ISRCTN72904618 https://doi.org/10.1186/ISRCTN72904618

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

EudraCT/CTIS number
2012-000845-11

IRAS number

ClinicalTrials.gov number
NCT01741532

Secondary identifying numbers
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

Secondary study design
Randomised controlled trial

Study setting(s)
Hospital

Study type(s)
Treatment

Participant information sheet
Not available in web format, please use the contact details to request a patient information sheet

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

Secondary outcome measures
Not provided at time of registration

Overall study start date
01/07/2013

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

Age group
Child

Lower age limit
4 Years

Sex
Both

Target number of participants
Planned Sample Size: 90; UK Sample Size: 8

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
England

United Kingdom

Study participating centre

Institute of Health and Society
Newcastle Upon Tyne
United Kingdom
NE2 4HH

Sponsor information

Organisation
Newcastle upon Tyne Hospitals NHS Foundation Trust (UK)

Sponsor details
New Victoria Wing
Queen Victoria Road
Newcastle Upon Tyne
England
United Kingdom
NE1 4LP

Sponsor type
Hospital/treatment centre

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, Ευρωπαϊκής Επιτροπής, Европейската комисия, Evropské komise, Commission européenne, Choimisiúin Eorpaigh, Europskoj komisiji, Commissione europea, La Commissione europea, Eiropas Komisiju, Europos Komisijos, Európai Bizottságról, Europese Commissie, Komisja Europejska, Comissão Europeia, Comisia Europeană, Európskej komisii, Evropski komisiji, Euroopan komission, Europeiska kommissionen, EC, EU

Funding Body Type
Government organisation

Funding Body Subtype

National government

Location

Results and Publications

Publication and dissemination plan
Not provided at time of registration

Intention to publish date

Individual participant data (IPD) sharing plan

IPD sharing plan summary
Not provided at time of registration

Study outputs

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