Clinical trial results:
A phase I safety and tolerability study of infusing the autologous progeny of an adult CD34+ subset into patients with type I diabetes mellitus and a successful renal transplant.

Summary

EudraCT number	2006-002328-40
Trial protocol	GB
Global end of trial date	31 May 2013

Results information

Result version number	v1 (current)
This version publication date	13 November 2019
First version publication date	13 November 2019

Trial information

Trial identification

Sponsor protocol code	HHSC/005

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT00788827
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Imperial College London
Sponsor organisation address	South Kensingston Campus, London, United Kingdom, SW7 2AZ
Public contact	Charles Pusey, Imperial College London, +44 20 8383 2308, c.pusey@imperial.ac.uk
Scientific contact	Charles Pusey, Imperial College London, +44 20 8383 2308, c.pusey@imperial.ac.uk

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	30 May 2014
Is this the analysis of the primary completion data?	Yes
Primary completion date	31 May 2013
Global end of trial reached?	Yes
Global end of trial date	31 May 2013
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess the safety and tolerance of a high dose of autologous expanded progeny of adult CD34+ stem cell subset (InsulinCytes) when introduced into either the body or tail of the pancreas.

Protection of trial subjects:

None

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	03 November 2008
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	United Kingdom: 7
Worldwide total number of subjects	7
EEA total number of subjects	7

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)	7
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Participants were recruited at Imperial College NHS Healthcare Trust, Hammersmith Hospital between November 2008 and May 2013

Pre-assignment

Screening details:

A total of seven participants were eligible for the study

Period 1

Period 1 title	Overall (overall period)
Is this the baseline period?	Yes
Allocation method	Non-randomised - controlled
Blinding used	Not blinded

Arms

Arm title	Autologous CD34+ Stem Cells

Arm description:

Patients received Autologous CD34+ Stem Cells

Arm type	Experimental
Investigational medicinal product name	CD34+ Stem Cells
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection/infusion
Routes of administration	Other use

Dosage and administration details:

The expanded autologous CD34+ cells were infused into the right hepatic artery of the patients in the imaging department.

Number of subjects in period 1	Autologous CD34+ Stem Cells
Started	7
Completed	5
Not completed	2
Lack of efficacy	2

Baseline characteristics

Reporting groups

Reporting group title	Overall

Reporting group description: -

Reporting group values	Overall	Total	
Number of subjects	7	7	
Age categorical Units: Subjects			
Adults (18-64 years)	7	7	
Age continuous Units: years geometric mean standard deviation	 54.6 ± 4.2	 -	
Gender categorical Units: Subjects			
Female	2	2	
Male	5	5	

Subject analysis sets

Subject analysis set title	Pre Infusion of Stem Cells
Subject analysis set type	Sub-group analysis

Subject analysis set description:
Mean HbA1c laboratory measurements pre stem cell infusion

Subject analysis set title	Post Infusion of Stem Cells
Subject analysis set type	Sub-group analysis

Subject analysis set description:
Mean HbA1c laboratory measurements post stem cell infusion

Reporting group values	Pre Infusion of Stem Cells	Post Infusion of Stem Cells	
Number of subjects	5	5	
Age categorical Units: Subjects			
Adults (18-64 years)	5	5	
Age continuous Units: years geometric mean standard deviation	 ±	 ±	
Gender categorical Units: Subjects			
Female Male			

End points

End points reporting groups

Reporting group title	Autologous CD34+ Stem Cells
Reporting group description: Patients received Autologous CD34+ Stem Cells	
Subject analysis set title	Pre Infusion of Stem Cells
Subject analysis set type	Sub-group analysis
Subject analysis set description: Mean HbA1c laboratory measurements pre stem cell infusion	
Subject analysis set title	Post Infusion of Stem Cells
Subject analysis set type	Sub-group analysis
Subject analysis set description: Mean HbA1c laboratory measurements post stem cell infusion	

Primary: Number of Participants Who Experienced Adverse Events_Haematoma at femoral catheter insertion

End point title	Number of Participants Who Experienced Adverse Events_Haematoma at femoral catheter insertion[1]
End point description:	
End point type	Primary
End point timeframe: 14 days	

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: No statistical analyses due to low number of participants.

End point values	Autologous CD34+ Stem Cells			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: Participants	1			

Statistical analyses

No statistical analyses for this end point

Primary: Number of Participants Who Experienced Adverse Events_Fatigue

End point title	Number of Participants Who Experienced Adverse Events_Fatigue[2]
End point description:	
End point type	Primary
End point timeframe: 14 days	

Notes:

[2] - 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: No statistical analyses due to low number of participants.

End point values	Autologous CD34+ Stem Cells			
Subject group type	Reporting group			
Number of subjects analysed	7			
Units: Participants	7			

Statistical analyses

No statistical analyses for this end point

Secondary: Hba1C Data of Pre and Post Stem Cell Infusion

End point title	Hba1C Data of Pre and Post Stem Cell Infusion

End point description:

End point type	Secondary

End point timeframe:

12 weeks

End point values	Pre Infusion of Stem Cells	Post Infusion of Stem Cells		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	5	5		
Units: percentage				
geometric mean (standard deviation)	7.2 (± 1.3)	7.24 (± 1.2)		

Statistical analyses

Statistical analysis title	Hba1C Data of Pre and Post Stem Cell Infusion
Comparison groups	Pre Infusion of Stem Cells v Post Infusion of Stem Cells
Number of subjects included in analysis	10
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.05
Method	Mixed models analysis

Secondary: Insulin level

End point title	Insulin level

End point description:

End point type	Secondary

End point timeframe:
12 weeks

End point values	Pre Infusion of Stem Cells	Post Infusion of Stem Cells		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	5	5		
Units: iu/day				
geometric mean (standard deviation)	59.4 (± 25.7)	54.06 (± 18.2)		

Statistical analyses

No statistical analyses for this end point

Secondary: Amylase Level

End point title	Amylase Level

End point description:

End point type	Secondary

End point timeframe:
12 weeks

End point values	Pre Infusion of Stem Cells	Post Infusion of Stem Cells		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	5	5		
Units: units/L				
geometric mean (standard deviation)	48.49 (± 25.2)	75.52 (± 34.2)		

Statistical analyses

No statistical analyses for this end point

Secondary: Serum Creatinine

End point title	Serum Creatinine

End point description:

End point type	Secondary

End point timeframe:
12 weeks

End point values	Pre Infusion of Stem Cells	Post Infusion of Stem Cells		
Subject group type	Subject analysis set	Subject analysis set		
Number of subjects analysed	5	5		
Units: umol/L				
geometric mean (standard deviation)	128.22 (± 11.9)	118.64 (± 15.5)		

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 12 weeks	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	10
Reporting groups	
Reporting group title	Autologous CD34+ Stem Cells
Reporting group description: Patients received Autologous CD34+ Stem Cells	

Serious adverse events	Autologous CD34+ Stem Cells		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 7 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Autologous CD34+ Stem Cells		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	7 / 7 (100.00%)		
Nervous system disorders			
Fatigue			
subjects affected / exposed	7 / 7 (100.00%)		
occurrences (all)	7		
Blood and lymphatic system disorders			
Haemotoma			
subjects affected / exposed	1 / 7 (14.29%)		
occurrences (all)	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