# Clinical trial results:
# Stem Cell therapy in IschEmic Non-treatable Cardiac disease - SCIENCE

## Summary

| | |
|---|---|
| EudraCT number | 2015-002929-19 |
| Trial protocol | NL DK SI DE AT PL |
| Global end of trial date | 31 December 2022 |

### Results information

| | |
|---|---|
| Result version number | v1 (current) |
| This version publication date | 19 May 2023 |
| First version publication date | 19 May 2023 |
| Summary attachment (see zip file) | Synopsis SCIENCE (Synopsis SCIENCE clinical trial Eudra-CT.pdf)<br>SCIENCE trial (European J of Heart Fail - 2023 - Qayyum - Effect of allogeneic adipose tissuederived mesenchymal stromal cell treatment.pdf) |

## Trial information

### Trial identification

| | |
|---|---|
| Sponsor protocol code | SCIENCE |

### Additional study identifiers

| | |
|---|---|
| ISRCTN number | - |
| ClinicalTrials.gov id (NCT number) | NCT02673164 |
| WHO universal trial number (UTN) | - |

Notes:

### Sponsors

| | |
|---|---|
| Sponsor organisation name | Rigshospitalet |
| Sponsor organisation address | Blegdamsvej, Copenhagen, Denmark, 2100 |
| Public contact | Jens Kastrup, Department of Cardiology, 2014, Rigshospitalet, 0045 35452819, jens.kastrup@regionh.dk |
| Scientific contact | Jens Kastrup, Department of Cardiology, 2014, Rigshospitalet, 0045 35452819, jens.kastrup@regionh.dk |

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 | 01 July 2022 |
| Is this the analysis of the primary completion data? | Yes |
| Primary completion date | 01 May 2022 |
| Global end of trial reached? | Yes |
| Global end of trial date | 31 December 2022 |
| Was the trial ended prematurely? | No |

Notes:

## General information about the trial

Main objective of the trial:

To perform at clinical double-blind placebo-controlled CSCC_ASC multicentre study in heart failure patients to investigate the regenerative capacity of the CSCC_ASC treatment.

Protection of trial subjects:

In hospital follow-up 24 hours after treatment.

Background therapy:

Maximal tolarable medical heart failure therapy

Evidence for comparator:

No

| | |
|---|---|
| Actual start date of recruitment | 01 January 2016 |
| Long term follow-up planned | No |
| Independent data monitoring committee (IDMC) involvement? | No |

Notes:

## Population of trial subjects

### Subjects enrolled per country

| | |
|---|---|
| Country: Number of subjects enrolled | Poland: 20 |
| Country: Number of subjects enrolled | Slovenia: 30 |
| Country: Number of subjects enrolled | Austria: 15 |
| Country: Number of subjects enrolled | Denmark: 34 |
| Country: Number of subjects enrolled | Germany: 20 |
| Country: Number of subjects enrolled | Netherlands: 14 |
| Worldwide total number of subjects | 133 |
| EEA total number of subjects | 133 |

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) | 58 |
| From 65 to 84 years | 75 |
| 85 years and over | 0 |

## Subject disposition

### Recruitment

Recruitment details:

A total of 133 of the planned 139 patients were included in the trial. It was not possible to prolong the inclusion period, due to finalization of the Horizon2020 grant period.

### Pre-assignment

Screening details:

A total of 133 patients (122 men and 11 women) with stable symptomatic chronic ischemic HFrEF were included into the study. Approximately 2/3 of the patients were in NYHA II class and 1/3 in NYHA III in both groups.

### Pre-assignment period milestones

| | |
|---|---|
| Number of subjects started | 133 |
| Number of subjects completed | 133 |

### Period 1

| | |
|---|---|
| Period 1 title | Baseline ASC (overall period) |
| Is this the baseline period? | Yes |
| Allocation method | Randomised - controlled |
| Blinding used | Double blind |
| Roles blinded | Subject, Investigator, Monitor, Data analyst, Carer, Assessor |

Blinding implementation details:

All participating in the clinical contact with the patients and the collected data were blinded. When all data were collected and analyzed then the code was broken by the producer of the cell and placebo vials.

### Arms

| | |
|---|---|
| Are arms mutually exclusive? | Yes |
| **Arm title** | ASC treatment |

Arm description: -

| | |
|---|---|
| Arm type | Experimental |
| Investigational medicinal product name | CSCC_ASC |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Injection/infusion |
| Routes of administration | Intramuscular use |

Dosage and administration details:

Injections of 110 mio. CSCC_ASCs in 5 mL CryoStor

| | |
|---|---|
| **Arm title** | Placebo |

Arm description: -

| | |
|---|---|
| Arm type | Placebo |
| Investigational medicinal product name | Saline |
| Investigational medicinal product code | |
| Other name | |
| Pharmaceutical forms | Injection/infusion |
| Routes of administration | Intramuscular use |

Dosage and administration details:

Injections of saline

| **Number of subjects in period 1** | ASC treatment | Placebo |
|---|---|---|
| Started | 90 | 43 |
| Completed | 90 | 41 |
| Not completed | 0 | 2 |
| dead | - | 2 |

## Baseline characteristics

| Reporting groups | |
|---|---|
| Reporting group title | Baseline ASC |

Reporting group description: -

| Reporting group values | Baseline ASC | Total | |
|---|---|---|---|
| Number of subjects | 133 | 133 | |
| Age categorical<br>Units: Subjects | | | |
| In utero | | 0 | |
| Preterm newborn infants (gestational age < 37 wks) | | 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) | | 0 | |
| From 65-84 years | | 0 | |
| 85 years and over | | 0 | |
| Age continuous | | | |
| The mean age was 66.4 ± 8.1 years in the ASC group and 64.0 ± 8.8 years in the placebo group | | | |
| Units: years | | | |
| arithmetic mean | 65.2 | | |
| standard deviation | ± 8.5 | - | |
| Gender categorical<br>Units: Subjects | | | |
| Female | 11 | 11 | |
| Male | 122 | 122 | |

## End points

| End points reporting groups | |
|---|---|
| Reporting group title | ASC treatment |
| Reporting group description: - | |
| Reporting group title | Placebo |
| Reporting group description: - | |

| Primary: LVESV (left ventricular end systolic volume) | |
|---|---|
| End point title | LVESV (left ventricular end systolic volume) |
| End point description: | |
| End point type | Primary |
| End point timeframe: | |
| 6 months after ASC or placebo intramyocardial injections | |

| End point values | ASC treatment | Placebo | | |
|---|---|---|---|---|
| Subject group type | Reporting group | Reporting group | | |
| Number of subjects analysed | 90 | 41 | | |
| Units: mL | | | | |
| number (not applicable) | 90 | 41 | | |

### Statistical analyses

| Statistical analysis title | T-test |
|---|---|
| Comparison groups | ASC treatment v Placebo |
| Number of subjects included in analysis | 131 |
| Analysis specification | Pre-specified |
| Analysis type | superiority |
| P-value | < 0.05 |
| Method | t-test, 2-sided |

## Adverse events

| Adverse events information | |
|---|---|
| Timeframe for reporting adverse events: 6 months after ASC/placebo treatment | |
| Assessment type | Systematic |

| Dictionary used | |
|---|---|
| Dictionary name | other |
| Dictionary version | 1 |

| Reporting groups | |
|---|---|
| Reporting group title | ASC and Placebo |

Reporting group description: -

| Serious adverse events | ASC and Placebo | | |
|---|---|---|---|
| Total subjects affected by serious adverse events | | | |
| subjects affected / exposed | 5 / 133 (3.76%) | | |
| number of deaths (all causes) | 5 | | |
| number of deaths resulting from adverse events | 0 | | |
| Cardiac disorders | | | |
| 1 year | | | |
| subjects affected / exposed | 5 / 133 (3.76%) | | |
| occurrences causally related to treatment / all | 0 / 5 | | |
| deaths causally related to treatment / all | 0 / 5 | | |

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

| Non-serious adverse events | ASC and Placebo | | |
|---|---|---|---|
| Total subjects affected by non-serious adverse events | | | |
| subjects affected / exposed | 65 / 133 (48.87%) | | |
| Cardiac disorders | | | |
| 1 year | | | |
| subjects affected / exposed | 65 / 133 (48.87%) | | |
| occurrences (all) | 65 | | |

## 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

### Online references

http://www.ncbi.nlm.nih.gov/pubmed/36644821