Clinical trial results:
Effects of recombinant human Erythropoietin on circulating and intramuscular endothelial progenitor cells, neovascularisation and oxidative metabolism of skeletal muscle in Friedreich's Ataxia

Summary

EudraCT number	2008-000040-13
Trial protocol	AT
Global end of trial date	01 August 2012

Results information

Result version number	v1 (current)
This version publication date	16 February 2022
First version publication date	16 February 2022

Trial information

Trial identification

Sponsor protocol code	21011075118

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	Medical University Innsbruck
Sponsor organisation address	Christoph-Probst-Platz 1, Innrain 52, Innsbruck, Austria, 6020
Public contact	Priv.Doz. Dr. Sylvia Bösch, University Hospital for Neurology, Anichstrasse 35, 6020 Innsbruck, +43 (0)512-504-26285, sylvia.boesch@tirol-kliniken.at
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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 August 2012
Is this the analysis of the primary completion data?	Yes
Primary completion date	01 August 2012
Global end of trial reached?	Yes
Global end of trial date	01 August 2012
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Aims of the proposed study 1) to investigate morphological and biochemical parameters of skeletal muscle systematically, to identify hematopoietic progenitor cells (CD34, CD133) as well as to measure baseline Frataxin expression in skeletal muscle (specimen) of FRDA patients. 2) to measure numbers of circulating CD34 and CD133 hematopoietic progenitor cells in peripheral blood of FRDA patients in response to rhuEPO treatment; 3) to study effects of rhuEPO treatment on Frataxin expression, numbers of CD34 and CD133 hematopoietic progenitor cells, satellite cells, capillary density and respiratory chain complex activi-ties of skeletal muscle tissue obtained by re-biopsy. 4) to identify changes in muscle energy metabolism (in-vivo marker) in skeletal muscle of rhuEPO treated FRDA patients applying magnetic resonance spectros-copy at baseline and study endpoint.

Protection of trial subjects:

Safety was assessed by red blood cell count and blood pressure in two weekly intervals.

Background therapy:

-

Evidence for comparator:

There was no evidence for a comparator.

Actual start date of recruitment	11 February 2009
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	Austria: 15
Worldwide total number of subjects	15
EEA total number of subjects	15

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)	15
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

All patients were recruited from the ataxia outpatient clinic of the Department of Neurology (Medical University Innsbruck).

Pre-assignment

Screening details:

Screening visits to check for inclusion and exclusion criteria were carried out in February and March 2009 after final trial registration.

Period 1

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

Blinding implementation details:

This trial was not blinded.

Arms

Are arms mutually exclusive?	Yes
Arm title	rhuEPO

Arm description: -

Arm type	Experimental
Investigational medicinal product name	Neorecormon
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

FRDA patients received 3,000 international units (IU) rhuEPO (Roche, Switzerland) thrice weekly over a study period of 8 weeks.

Arm title	Control

Arm description: -

Arm type	No intervention

No investigational medicinal product assigned in this arm

Number of subjects in period 1	rhuEPO	Control
Started	7	8
Completed	7	8

Baseline characteristics

Reporting groups	
Reporting group title	rhuEPO
Reporting group description: -	
Reporting group title	Control
Reporting group description: -	

Reporting group values	rhuEPO	Control	Total
Number of subjects	7	8	15
Age categorical Units: Subjects			
In utero	0	0	0
Preterm newborn infants (gestational age < 37 wks)	0	0	0
Newborns (0-27 days)	0	0	0
Infants and toddlers (28 days-23 months)	0	0	0
Children (2-11 years)	0	0	0
Adolescents (12-17 years)	0	0	0
Adults (18-64 years)	7	8	15
From 65-84 years	0	0	0
85 years and over	0	0	0
Age continuous Units: years			
arithmetic mean	40.00	40.06	
standard deviation	± 14.01	± 13.26	-
Gender categorical Units: Subjects			
Female	1	2	3
Male	6	6	12

End points

End points reporting groups

Reporting group title	rhuEPO
Reporting group description: -	
Reporting group title	Control
Reporting group description: -	

Primary: Inorganic Phosphate (resting state)

End point title	Inorganic Phosphate (resting state)
End point description:	
Phosphorus 31 magnetic resonance spectroscopy (31P MRS) offers a non invasive investigation of human skeletal muscle bioenergetics by monitoring relative and absolute changes of phosphocreatine (PCr), inorganic phosphate (Pi) and adenosine triphosphate (ATP) during incremental exercise and recovery.	
End point type	Primary
End point timeframe:	
Day 0- week 8	

End point values	rhuEPO	Control		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	7	8		
Units: Pi				
arithmetic mean (standard deviation)	3.57 (± 0.86)	2.85 (± 0.44)		

Statistical analyses

Statistical analysis title	Pi (resting state)
Comparison groups	Control v rhuEPO
Number of subjects included in analysis	15
Analysis specification	Pre-specified
Analysis type	other
P-value	= 0.01
Method	Wilcoxon (Mann-Whitney)

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events: Day 0- week 8	
Assessment type	Systematic

Dictionary used	
Dictionary name	CTCAE
Dictionary version	4.0

Reporting groups	
Reporting group title	rhuEPO
Reporting group description: -	
Reporting group title	Control
Reporting group description: -	

Serious adverse events	rhuEPO	Control	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 7 (0.00%)	0 / 8 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	

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

Non-serious adverse events	rhuEPO	Control	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 7 (0.00%)	0 / 8 (0.00%)	

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: No AEs and SAEs were observed during this trial.

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/23922695