Clinical trial results:
A pilot clinical trial with the iron chelator Deferiprone in Parkinson's disease

Summary

EudraCT number	2011-001148-31
Trial protocol	GB
Global end of trial date	04 December 2014

Results information

Result version number	v1 (current)
This version publication date	25 March 2020
First version publication date	25 March 2020

Trial information

Trial identification

Sponsor protocol code	11/SC/0101

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01539837
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Imperial College London
Sponsor organisation address	South Kensington Campus, London, United Kingdom, SW7 2AZ
Public contact	Professor David Dexter, Imperial College London, +44 (0)20 7594 6665, d.dexter@imperial.ac.uk
Scientific contact	Professor David Dexter, Imperial College London, +44 (0)20 7594 6665, d.dexter@imperial.ac.uk

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	08 December 2015
Is this the analysis of the primary completion data?	Yes
Primary completion date	10 September 2014
Global end of trial reached?	Yes
Global end of trial date	04 December 2014
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To assess whether 6 month treatment with Deferiprone is well tolerated by PD patients and assess whether such treatment removes excess iron in the brain area affected in PD, the substantia nigra, as assessed by MRI.

Protection of trial subjects:

Anti-Parkinsonian medication dosages were maintained at a constant level for each individual throughout the study.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	13 February 2012
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	United Kingdom: 22
Worldwide total number of subjects	22
EEA total number of subjects	22

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)	10
From 65 to 84 years	12
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

22 participants were recruited by between 18/04/2012 – 27/03/2013

Pre-assignment

Screening details:

The original group of 22 patients, 19 completed the 6-month course of deferiprone, with three brain MRI scans, at 0, 3 and 6 months.

Period 1

Period 1 title	Overall (overall period)
Is this the baseline period?	Yes
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator, Carer

Blinding implementation details:

The code for the randomisation was only disclosed at the conclusion of the trial or if the patient experienced unexpected adverse side effects. Patients, care providers and those assessing clinical outcomes were
blinded to the intervention given.

Arms

Are arms mutually exclusive?	Yes
Arm title	Placebo

Arm description:

Participants received placebo

Arm type	Placebo
Investigational medicinal product name	Feriprox
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

20mg/kg/day for 6 month

Arm title	Deferiprone 20mg

Arm description: -

Arm type	Experimental
Investigational medicinal product name	Deferiprone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

20mg/kg/day for 6 months

Arm title	Deferiprone 30mg

Arm description: -

Arm type	Experimental

Investigational medicinal product name	Deferiprone
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

30mg/kg/day for 6 months

Number of subjects in period 1	Placebo	Deferiprone 20mg	Deferiprone 30mg
Started	8	7	7
Completed	8	6	5
Not completed	0	1	2
bad compliance	-	-	1
reduce neutrophil level	-	1	1

Baseline characteristics

Reporting groups	
Reporting group title	Placebo
Reporting group description: Participants received placebo	
Reporting group title	Deferiprone 20mg
Reporting group description: -	
Reporting group title	Deferiprone 30mg
Reporting group description: -	

Reporting group values	Placebo	Deferiprone 20mg	Deferiprone 30mg
Number of subjects	8	7	7
Age categorical Units: Subjects			
Adults (18-64 years)	2	1	5
From 65-84 years	6	6	2
Age continuous Units: years			
arithmetic mean	64.38	68.57	62.85
standard deviation	± 3.23	± 2.17	± 2.74
Gender categorical Units: Subjects			
Female	5	3	2
Male	3	4	5
Parkinson’s disease duration Units: years			
arithmetic mean	3.54	2.82	3.02
standard deviation	± 0.34	± 0.69	± 2.69

Reporting group values	Total		
Number of subjects	22		
Age categorical Units: Subjects			
Adults (18-64 years)	8		
From 65-84 years	14		
Age continuous Units: years arithmetic mean standard deviation	- -		
Gender categorical Units: Subjects			
Female	10		
Male	12		
Parkinson’s disease duration Units: years arithmetic mean standard deviation	- -		

End points

End points reporting groups

Reporting group title	Placebo
Reporting group description: Participants received placebo	
Reporting group title	Deferiprone 20mg
Reporting group description: -	
Reporting group title	Deferiprone 30mg
Reporting group description: -	

Primary: Numbers of Serious Adverse Event

End point title	Numbers of Serious Adverse Event[1]
End point description:	
End point type	Primary
End point timeframe: 6 months	

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: Pilot study, low participants number, no statistical analyses.

End point values	Placebo	Deferiprone 20mg	Deferiprone 30mg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	8	7	7	
Units: Number of participants	0	0	0	

Statistical analyses

No statistical analyses for this end point

Secondary: Iron Concentrations in the Dentate Nucleus

End point title	Iron Concentrations in the Dentate Nucleus
End point description:	
End point type	Secondary
End point timeframe: 6 months	

End point values	Placebo	Deferiprone 20mg	Deferiprone 30mg	
Subject group type	Reporting group	Reporting group	Reporting group	
Number of subjects analysed	8	7	7	
Units: T2*ms				
arithmetic mean (standard error)	30.74 (± 0.65)	30.59 (± 0.87)	29.86 (± 1.10)	

Statistical analyses

Statistical analysis title	Iron Concentrations in the Dentate Nucleus
Comparison groups	Deferiprone 20mg v Deferiprone 30mg v Placebo
Number of subjects included in analysis	22
Analysis specification	Post-hoc
Analysis type	superiority
P-value	< 0.01
Method	Pairwise comparisons,post-hoc Bonferoni

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 6 months	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	10
Reporting groups	
Reporting group title	Placebo
Reporting group description: Participants received placebo	
Reporting group title	Deferiprone 20mg
Reporting group description: -	
Reporting group title	Deferiprone 30mg
Reporting group description: -	

Serious adverse events	Placebo	Deferiprone 20mg	Deferiprone 30mg
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 8 (0.00%)	0 / 7 (0.00%)	0 / 7 (0.00%)
number of deaths (all causes)	0	0	0
number of deaths resulting from adverse events	0	0	0

Frequency threshold for reporting non-serious adverse events: 0 %

Non-serious adverse events	Placebo	Deferiprone 20mg	Deferiprone 30mg
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 8 (0.00%)	6 / 7 (85.71%)	6 / 7 (85.71%)
Blood and lymphatic system disorders			
Decline in white cell counts			
subjects affected / exposed	0 / 8 (0.00%)	1 / 7 (14.29%)	1 / 7 (14.29%)
occurrences (all)	0	1	1
Gastrointestinal disorders			
Gastrointestinal upset			
subjects affected / exposed	0 / 8 (0.00%)	1 / 7 (14.29%)	2 / 7 (28.57%)
occurrences (all)	0	1	2
Musculoskeletal and connective tissue disorders			

Muscular joint pain subjects affected / exposed occurrences (all)	0 / 8 (0.00%) 0	4 / 7 (57.14%) 4	3 / 7 (42.86%) 3

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/28469157