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Treatment of the neurodegenerative disease Friedreich´s ataxia

FRIEDREICH´S ATAXIA (FRDA):

- **Clinical symptoms:** progressive ataxia of limbs, sensory loss, pyramidal signs, diabetes mellitus and hypertrophic cardiomyopathy. Wheelchair dependence within 10 years from onset.
- **Cause of disease:** GAA-trinucleotide expansion in frataxin gene results in frataxin-deficiency causing mitochondral iron accumulation, oxidative stress and cell damage. GAA–triplett repeat number inversely correlates with frataxin-levels and age of onset.
- **Treatment of disease:** Antioxidants, iron chelators, frataxin-increasing drugs and gene therapy were proposed.
  Currently no FDA/EMA approved treatment available for Friedreich`s ataxia.
- **Frequency:**
  Prevalence: 2:100,000 - 4:100,000
  Carrier frequency : 1:60 - 1:100

Frataxin
(Dhe-Phaganon et.al 2000)

Most common inherited ataxia in Europe, Middle East, South Asia (Indian subcontinent), and North Africa, lower prevalence in Mexico. Not documented in Southeast Asia, sub-Saharan Africa, or among Native Americans.

INVENTION

USE OF ERYTHROPOIETIN FOR THE TREATMENT OF FRIEDREICH´S ATAXIA:

- In addition to its neuro- and cardioprotective properties recombinant human erythropoietin (rhuEPO) significantly increases frataxin expression in primary lymphocytes from FA-patients, and other cell types like neurons and primary human cardiac cells in vitro. *(Sturm et.al 2005, Eur. J.Clin. Invest. 35(11):711-7)* and Carbamylated erythropoietin increases frataxin independent from the erythropoietin receptor (Sturm et al, Eur J Clin Invest 2010; 40 (6): 561-565)
- rhuEPO was successfully tested as a treatment for Friedreich`s ataxia in Phase IIa/IIb and dosefinding studies.

REFERENCE:
015.04

DEVELOPMENT STATUS:
- Dosefinding studies, Phase IIa/IIb, significant neurological improvement within 8 months of treatment assessed by two ataxia rating scales.
- Biomarkers show an increase in frataxin and reduction of oxidative stress.
- RhuEPO could obtain orphan drug designation from FDA/EMA for Friedreich`s ataxia.

COOPERATION OPTIONS:
- Development partnership
- License agreement
- Patent sale or other

IPR:
Patents granted
EP1812043,
US7790675,
AU2005304016,
AT500929,
CA2587188,
NZ555178

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