Clinical trial results:
Highdose Chemotherapy and transplantation of 34+ selected stem cell for progressive systemic sclerosis
Modification according to manifestation

Summary

EudraCT number	2011-002434-40
Trial protocol	DE
Global end of trial date	27 September 2023
Results information	
Result version number	v1 (current)
This version publication date	01 May 2025
First version publication date	01 May 2025

Trial information

Trial identification	
Sponsor protocol code	AST-MOMA
Additional study identifiers	
ISRCTN number	-
ClinicalTrials.gov id (NCT number)	-
WHO universal trial number (UTN)	-

Notes:

Sponsors	
Sponsor organisation name	University hospital Tuebingen
Sponsor organisation address	Hoppe-Seyler-Straße 3, Tübingen, Germany, 72076
Public contact	Department Internal Medicine II, University Hospital Tuebinegn, 0049 70712980681, joerg.henes@med.uni-tuebingen.de
Scientific contact	Department Internal Medicine II, University Hospital Tuebinegn, 0049 70712980681, joerg.henes@med.uni-tuebingen.de

Notes:

Paediatric regulatory details	
Is trial part of an agreed paediatric investigation plan (PIP)	No
Does article 45 of REGULATION (EC) No 1901/2006 apply to this trial?	No
Does article 46 of REGULATION (EC) No 1901/2006 apply to this trial?	No

Notes:

Results analysis stage

Analysis stage	Final
Date of interim/final analysis	02 January 2024
Is this the analysis of the primary completion data?	Yes
Primary completion date	04 September 2023
Global end of trial reached?	Yes
Global end of trial date	27 September 2023
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To optimise the treatment for severe systemic sclerosis with high dose chemotherapy and autologous stem cell transplantation by adapting the procedure to the individual organ manifestation.

Protection of trial subjects:

The procedures set out in this trial protocol, pertaining to the conduct, evaluation, and documentation of this trial, are designed to ensure that all persons involved in the trial act according to Good Clinical Practice (GCP) and the ethical principles described in the applicable version of the Declaration of Helsinki.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	15 August 2012
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Germany: 44
Worldwide total number of subjects	44
EEA total number of subjects	44

Notes:

Subjects enrolled per age group

In utero	0
Preterm newborn - gestational age < 37 wk	0
Newborns (0-27 days)	0
Infants and toddlers (28 days-23 months)	0
Children (2-11 years)	0
Adolescents (12-17 years)	0
Adults (18-64 years)	44
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

44 patients were included, during screening 9 patients were excluded, 35 patients were transplanted

Pre-assignment

Screening details:

Patienten mit Cyclophosphamid refraktärer systemischer Sklerose mit schwerer Organbeteiligung

Pre-assignment period milestones

Number of subjects started	44
Number of subjects completed	44

Period 1

Period 1 title	aHSCT (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Blinding implementation details:

n.a

Arms

Arm title	Investigational arm

Arm description:

single arm design

Arm type	Experimental
Investigational medicinal product name	HSCT
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Infusion

Dosage and administration details:

n.a

Number of subjects in period 1	Investigational arm
Started	44
Completed	44

Baseline characteristics

End points

End points reporting groups

Reporting group title	Investigational arm
Reporting group description: single arm design	

Primary: Overall survival 3 years

End point title	Overall survival 3 years[1]
End point description:	
End point type	Primary
End point timeframe: 3 years	

Notes:

[1] - No statistical analyses have been specified for this primary end point. It is expected there is at least one statistical analysis for each primary end point.
Justification: Statistical details and charts can be found in the scientific publication: https://ard.bmj.com/content/83/Suppl_1/175.1

End point values	Investigational arm			
Subject group type	Reporting group			
Number of subjects analysed	44			
Units: Percentage	44			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events: Every adverse event must be documented within 36 months after transplantation.	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	26

Frequency threshold for reporting non-serious adverse events: 5 %

Notes:

[1] - There are no non-serious adverse events recorded for these results. It is expected that there will be at least one non-serious adverse event reported.
Justification: Deatils of Adverse events can be found in the scientific publiciation: https://ard.bmj.com/content/83/Suppl_1/175.1

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported