



## Clinical trial results:

### The pharmacological effects of granulocyte-colony stimulating factor (GCSF) on frataxin expression in patients with Friedreich Ataxia

#### Summary

EudraCT number	2017-003084-34
Trial protocol	GB
Global end of trial date	12 April 2019

#### Results information

Result version number	v1 (current)
This version publication date	26 January 2020
First version publication date	26 January 2020
Summary attachment (see zip file)	FA GCSF result synopsis (Clinical_Study_Synopsis_GCSF FA.pdf)

#### Trial information

##### Trial identification

Sponsor protocol code	GCSF_FRDA_v1
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##### Additional study identifiers

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

Notes:

#### Sponsors

Sponsor organisation name	University of Bristol
Sponsor organisation address	Research and Enterprise Development One Cathedral Square, Bristol, United Kingdom, BS1 5DD
Public contact	Alastair Wilkins, University of Bristol, research-governance@bristol.ac.uk
Scientific contact	Alastair Wilkins, University of Bristol, 44 1174147802, alastair.wilkins@bristol.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	19 December 2019
Is this the analysis of the primary completion data?	No
Global end of trial reached?	Yes
Global end of trial date	12 April 2019
Was the trial ended prematurely?	No

Notes:

## General information about the trial

Main objective of the trial:

Does administration of GCSF lead to improvements in blood markers of Friedreich Ataxia?

We will study a small number of patients with the condition and will administer GCSF (at identical doses to those given to 'healthy' people prior to bone marrow donation) for a short period of time. We will define whether administration of the drug leads to changes in blood markers which would indicate a positive response to the drug. The study will also allow us to decide what blood markers we can monitor in the subsequent trial. This has not been studied before and is a vital step in the development of a stem cell research trial. Once information has been obtained from this study, a larger trial of GCSF in FRDA can be developed.

Protection of trial subjects:

The trial was carried out using the principles of Good Clinical Practice and was approved by UK Medicines and Healthcare products Regulatory Agency (MHRA), Health Research Authority (HRA) and Research Ethics Committee (REC).

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	01 October 2017
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:

7 participants:

Inclusion criteria:

Genetic diagnosis of Friedreich's Ataxia (FA)

Inclusion criteria: Genetic diagnosis of FA; Age of over 18

### Pre-assignment

Screening details:

Patients attending local/ regional or national atxia clinic were screened

Genetic diagnosis of Friedreich's Ataxia (FA)

Inclusion criteria: Genetic diagnosis of FA; Age of over 18

### Period 1

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

Blinding implementation details:

N/A

### Arms

Arm title	Overall trial
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Arm description:

Single arm; all participants received the same intervention

Arm type	Experimental
Investigational medicinal product name	Granulocyte-colony stimulating factor
Investigational medicinal product code	
Other name	Lenograstim, Granocyte
Pharmaceutical forms	Powder for suspension for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

1.28 million units/kg daily for 5 days

Number of subjects in period 1	Overall trial
Started	7
Completed	7

## Baseline characteristics

### Reporting groups

Reporting group title	Overall trial
Reporting group description:	
Single arm; all participants received the same intervention	

Reporting group values	Overall trial	Total	
Number of subjects	7	7	
Age categorical			
Units: Subjects			
In utero	0	0	
Preterm newborn infants (gestational age < 37 wks)	0	0	
Newborns (0-27 days)	0	0	
Infants and toddlers (28 days-23 months)	0	0	
Children (2-11 years)	0	0	
Adolescents (12-17 years)	0	0	
Adults (18-64 years)	7	7	
From 65-84 years	0	0	
85 years and over	0	0	
Gender categorical			
Units: Subjects			
Female	4	4	
Male	3	3	

## End points

### End points reporting groups

Reporting group title	Overall trial
Reporting group description:	
Single arm; all participants received the same intervention	

### Primary: Change in frataxin expression in peripheral blood cells after granulocyte-colony stimulating factor (G-CSF) administration to patients with Friedreich's Ataxia

End point title	Change in frataxin expression in peripheral blood cells after granulocyte-colony stimulating factor (G-CSF) administration to patients with Friedreich's Ataxia <sup>[1]</sup>
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End point description:

End point type	Primary
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End point timeframe:

Day 5-19 after commencement of administration of IMP

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: All participants received the IMP. No control comparator

End point values	Overall trial			
Subject group type	Reporting group			
Number of subjects analysed	6 <sup>[2]</sup>			
Units: pg/μg of protein				
number (not applicable)	6			

Notes:

[2] - 1 participant did not complete trial

### Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

Between commencement of administration of IMP and day 19 of the study in each participant

Assessment type	Non-systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	21
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### Reporting groups

Reporting group title	Single arm
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Reporting group description: -

Serious adverse events	Single arm		
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: 5 %

Non-serious adverse events	Single arm		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 7 (42.86%)		
Nervous system disorders			
headache			
subjects affected / exposed	1 / 7 (14.29%)		
occurrences (all)	1		
Musculoskeletal and connective tissue disorders			
bone pain			
subjects affected / exposed	2 / 7 (28.57%)		
occurrences (all)	2		

## 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