type:	First submission
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	24-10-2014
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)
Approved WMO Date:	29-01-2015
Application type:	Amendment
Review commission:	METC Erasmus MC, Universitair Medisch Centrum Rotterdam (Rotterdam)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

Other (possibly less up-to-date) registrations in this register

No registrations found.

In other registers

Register	ID
EudraCT	EUCTR2010-019340-40-NL
ClinicalTrials.gov	NCT01288573
CCMO	NL47333.078.13