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# A randomized, double-blind, placebo-controlled trial of deferiprone in patients with pantothenate kinase-associated neurodegeneration (PKAN)

**Submission date**
08/11/2013

**Recruitment status**
No longer recruiting

**Registration date**
08/11/2013

**Overall study status**
Completed

**Last Edited**
09/08/2019

**Condition category**
Nervous System Diseases

[_] Prospectively registered
[_] Protocol
[_] Statistical analysis plan
[X] Results
[_] Individual participant data

**Plain English summary of protocol**
Not provided at time of registration

## Contact information

**Type(s)**
Scientific

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## Additional identifiers

**Clinical Trials Information System (CTIS)**
2012-000845-11

**ClinicalTrials.gov (NCT)**
NCT01741532

### Protocol serial number
15679

# Study information

### Scientific Title
TIRCON: A randomized, double-blind, placebo-controlled trial of deferiprone in patients with pantothenate kinase-associated neurodegeneration (PKAN)

### Acronym
TIRCON

### Study objectives
1. To evaluate the change in severity of dystonia (BAD scale) in patients with PKAN treated with deferiprone for 18 months compared to placebo.
2. To evaluate the patients global impression of conditions improvement in patients treated with deferiprone for 18 months compared to placebo (PGI-I).

### Ethics approval required
Old ethics approval format

### Ethics approval(s)
13/YH/0171

### Study design
Randomised; Interventional; Design type: Treatment

### Primary study design
Interventional

### Study type(s)
Treatment

### Health condition(s) or problem(s) studied
Topic: Medicines for Children Research Network; Subtopic: All Diagnoses; Disease: All Diseases

### Interventions
Main Intervention, Haematology
UPDRS (Unified Parkinson's Disease Rating Scale)
PK Sample collection (in a subset of patients)
Genetic Sample (only taken in patients who experience neutropenia)

### Intervention Type
Other

### Phase
Phase III

### Primary outcome(s)

Change in the BarryAlbrightDystonia Scale (BAD) total score from baseline to month 18 in patients

### Key secondary outcome(s))
Not provided at time of registration

### Completion date
01/07/2015

## Eligibility

### Key inclusion criteria
1. Males and females 4 years of age and older at screening visit
2 .Patients must have PKAN, confirmed by genetic testing
3. Patients having a BAD total score > = 3 at the screening visit
4. Patients who have Deep Brain Stimulation (DBS) systems or baclofen pumps in place will be eligible for the study, but they must have had a stable setting for at least 2 months prior to the screening visit and stimulation parameters /pump settings must remain stable for the duration of the trial. Enrollment of nonDBS patients will be given priority in order to ensure the majority can undergo imaging
5. Potentially sexually active female patients of childbearing potential must have a negative pregnancy test result at Screening Visit (if applicable; in cases where the Investigator determines there is no reasonable risk of pregnancy because of significant incapacity, pregnancy testing will not be performed)
6. Fertile potentially sexually active males must use an effective method of contraception or must confirm partners use of effective contraception
7. Informed consent/assent obtained before any studyrelated activities are undertaken
8. Ability and willingness to adhere to the protocol including appointments and evaluation schedule

### Participant type(s)
Patient

### Healthy volunteers allowed
No

### Age group
Child

### Lower age limit
4 years

### Sex
All

### Key exclusion criteria
1. Evidence of iron deficiency defined by Fe:TIBC ratio <15%, or serum ferritin < 12 ng/mL
2. Treatment with deferiprone in the past 12 months
3. Previous failure of treatment with deferiprone, or previous discontinuation of treatment with deferiprone due to adverse events

4. Evidence of abnormal liver or renal function (serum liver enzyme level(s) > 3 times upper limit of normal at screening) or abnormal creatinine levels at screening visit
5. Disorders associated with neutropenia (absolute neutrophil count (ANC) < 1.5 x 109/L) or thrombocytopenia (platelet count < 50 x 109/L) in the 12 months preceding the initiation of the study medication. Exception: for patients whose neutropenia was attributed by the treating physician to episodes of infection or to drugs associated with a decline in the neutrophil count and in whom ANC has fully recovered at the screening visit
6. Pregnant, breastfeeding, or planning to become pregnant during the study
7. Initiation or discontinuation of treatment with baclofen, trihexyphenidyl, clonazepam, tizanidine within 30 days prior to baseline; and initiation or discontinuation of treatment with tetrabenazine within 90 days prior to baseline
8. Treatment with an investigational drug within 30 days or 5 halflives
(whichever is longer) preceding the baseline
9. Currently taking iron chelators
10. Patients who, in the opinion of the physician, represent a high medical or psychological risk
11. History of or active drug or alcohol use or dependence that, in the opinion of the site investigator, would interfere with adherence to study requirements
12. Patients and patient's legal representative (if applicable) with a mental incapacity, unwillingness or language barriers precluding adequate understanding or cooperation.
13. Baclofen pump placement less than two months prior to the beginning of the study

### Date of first enrolment
01/07/2013

### Date of final enrolment
01/07/2015

## Locations

### Countries of recruitment
United Kingdom

England

### Study participating centre
### Institute of Health and Society
Newcastle Upon Tyne
United Kingdom
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## Sponsor information

### Organisation
Newcastle upon Tyne Hospitals NHS Foundation Trust (UK)

### ROR

https://ror.org/05p40t847

# Funder(s)

**Funder type**
Government

**Funder Name**
European Commission

**Alternative Name(s)**
European Union, Comisión Europea, Europäische Kommission, EU-Kommissionen, Euroopa Komisjoni, EC, EU

**Funding Body Type**
Government organisation

**Funding Body Subtype**
National government

**Location**

# Results and Publications

## Individual participant data (IPD) sharing plan

## IPD sharing plan summary

## Study outputs

| Output type | Details | Date created | Date added | Peer reviewed? | Patient-facing? |
|---|---|---|---|---|---|
| Results article | results | 01/07/2019 | 09/08/2019 | Yes | No |
| Basic results | | | 09/08/2019 | No | No |
| HRA research summary | | | 28/06/2023 | No | No |
| Participant information sheet | Participant information sheet | 11/11/2025 | 11/11/2025 | No | Yes |