



## 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 Tuebingen, 0049 70712980681, joerg.henes@med.uni-tuebingen.de |
| Scientific contact           | Department Internal Medicine II, University Hospital Tuebingen, 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

|                                       |                     |
|---------------------------------------|---------------------|
| <b>Number of subjects in period 1</b> | Investigational arm |
| Started                               | 44                  |
| Completed                             | 44                  |

## Baseline characteristics

## End points

### End points reporting groups

|   |                     |
|---|---------------------|
| Reporting group title                             | Investigational arm |
| Reporting group description:<br>single arm design |                     |

### Primary: Overall survival 3 years

|                        |   |
|------------------------|---|
| End point title        | Overall survival 3 years <sup>[1]</sup> |
| End point description: |   |

|                                 |         |
|---------------------------------|---------|
| End point type                  | Primary |
| End point timeframe:<br>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](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

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### Adverse events information<sup>[1]</sup>

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Timeframe for reporting adverse events:

Every adverse event must be documented within 36 months after transplantation.

|                 |            |
|-----------------|------------|
| Assessment type | Systematic |
|-----------------|------------|

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### Dictionary used

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|                 |        |
|-----------------|--------|
| Dictionary name | MedDRA |
|-----------------|--------|

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|                    |    |
|--------------------|----|
| Dictionary version | 26 |
|--------------------|----|

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

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#### 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: Details of Adverse events can be found in the scientific publication: [https://ard.bmj.com/content/83/Suppl\\_1/175.1](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

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### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

None reported