

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

Submission date 12/07/2017	Recruitment status No longer recruiting	<input type="checkbox"/> Prospectively registered
		<input type="checkbox"/> Protocol
Registration date 18/08/2017	Overall study status Completed	<input type="checkbox"/> Statistical analysis plan
		<input checked="" type="checkbox"/> Results
Last Edited 06/06/2025	Condition category Injury, Occupational Diseases, Poisoning	<input type="checkbox"/> 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
Mr Adomas Bukauskas

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Additional identifiers

EudraCT/CTIS number
Nil known

IRAS number

ClinicalTrials.gov number
Nil known

Secondary identifying numbers
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

Secondary study design

Longitudinal study

Study setting(s)

Hospital

Study type(s)

Treatment

Participant information sheet

Not available in web format, please use the contact details to request a patient information sheet

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 measure

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

Secondary outcome measures

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

Overall study start date

09/10/2013

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

Age group

Adult

Lower age limit

18 Years

Sex

Both

Target number of participants

45

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

Vilnius

Lithuania

LT-08406

Sponsor information

Organisation

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

Sponsor details

Savickio 4

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Sponsor type

Research organisation

Funder(s)

Funder type

Research organisation

Funder Name

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

Results and Publications

Publication and dissemination plan

Planned publication in a peer-reviewed journal

Intention to publish date

01/06/2025

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