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...

Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Observationeel onderzoek, zonder invasieve metingen

Samenvatting

ID

NL-OMON20380

Bron
Nationaal Trial Register

Verkorte titel
LEEF

Aandoening

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

Ondersteuning

Primaire sponsor: None
Overige ondersteuning: Leiden University Medical Center

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

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.

Toelichting onderzoek

Achtergrond van het onderzoek

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.

Doel van het onderzoek

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.

Onderzoeksopzet

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

Onderzoeksproduct en/of interventie

NA

Contactpersonen

Publiek

LUMC
Anne de Pagter

Wetenschappelijk

LUMC
Anne de Pagter

Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

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

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

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.

Onderzoeksopzet

Opzet

Type:	Observationeel onderzoek, zonder invasieve metingen
Onderzoeksmodel:	Anders
Toewijzing:	N.v.t. / één studie arm
Blindering:	Open / niet geblindeerd
Controle:	N.v.t. / onbekend

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	17-12-2020
Aantal proefpersonen:	250
Type:	Verwachte startdatum

Voornemen beschikbaar stellen Individuele Patiënten Data (IPD)

Wordt de data na het onderzoek gedeeld: Nog niet bepaald

Ethische beoordeling

Positief advies

Datum:	14-12-2020
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL9112
CCMO	NL20.181

Resultaten