



Clinical trial results:

Eine offene, nicht randomisierte, einarmige Pilotstudie zur Beurteilung der Wirksamkeit von Erythropoetin bei Friedreich Ataxie.

Summary

EudraCT number	2005-005938-12
Trial protocol	AT
Global end of trial date	01 November 2007

Results information

Result version number	v1 (current)
This version publication date	13 December 2021
First version publication date	13 December 2021

Trial information

Trial identification

Sponsor protocol code	29-09-1963
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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
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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 November 2007
Is this the analysis of the primary completion data?	Yes
Primary completion date	01 November 2007
Global end of trial reached?	Yes
Global end of trial date	01 November 2007
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Basierend auf eigenen in-vitro Voruntersuchungen soll nun im Rahmen einer Pilotstudie die Sicherheit und Wirksamkeit von Erythropoetin in der Behandlung von Patienten mit Friedreich Ataxie untersucht werden. Diese Untersuchung stellt nach bisher frustrierten Therapieversuchen (z.B. Desoxyferramin, Idebenone) einen gänzlich neuen Therapieansatz dar.

HAUPTZIELKRITERIUM: Stabile Aufregulierung von FRATAXIN bei Patienten mit Friedreich Ataxie über 8 Wochen.

Recently, we showed that rHuEPO increases frataxin levels in isolated lymphocytes from FRDA patients in vitro. Based on these findings, we initiated a clinical pilot trial to investigate the effect of rHuEPO on frataxin levels in FRDA patients.

Protection of trial subjects:

Safety was assessed by weekly measurement of hematocrit (Hc), hemoglobin, erythrocyte counts, reticulocytes, and thrombocytes. Blood pressure using Riva Rocci technique was monitored weekly. Electrocardiogram was performed at baseline and last visit.

Background therapy: -

Evidence for comparator:

There was no evidence for a comparator.

Actual start date of recruitment	08 June 2006
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: 12
Worldwide total number of subjects	12
EEA total number of subjects	12

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)	12
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Patients with definite FRDA who were 18 years or older were included.

Pre-assignment

Screening details:

Twelve patients with a definite diagnosis of FRDA were included. All patients had ataxia, two patients suffered from mild cardiomyopathy, and no patient had diabetes.

Period 1

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

Arms

Arm title	rHuEPO
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	Neo-Recormon
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Concentrate for solution for injection
Routes of administration	Subcutaneous use

Dosage and administration details:

FRDA patients received 5,000IU rHuEPO (Neorecormone; Roche, Vienna, Austria) three times weekly subcutaneously for a period of 8 weeks.

Number of subjects in period 1	rHuEPO
Started	12
Completed	11
Not completed	1
Consent withdrawn by subject	1

Baseline characteristics

Reporting groups

Reporting group title	Treatment
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Reporting group description: -

Reporting group values	Treatment	Total	
Number of subjects	12	12	
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)	12	12	
From 65-84 years	0	0	
85 years and over	0	0	
Age continuous			
Units: years			
arithmetic mean	34.9		
full range (min-max)	18 to 55	-	
Gender categorical			
Units: Subjects			
Female	4	4	
Male	8	8	

End points

End points reporting groups

Reporting group title	rHuEPO
Reporting group description:	-

Primary: Frataxin levels

End point title	Frataxin levels ^[1]
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End point description:

Frataxin levels at baseline (BL) and after 8 weeks of treatment with recombinant human erythropoietin (rHuEPO) were measured. Individual frataxin levels at baseline were set as 100% and compared to frataxin levels after 8 weeks of treatment.

End point type	Primary
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End point timeframe:

Day 0- end of week 8

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: Changes of frataxin levels at week 8 versus baseline were performed using the paired t test (n=10; p < 0.01).

End point values	rHuEPO			
Subject group type	Reporting group			
Number of subjects analysed	10			
Units: change of frataxin levels in %				
arithmetic mean (full range (min-max))				
change in % at week 8	27 (15 to 63)			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information^[1]

Timeframe for reporting adverse events:

Day 0 - end of week 8

Assessment type	Systematic
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Dictionary used

Dictionary name	CTCAE
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Dictionary version	3.0
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Reporting groups

Reporting group title	rHuEPO
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Reporting group description: -

Serious adverse events	rHuEPO		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 12 (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 / 12 (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 during 8 weeks, 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/17702040>

<http://www.ncbi.nlm.nih.gov/pubmed/18759345>