Long-term integrity of hematopoiesis in pediatric stem cell transplantation survivors

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We aim to determine the long-term integrity of hematopoiesis in pediatric and young adult HSCT survivors.

Ethical review	Approved WMO
Status	Recruiting
Health condition type	Anaemias nonhaemolytic and marrow depression
Study type	Observational invasive

Summary

ID

NL-OMON54142

Source
ToetsingOnline

Brief title
Long-term hematopoiesis in transplantation survivors (Long-term HIT)

Condition

- Anaemias nonhaemolytic and marrow depression
- Immune disorders NEC

Synonym
Hematopoietic stem cell transplantation; bone marrow transplantation

Research involving
Human

Sponsors and support

Primary sponsor: Prinses Máxima Centrum voor Kinderoncologie
Source(s) of monetary or material Support: Ministerie van OC&W,Stichting KIKA

Intervention

Keyword: Cytopenia, Follow-up, Hematologic dysfunction, Hematopoietic stem cell transplantation

Outcome measures

Primary outcome

The primary aim of this study is to determine the percentage of long-term HSCT survivors with clonal hematopoiesis (CH), and to identify clinical determinants that are associated with CH.

Secondary outcome

We also aim to determine the percentage of long-term HSCT survivors with the following other types of hematopoietic dysfunction: (1) cytopenia; (2) loss of donor chimerism; (3) bone marrow failure; (4) myelodysplasia; and (5) donor-cell leukemia. Finally, we will perform in-depth molecular studies to analyze hematopoietic integrity upon HSCT, including genomic and flow cytometric analyses.

Study description

Background summary

Hematopoietic stem cell transplantation (HSCT) is a last-resort, curative therapy for patients suffering from various, otherwise lethal, diseases. Due to improved treatment strategies, the number of HSCT survivors and their life expectancy continue to increase. In pediatric and young adult HSCT survivors, the donor stem cells may have to live far beyond the normal human life span. It remains unknown whether transplanted HSCs can sustain life-long healthy blood production in these recipients. In the current project, we hypothesize that HSCT compromises HSC longevity and predisposes to (age-related) hematopoietic dysfunction in the recipient.

Study objective

We aim to determine the long-term integrity of hematopoiesis in pediatric and young adult HSCT survivors.

Study design

This is an observational, explorative study, embedded in the HSCT follow-up outpatient clinics of the Princess Máxima Center and UMC Utrecht. The study visit will be combined with a clinical visit, as part of regular post-HSCT follow-up. All participants will undergo clinical assessment of HSCT-related long-term effects by a trained physician, including measurement of differential blood counts, as part of routine clinical care. For this study, we will use these clinical data, and collect an additional blood sample and buccal swab for in-depth assessment of hematopoietic integrity after HSCT.

Study burden and risks

This study requires pediatric and young adult recipients, as their post-transplant survival exceeds that of older adult HSCT recipients by several decades, posing unique challenges on the integrity and longevity of the engrafted HSCs. In addition, as clinical HSCT regimens differ between children and adults (e.g. use of irradiation, chemotherapy dose), results obtained in adult HSCT recipients cannot be translated directly to children.
This study will provide unique insights into the long-term effects of HSCT on hematopoiesis, while posing minimal risks to the participants. Results from this study may contribute to improved, risk-adjusted clinical follow-up. Moreover, understanding of the clinical determinants of long-term HSCT-related hematopoietic dysfunction may result in better HSCT protocols.

Contacts

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Trial sites

Listed location countries

Netherlands

Eligibility criteria

Age
Adolescents (12-15 years)
Adolescents (16-17 years)
Adults (18-64 years)
Children (2-11 years)

Inclusion criteria

Underwent allogeneic HSCT at age <40 yrs
A minimum survival of 5 years after HSCT.

Exclusion criteria

Failure of the HSCT recipient, donor and/or their legal representatives to understand the patient information and informed consent form (either due to intellectual disability or to language problems).

Recipients of a *NiCord* HSCT. NiCord is a clinical trial on the safety and efficacy of transplantation of ex vivo expanded cord blood HSCs. As outcome measures of our study overlap with the outcome of this trial, NiCord recipients will be excluded.

Study design

Design

Study type: Observational invasive

Masking:	Open (masking not used)
Control:	Uncontrolled

Primary purpose:	Basic science

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	12-01-2022
Enrollment:	375
Type:	Actual

Ethics review

Approved WMO Date:	10-09-2021
Application type:	First submission
Review commission:	METC NedMec
Approved WMO Date:	01-04-2022
Application type:	Amendment
Review commission:	METC NedMec
Approved WMO Date:	11-05-2023
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)
Approved WMO Date:	25-06-2024
Application type:	Amendment
Review commission:	METC Universitair Medisch Centrum Utrecht (Utrecht)

Study registrations

Followed up by the following (possibly more current) registration

No registrations found.

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

ID: 20624
Source: Nationaal Trial Register
Title:

In other registers

Register	ID
Other	Netherlands Trial Register NL9587
CCMO	NL77721.041.21