

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

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

**ORCID ID**  
<https://orcid.org/0000-0001-5857-4638>

**Contact details**  
Seliu 29-41  
Vilnius  
Lithuania  
LT-08125

## Additional identifiers

**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

## Primary study design

Observational

## Study design

Observational prospective non-interventional single-center study

## 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

Vilnius

Lithuania

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?
<a href="#">Results article</a>		13/05/2025	06/06/2025	Yes	No