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Mesenchymal stromal cells for the treatment of acute graft versus host disease

Submission date
12/07/2017

Recruitment status
No longer recruiting

Registration date
18/08/2017

Overall study status
Completed

Last Edited
06/06/2025

Condition category
Injury, Occupational Diseases, Poisoning

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

Plain English summary of protocol

Background and study aims
Acute graft-versus-host disease (aGVHD) is a possible complication of a bone marrow stem cell transplant from another person (allogeneic hematopoietic stem cell transplantation). It is caused by the donated immune cells reacting against the host tissues. It is potentially lethal and around half of stem cell recipients develop it. Acute GVHD typically occurs in the first 100 days after transplant and affects three organs: skin, liver and gastrointestinal (digestive) tract. The first treatment is to use glucocorticoid drugs but half of the patients do not respond to treatment (steroid refractory). The prognosis of patients who develop acute GVHD and do not respond to treatment is dismal. Numerous strategies to treat steroid-refractory aGVHD with a second treatment have been tested, but none have shown an effect. Therefore, there is no established second-line treatment for steroid-refractory acute GvHD. Mesenchymal stromal cells (MSCs) can decrease the activity of most cells of the immune system. The aim of this study is to assess the effectiveness of MSC treatment for steroid-refractory aGVHD.

Who can participate?
Patients aged 18 and older with steroid-refractory aGVHD after hematopoietic stem cell transplantation

What does the study involve?
All participants are treated with mesenchymal stromal cells. Blood samples are taken during treatment and at follow ups. Patients are followed up to assess their response to treatment, relapse and survival rates.

What are the possible benefits and risks of participating?
There are no benefits or risks for participants, but the results could be used to improve the treatment of patients with aGVHD in the future.

Where is the study run from?
Vilnius University Hospital Santaros Klinikos (Lithuania)

When is the study starting and how long is it expected to run for?
October 2013 to December 2023

Who is funding the study?
Hematology and Oncology Research Association of Lithuania

Who is the main contact?
Mr Adomas Bukauskas

Contact information

Type(s)
Scientific

Contact name
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Additional identifiers

Clinical Trials Information System (CTIS)
Nil known

ClinicalTrials.gov (NCT)
Nil known

Protocol serial number
TPSL-LTU-2015

Study information

Scientific Title
Mesenchymal stromal cells in patients with steroid refractory acute graft versus host disease after allogeneic hematopoietic stem cell transplantation or donor lymphocyte infusion

Study objectives
Mesenchymal stromal cells have been shown to have immunosuppressive effects. Through various mechanisms mesenchymal stromal cells exert an effect on most cells of the immune system and inhibit proliferation, activation, and cytokine release. Donor T cell activation and inflammatory cytokines play a major role in acute graft versus host disease (aGVHD) pathogenesis. The trialists hypothesize that by immunomodulatory properties mesenchymal stromal cells will be effective in controlling steroid refractory aGVHD and will prolong overall survival.

Ethics approval required
Old ethics approval format

Ethics approval(s)
Vilnius regional Bioethics Committee, 03/11/2015, ref: 1582000-15-814-326

Study design
Observational prospective non-interventional single-center study

Primary study design
Observational

Study type(s)
Treatment

Health condition(s) or problem(s) studied
Steroid refractory acute graft versus host disease after hematopoietic stem cell transplantation or donor lymphocyte infusion

Interventions
Patients with steroid refractory acute graft versus host disease were treated with mesenchymal stromal cells as compassionate use medicinal product (standard of care). Doses have not been predetermined in the protocol and follow standard practice. Additionally, blood samples were taken during treatment phase and at follow ups.

Intervention Type
Biological/Vaccine

Phase
Not Applicable

Drug/device/biological/vaccine name(s)
Mesenchymal stromal cells

Primary outcome(s)
Overall response rate (ORR) to mesenchymal stromal cells, defined as complete response (CR) or partial response (PR) of acute graft versus host disease, measured at D7, D14, D21, D28, D35, D42, D49, D56, 3 months

Key secondary outcome(s))
Measured at D7, D14, D21, D28, D35, D42, D49, D56, 3 months, 6 months, 9 months, 12 months, 18 months, 24 months and each year thereafter:
1. Overall survival (OS), defined as time from the start of treatment until death from any cause
2. Event free survival, defined as time from the start of treatment until the following events (whichever occurs first): death, hematologic malignancy relapse, no PR/CR by 3 months since the study entry, aGVHD relapse after PR/CR requiring next line treatment, progression to extensive chronic GVHD

Completion date
31/12/2023

Eligibility

Key inclusion criteria
1. Steroid refractory acute graft versus host disease after hematopoietic stem cell transplantation
2. 18 years old and older
3. Signed informed consent form

Participant type(s)
Patient

Healthy volunteers allowed
No

Age group
Adult

Lower age limit
18 years

Sex
All

Total final enrolment
93

Key exclusion criteria
Does not meet inclusion criteria

Date of first enrolment
09/10/2013

Date of final enrolment
10/04/2017

Locations

Countries of recruitment
Lithuania

Study participating centre
Vilnius University Hospital Santaros Klinikos
Santariskiu 2
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LT-08406

Sponsor information

Organisation
Hematology and Oncology Research Association of Lithuania (Hematologijos ir onkologijos tyrėjų asociacija LTU)

Funder(s)

Funder type
Research organisation

Funder Name
Hematology and Oncology Research Association of Lithuania (Hematologijos ir onkologijos tyrėjų asociacija LTU)

Results and Publications

Individual participant data (IPD) sharing plan
The data sharing plans for the current study are unknown and will be made available at a later date.

IPD sharing plan summary
Data sharing statement to be made available at a later date

Study outputs

Output type	Details	Date created	Date added	Peer reviewed?	Patient-facing?
Results article		13/05/2025	06/06/2025	Yes	No
Participant information sheet	Participant information sheet	11/11/2025	11/11/2025	No	Yes