



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 |
|-----------------------|------------|

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.Doiz.Dr.Sylvia Bösch MSc, Medical University Innsbruck, University Hospital for Neurology, Anichstrasse 35, +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 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 |
|-----------------------|-----------|

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] |
|-----------------|--------------------------------|

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 |
|----------------|---------|

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 |
|-----------------|------------|

Dictionary used

| | |
|-----------------|-------|
| Dictionary name | CTCAE |
|-----------------|-------|

| | |
|--------------------|-----|
| Dictionary version | 3.0 |
|--------------------|-----|

Reporting groups

| | |
|-----------------------|--------|
| Reporting group title | rHuEPO |
|-----------------------|--------|

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>