Clinical trial results:
Untersuchung der Dosis-Wirk-Beziehung unterschiedlicher Erythropoetin-Dosen auf Frataxin bei Friedreich Ataxie

Summary

EudraCT number	2007-004919-55
Trial protocol	AT
Global end of trial date	01 December 2011

Results information

Result version number	v1 (current)
This version publication date	28 January 2022
First version publication date	28 January 2022

Trial information

Trial identification

Sponsor protocol code	04-01-1980

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, Medical University Innsbruck, Department of Neurology, Anichstrasse 35, 6020 Innsbruck, +43 (0)512-504-26285, sylvia.boesch@tirol-kliniken.at
Scientific contact	Priv.Doz. Dr. Sylvia Bösch, Medical University Innsbruck, Department of Neurology, Anichstrasse 35, 6020 Innsbruck, +43 (0)512-504-26285, sylvia.boesch@tirol-kliniken.at

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	29 April 2011
Is this the analysis of the primary completion data?	Yes
Primary completion date	29 April 2011
Global end of trial reached?	Yes
Global end of trial date	01 December 2011
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Dosis Wirkungs Beziehung zwischen Erythropoetin und Frataxin bei Patienten mit Friedreich Ataxie

Protection of trial subjects:

Blood samples were taken to monitor haemoglobin levels, haematocrit and platelet counts. Analyses of blood parameters were done by standard laboratory procedures in an ISO 9001:2009 certified laboratory. Blood pressure was measured regularly. ECG was performed at screening and last visit.

Background therapy:

-

Evidence for comparator:

There was no evidence for a comparator.

Actual start date of recruitment	27 December 2007
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: 5
Worldwide total number of subjects	5
EEA total number of subjects	5

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)	5
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Five patients with definite diagnosis of FRDA were included in this study after written informed consent.

Pre-assignment

Screening details:

None of the patients had idebenone, co-enzyme Q10 or other antioxidants 6 weeks prior to the study.

Period 1

Period 1 title	Overall study (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Single blind
Roles blinded	Subject

Blinding implementation details:

In this trial FRDA patients received three different single injections of rhuEPO (5,000, 10,000 and 30,000 IU; epoetin beta, Roche, Basel, Switzerland) subcutaneously in monthly intervals. Laboratory staff and patients were blinded for the amount of administered rhuEPO.

Arms

Arm title	rhuEPO

Arm description:

In this single site, one arm pilot trial FRDA patients received three different single injections of rhuEPO (5,000, 10,000 and 30,000 IU; epoetin beta, Roche, Basel, Switzerland) subcutaneously in monthly intervals.

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:

Patients received three rhuEPO singledose injections (5,000, 10,000,30,000 IU) at intervals of 1 month.

Number of subjects in period 1	rhuEPO
Started	5
Completed	5

Baseline characteristics

Reporting groups

Reporting group title	Overall study

Reporting group description: -

Reporting group values	Overall study	Total	
Number of subjects	5	5	
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)	5	5	
From 65-84 years	0	0	
85 years and over	0	0	
Age continuous Units: years			
median	49		
inter-quartile range (Q1-Q3)	31 to 52	-	
Gender categorical Units: Subjects			
Female	1	1	
Male	4	4	

End points

End points reporting groups

Reporting group title	rhuEPO

Reporting group description:

In this single site, one arm pilot trial FRDA patients received three different single injections of rhuEPO (5,000, 10,000 and 30,000 IU; epoetin beta, Roche, Basel, Switzerland) subcutaneously in monthly intervals.

Primary: Monthly Frataxin Measurements

End point title	Monthly Frataxin Measurements[1]

End point description:

We found a 19% frataxin increase after applying 5,000 IU rhuEPO (median 119%; IQR 106–254%). Frataxin measurements after 2 months showed a boost up to 263% (IQR 142–417%) as compared to rhuEPO naïve baseline frataxin levels. Finally, frataxin levels increased to maximal levels of 310% (IQR 182–480%; p=0.03) after 3 months.

End point type	Primary

End point timeframe:

Baseline - 3 months

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: Kolmogorov–Smirnov test was used to assess normal distribution of frataxin levels.

End point values	rhuEPO			
Subject group type	Reporting group			
Number of subjects analysed	5			
Units: Percentage				
median (inter-quartile range (Q1-Q3))				
after 1 month	119 (106 to 254)			
after 2 months	263 (142 to 417)			
after 3 months	310 (182 to 480)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information[1]

Timeframe for reporting adverse events:

27.12.2007- 29.04.2011

Assessment type	Systematic

Dictionary used

Dictionary name	CTCAE
Dictionary version	3.0

Reporting groups

Reporting group title	rhuEPO

Reporting group description:

In this single site, one arm pilot trial FRDA patients received three different single injections of rhuEPO (5,000, 10,000 and 30,000 IU; epoetin beta, Roche, Basel, Switzerland) subcutaneously in monthly intervals.

Serious adverse events	rhuEPO		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 5 (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	rhuEPO		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 5 (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: rhuEPO was well tolerated, no non- serious adverse events were observed.

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/21597884