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

Gepubliceerd: 11-09-2005 Laatst bijgewerkt: 18-08-2022

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.

Ethische beoordeling	Positief advies
Status	Werving gestart
Type aandoening	-
Onderzoekstype	Interventie onderzoek

Samenvatting

ID

NL-OMON23346

Bron
NTR

Verkorte titel
ASTIS-trial

Aandoening

Systemic sclerosis.

Ondersteuning

Primaire sponsor: EBMT/EULAR Working Party Autoimmune Diseases.
Overige ondersteuning: EULAR; Amgen Europe; Sangstat; Horton Foundation (Switzerland).

Onderzoeksproduct en/of interventie

Uitkomstmaten

Primaire uitkomstmaten

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.

Toelichting onderzoek

Achtergrond van het onderzoek

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.

Doel van het onderzoek

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.

Onderzoeksproduct en/of interventie

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).

Contactpersonen

Publiek

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Wetenschappelijk

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Deelname eisen

Belangrijkste voorwaarden om deel te mogen nemen (Inclusiecriteria)

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.

Belangrijkste redenen om niet deel te kunnen nemen (Exclusiecriteria)

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

Onderzoeksopzet

Opzet

Type:	Interventie onderzoek
Onderzoeksmodel:	Parallel
Blindering:	Open / niet geblindeerd
Controle:	Geneesmiddel

Deelname

Nederland

Status:	Werving gestart
(Verwachte) startdatum:	22-03-2001
Aantal proefpersonen:	200
Type:	Verwachte startdatum

Ethische beoordeling

Positief advies

Datum:	11-09-2005
Soort:	Eerste indiening

Registraties

Opgevolgd door onderstaande (mogelijk meer actuele) registratie

Geen registraties gevonden.

Andere (mogelijk minder actuele) registraties in dit register

Geen registraties gevonden.

In overige registers

Register	ID
NTR-new	NL300
NTR-old	NTR338
Ander register	: N/A
ISRCTN	ISRCTN54371254

Resultaten

Samenvatting resultaten
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.