Late Effects and Follow up after pediatric hematological stem cell transplantation and cell therapy (LEEF)

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Better knowledge and awareness of Late Effects after pediatric HSCT for non-malignant diseases will lead to optimal screening procedures after HSCT that may eventually contribute to reduce transplant-related long term morbidity and mortality and...

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Observational non invasive

Summary

ID

NL-OMON20380

Source
Nationaal Trial Register

Brief title
LEEF

Health condition

Inborn errors of immunity, hemoglobinopathies, and bone marrow failure.

Sponsors and support

Primary sponsor: None
Source(s) of monetary or material Support: Leiden University Medical Center

Intervention

Outcome measures

Primary outcome

1. To investigate the late effects e.g. organ function, graft function, growth, psychosocial and neurocognitive development, dental abnormalities, involved health care providers, socioeconomic and demographic characteristics of children and adults after pediatric HSCT for non-malignant diseases. 2. To identify biological, demographic and psychological and therapeutic determinants for morbidity, mortality and treatment outcome in children and adults with a pediatric HSCT for non-malignant diseases. 3. To evaluate and improve aspects of value-based healthcare organization and other (perceived) care aspects in children and adults with a pediatric HSCT for non-malignant diseases by measurement of patient and treatment characteristics, patient-reported outcomes, patient and healthcare providers reported experiences, perceived patient-centeredness of care, health care use, costs, and to perform analyses of associations between these factors and health care outcome.

Secondary outcome

Not applicable

Study description

Background summary

This retrospective and prospective observational cohort study aims to determine the Late Effects of allogeneic stem cell transplantation (HSCT) at pediatric age in the Netherlands for non-malignant diseases and to identify factors that contribute to these Late Effects. These factors include: biological, sociodemographic, psychological and clinical determinants. Clinical data will be combined with patient/caregivers reported outcome measures and patient/caregivers/healthcare providers reported experience measures. Data will be collected retrospectively and prospectively at participants' yearly regular clinic visits for routine follow-up.

Study objective

Better knowledge and awareness of Late Effects after pediatric HSCT for non-malignant diseases will lead to optimal screening procedures after HSCT that may eventually contribute to reduce transplant-related long term morbidity and mortality and improve quality of life. Next, with emerging therapies (e.g. gene therapy) in the near future, structured insight into the (late) effects of these stem cell therapies is essential for evaluation of its effects.

Study design

Healthcare outcome measures will be collected retrospectively and prospectively at participants' annual regular clinic visits as part of standard care.

Intervention

NA

Contacts

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Eligibility criteria

Inclusion criteria

In order to be eligible to participate in this study, a subject must meet all of the following criteria: • >2 years after pediatric HSCT for non-malignant diseases • Written informed consent by the patient or legal guardians, and pediatric consent when indicated

Exclusion criteria

A potential subject who meets any of the following criteria will be excluded from participation in this study: • Any medical or social reasons, which obstruct or inhibit study participation according to the treating physician; • Patient or legal guardians unable or unwilling to give consent, or lack of pediatric consent when indicated.

Study design

Design

Study type:	Observational non invasive
Intervention model:	Other
Allocation:	Non controlled trial

Masking:	Open (masking not used)
Control:	N/A , unknown

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	17-12-2020
Enrollment:	250
Type:	Anticipated

IPD sharing statement

Plan to share IPD: Undecided

Ethics review

Positive opinion

Date:	14-12-2020
Application type:	First submission

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
NTR-new	NL9112
CCMO	NL20.181

Study results