Health related quality of life (HRQoL) in childhood survivors of refractory GvHD treated with mesenchymal stromal cell infusions - a multi-center study

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Ethical review	Approved WMO
Status	Pending
Health condition type	Other condition
Study type	Observational non invasive

Summary

ID

NL-OMON32001

Source
ToetsingOnline

Brief title
HRQoL after MSC treatment

Condition

- Other condition
- Leukaemias
- Immunodeficiency syndromes

Synonym
bone marrow transplantations, malignant diseases

Health condition

Beenmergtransplantatie

Research involving
Human

Sponsors and support

Primary sponsor: Academisch Medisch Centrum
Source(s) of monetary or material Support: Ministerie van OC&W

Intervention

Keyword: GVHD, health related quality of life, MSC, pediatric

Outcome measures

Primary outcome

Health related quality of life, measured by the PEDS QL and clinical status

assessment.

Secondary outcome

not applicable.

Study description

Background summary

Refractory GvHD and mesenchymal stromal cell treatment
Hematopoietic stem cell transplantation (HSCT) has been a successful therapy in use since the 1960*s and a proven cure for patients suffering from hematological disorders as well as immune deficiencies and metabolic disorders. For many of these children stem cell transplantation is the only curative option.
Despite advances in pre-transplant immune suppression and donor HLA typing methods (and thus donor selection), acute (a) GvHD remains a significant cause of transplant related mortality and morbidity following allogeneic HSCT.
The initial management of aGvHD comprises of steroid treatment. This may be combined with CSA, or tacrolimus. The majority of centers utilize methyl prednisolone at 2.0 mg/kg/day. Approximately 50% of patients will remit or improve with this treatment but the remainder requires second-line treatment, which to date remains unsatisfactory.
There is presently no consensus as to salvage treatment in steroid refractory aGvHD. Acute GvHD is considered steroid refractory when there is no response

to methyl prednisolone at 2.0 mg/kg/day for one week, or when there is progressive disease at 72 hours with this dose.
Numerous agents have been reported as second line treatment and continue to be evaluated. Whatever there initial effects, they have not fulfilled their expectations and have had little impact on overall survival, which remains dismal.
Recently, the infusion of third party MSC's has been described which effectively eradicated steroid refractory GvHD that had failed all other attempts at treatment. The hypothesis of action is that MSC's demonstrate powerful immune-modulatory functions that can effectively down-regulate T cells and thus diminish or eradicate GvHD.
A multi center treatment of steroid refractory GvHD with MSC infusions has recently been reported with response rates of 70% and overall survival in children in the region of 40%.

Study objective

We propose to investigate the health related quality of life (HRQoL) in childhood survivors of refractory GvHD who have been treated with mesenchymal stem cell transfusions. This will be a multi center study involving the Division of Clinical Immunology and Centre for Allogeneic Stem Cell Transplantation, Karolinska Institute, Huddinge University, Stockholm, Sweden, the Department of Pediatric Hematology Oncology, Fondazione IRCCS Policlinico S. Matteo, University of Pavia, Italy, the Department of Paediatric Haematology and Oncology, IRCSS Giannina Gaslini, Genova, Italy and Department of Pediatric Stem Cell Transplantation, Leiden University Medical Centre, Leiden, the Netherlands. Participating centers have agreed to collaborate once ethical committee approval has been obtained by the organizing center, i.e. LUMC.

Study design

I Prospective, repeated measurements.
II. Retrospective.

Study burden and risks

not applicable

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

Patients who have undergone treatment with MSC*s for steroid refractory acute GvHD and are at least one month post infusion
Anticipated life expectancy > 1 month.
Signed informed consent by the patient and/or parent(s) or legal guardian(s).
Aged between 2-18 years

Exclusion criteria

Expected life expectancy < 1 month

Study design

Design

Study type:	Observational non invasive
Intervention model:	Other
Allocation:	Non-randomized controlled trial
Masking:	Open (masking not used)
Control:	Active
Primary purpose:	Other

Recruitment

NL

Recruitment status:	Pending
Start date (anticipated):	01-01-2008
Enrollment:	10
Type:	Anticipated

Ethics review

Approved WMO

Application type:	First submission
Review commission:	METC Leids Universitair Medisch Centrum (Leiden)

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
CCMO	NL20408.058.07