Autologous Stem cell Transplantation International Scleroderma ('ASTIS') Trial.

No registrations found.

Ethical review	Positive opinion
Status	Recruiting
Health condition type	-
Study type	Interventional

Summary

ID

NL-OMON23346

Source
NTR

Brief title
ASTIS-trial

Health condition

Systemic sclerosis.

Sponsors and support

Primary sponsor: EBMT/EULAR Working Party Autoimmune Diseases.
Source(s) of monetary or material Support: EULAR; Amgen Europe; Sangstat; Horton Foundation (Switzerland).

Intervention

Outcome measures

Primary outcome

The primary endpoint is event-free survival defined as the time in days from the day of randomization until the occurrence of death or the development of persistent major organ failure (heart, lung, kidney) during the study period of 2 years.

Secondary outcome

Key secondary outcomes include: treatment related mortality, treatment toxicity, and progression-free survival, defined as the time in days since the day of randomization until death or predefined changes in skin score, organ function, body weight, functional disability from baseline has been documented at two consecutive 3-month evaluations within the study period of 2 years.

Study description

Background summary

This multicenter prospective randomized controlled phase III study will compare efficacy and safety of high dose immunoablation and autologous hematopoietic stem cell transplantation (HSCT) (considered the investigational treatment), versus monthly intravenous pulse-therapy cyclophosphamide (considered the control treatment) in selected patients with diffuse systemic sclerosis at risk for premature mortality.

The primary endpoint is event-free survival defined as the time in days from the day of randomization until the occurrence of death or the development of persistent major organ failure (heart, lung, kidney) during the study period of 2 years. It is intended to enrol 200 patients and to have an annual follow up of each patient for at least 5 years.

Study objective

It is postulated that the investigational treatment has superior efficacy based on observations of longterm remissions in a number of patients, although this has to be balanced against potentially higher toxicity.

Intervention

This multicenter prospective randomized controlled phase III study compares efficacy and safety of high dose immunoablation and autologous hematopoietic stem cell transplantation (HSCT) (considered the investigational treatment), versus monthly intravenous pulse-therapy cyclophosphamide (considered the control treatment).
The investigational treatment arm comprises the following consecutive steps: mobilization of hematopoietic stem cells with i.v. cyclophosphamide (2x2 gr/m2) and filgrastim (10 mg/kg/day), leukapheresis and selection of CD34+ stem cells, conditioning with i.v. cyclophosphamide (200 mg/kg) and rbATG (7.5 mg/kg), followed by HSCT.
The procedures are normally completed within 3-4 months after randomization. The control treatment arm consists of 12 consecutive monthly i.v. pulses cyclophosphamide (750 mg/m2).

Contacts

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Eligibility criteria

Inclusion criteria

Patients with diffuse systemic sclerosis, aged 16-65 yrs, and:

1. Disease duration 4 years or less, plus evidence of heart, lung or kidney involvement, plus skin score 15 or more, or:

2. Disease duration 2 years or less, plus evidence of an acute phase reaction in blood, plus skin score 20 or more.

Exclusion criteria

Patients with concomitant severe disease, extensive pretreatment according to predefined criteria with cyclophosphamide are excluded.

Study design

Design

Study type:	Interventional
Intervention model:	Parallel
Masking:	Open (masking not used)
Control:	Active

Recruitment

NL

Recruitment status:	Recruiting
Start date (anticipated):	22-03-2001
Enrollment:	200
Type:	Anticipated

Ethics review

Positive opinion

Date:	11-09-2005
Application type:	First submission

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
NTR-new	NL300
NTR-old	NTR338

Register	ID
Other	: N/A
ISRCTN	ISRCTN54371254

Study results

Summary results

van Laar JM, Farge D, Tyndall A, on behalf of the EBMT/EULAR Scleroderma Study Group. Stem cell transplantation in systemic sclerosis: hope on the horizon. Ann Rheum Dis 2005, in press.