

Autologous Stem cell Transplantation International Scleroderma (ASTIS) trial

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| Submission date 21/09/2005 | Recruitment status No longer recruiting | <input type="checkbox"/> Prospectively registered |
| Registration date 19/10/2005 | Overall study status Completed | <input checked="" type="checkbox"/> Protocol |
| Last Edited 25/07/2014 | Condition category Musculoskeletal Diseases | <input type="checkbox"/> Statistical analysis plan |
| | | <input checked="" type="checkbox"/> Results |
| | | <input type="checkbox"/> Individual participant data |

Plain English summary of protocol
Not provided at time of registration

Contact information

Type(s)
Scientific

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Additional identifiers

Protocol serial number
NTR338

Study information

Scientific Title
High dose immunoablation and autologous haematopoietic stem cell transplantation versus monthly intravenous pulse therapy

Acronym

ASTIS

Study objectives

It is postulated that high dose immunoablation and autologous stem cell transplantation has superior clinical benefit in comparison to intravenous pulse therapy cyclophosphamide, with respect to survival and prevention of major organ failure (referred to as event-free survival which is considered the primary endpoint) and has a greater impact on skin thickening, visceral involvement, functional status and quality of life.

On 17/04/2012 the following changes were made to the trial record:

1. Australia has been removed from the countries of recruitment and Belgium added.
2. The target number of participants has been changed from 200 to 156.

Ethics approval required

Old ethics approval format

Ethics approval(s)

Ethics approval received from the local medical ethics committee

Primary study design

Interventional

Study design

Multicentre randomised active-controlled parallel-group trial

Study type(s)

Treatment

Health condition(s) or problem(s) studied

Systemic sclerosis

Interventions

This multicentre prospective randomised controlled phase III study compares efficacy and safety of high dose immunoablation and autologous haematopoietic 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:

1. Mobilisation of haematopoietic stem cells with intravenous (IV) cyclophosphamide ($2 \times 2 \text{ g/m}^2$) and filgrastim (10 mg/kg/day)
2. Leukapheresis and selection of CD34+ stem cells
3. Conditioning with IV cyclophosphamide (200 mg/kg) and rbATG (7.5 mg/kg)
4. HSCT

The procedures are normally completed within three to four months after randomisation.

The control treatment arm consists of 12 consecutive monthly IV pulses cyclophosphamide (750 mg/m^2).

As of 17/04/2012, the sponsor name has been amended from European Group Bone Marrow + Transplantation (EBMT)/European League Against Rheumatism (EULAR) Working Party Autoimmune Diseases to European Group for Bone Marrow Transplantation.

Intervention Type

Drug

Phase

Phase III

Drug/device/biological/vaccine name(s)

Cyclophosphamide, filgrastim, rabbit Anti-Thymocyte Globulin (rbATG)

Primary outcome(s)

Current primary outcome measure(s) as of 29/05/2012

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

Previous primary outcome measure(s)

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

Key secondary outcome(s)

Key secondary outcomes include:

1. Treatment related mortality
2. Treatment toxicity
3. Progression-free survival

Completion date

01/01/2008

Eligibility**Key inclusion criteria**

Patients with diffuse systemic sclerosis, aged 16 to 65 years, and:

1. Disease duration four years or less, plus evidence of heart, lung or kidney involvement, plus skin score of 15 or more, or
2. Disease duration two years or less, plus evidence of an acute phase reaction in blood, plus skin score 20 or more

Participant type(s)

Patient

Healthy volunteers allowed

No

Age group

Adult

Sex

All

Key exclusion criteria

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

Date of first enrolment

22/03/2001

Date of final enrolment

01/01/2008

Locations**Countries of recruitment**

United Kingdom

England

Austria

Belgium

Canada

France

Germany

Greece

Italy

Netherlands

Switzerland

Study participating centre

Institute of Cellular Medicine,

Newcastle upon Tyne

United Kingdom

NE2 4HH

Sponsor information**Organisation**

European Group for Bone Marrow Transplantation

Funder(s)

Funder type

Industry

Funder Name

European League Against Rheumatism (EULAR)

Funder Name

Amgen Europe

Funder Name

Sangstat B.V. (The Netherlands)

Funder Name

Horton Foundation (Switzerland)

Funder Name

AP - HP

Alternative Name(s)

Assistance Publique Hôpitaux de Paris, Assistance Publique–Hôpitaux de Paris, AP-HP

Funding Body Type

Private sector organisation

Funding Body Subtype

Trusts, charities, foundations (both public and private)

Location

France

Funder Name

European Group for Blood and Marrow Transplantation (EBMT)

Results and Publications

Individual participant data (IPD) sharing plan

IPD sharing plan summary

Not provided at time of registration

Study outputs

| Output type | Details | Date created | Date added | Peer reviewed? | Patient-facing? |
|----------------------------------|----------|--------------|------------|----------------|-----------------|
| Results article | results | 25/06/2014 | | Yes | No |
| Protocol article | protocol | 01/10/2005 | | Yes | No |