Clinical trial results:
Can stimulation of the nNOS system in muscle disease with nNOS insufficiency improve heart and skeletal muscle function and cognition?

Summary

EudraCT number	2010-024659-10
Trial protocol	DK
Global end of trial date	17 January 2013

Results information

Result version number	v1 (current)
This version publication date	13 December 2021
First version publication date	13 December 2021
Summary attachment (see zip file)	Sildenafil published paper (Pub paper.pdf)

Trial information

Trial identification

Sponsor protocol code	2010-024659-10

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT01350154
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Righospitalet
Sponsor organisation address	6921NM, Rigshospitalet, KBH Ø, Denmark, 2100
Public contact	Neuromuscular Research Unit and Dept Neurol, Rigshospitalet, Copenhagen University Hospital, Neuromuscular Research Unit and Dept Neurol, Rigshospitalet, Copenhagen University Hospital, 0045 35453545, nanna.witting@regionh.dk
Scientific contact	Neuromuscular Research Unit and Dept Neurol, Rigshospitalet, Copenhagen University Hospital, Neuromuscular Research Unit and Dept Neurol, Rigshospitalet, Copenhagen University Hospital, 35456921 35453545, nanna.witting@regionh.dk

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 May 2013
Is this the analysis of the primary completion data?	Yes
Primary completion date	17 January 2013
Global end of trial reached?	Yes
Global end of trial date	17 January 2013
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

There are 3 parallel sub-studies
Substudy 1;Effect of Sildenafil on handgrip capacity
Substudy 2; heart enddiastolic volume
Substudy 3; Stimulated brain blood flow and cognition

Protection of trial subjects:

Minimal intervention

Background therapy:

None

Evidence for comparator:

Please see Ann Neurol . 2014 Oct;76(4):550-7. doi: 10.1002/ana.24216. Epub 2014 Jul 15.
Effect of sildenafil on skeletal and cardiac muscle in Becker muscular dystrophy
Nanna Witting et al

Actual start date of recruitment	23 August 2011
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	Yes

Notes:

Population of trial subjects

Subjects enrolled per country

Country: Number of subjects enrolled	Denmark: 16
Worldwide total number of subjects	16
EEA total number of subjects	16

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)	16

From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Ann Neurol
. 2014 Oct;76(4):550-7. doi: 10.1002/ana.24216. Epub 2014 Jul 15.
Effect of sildenafil on skeletal and cardiac muscle in Becker muscular dystrophy
Nanna Witting 1, Christina Kruuse, Bo Nyhuus, Kira P Prahm, Gulsenay Citirak, Stine J Lundgaard, Sebastian von Huth, Niels Vejlstrup, Ulrich Lindberg, Thomas O Krag, John Vissing

Pre-assignment

Screening details:

Ann Neurol
. 2014 Oct;76(4):550-7. doi: 10.1002/ana.24216. Epub 2014 Jul 15.
Effect of sildenafil on skeletal and cardiac muscle in Becker muscular dystrophy
Nanna Witting 1, Christina Kruuse, Bo Nyhuus, Kira P Prahm, Gulsenay Citirak, Stine J Lundgaard, Sebastian von Huth, Niels Vejlstrup, Ulrich Lindberg, Thomas O Krag, John Vissing

Period 1

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

Arms

Arm title	Active 1
Arm description: -	
Arm type	Placebo
Investigational medicinal product name	placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

cc to published paper attached

Number of subjects in period 1	Active 1
Started	16
Completed	16

Period 2

Period 2 title	Period 2
Is this the baseline period?	No
Allocation method	Randomised - controlled
Blinding used	Double blind
Roles blinded	Subject, Investigator

Arms

Arm title	Active 2

Arm description: -

Arm type	Active comparator
Investigational medicinal product name	Sildenafil
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Tablet
Routes of administration	Oral use

Dosage and administration details:

Please see
Ann Neurol
. 2014 Oct;76(4):550-7. doi: 10.1002/ana.24216. Epub 2014 Jul 15.
Effect of sildenafil on skeletal and cardiac muscle in Becker muscular dystrophy
Nanna Witting 1, Christina Kruuse, Bo Nyhuus, Kira P Prahm, Gulsenay Citirak, Stine J Lundgaard, Sebastian von Huth, Niels Vejlstrup, Ulrich Lindberg, Thomas O Krag, John Vissing

Number of subjects in period 2	Active 2
Started	16
Completed	16

Baseline characteristics

Reporting groups

Reporting group title	1.period

Reporting group description: -

Reporting group values	1.period	Total	
Number of subjects	16	16	
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)	0	0	
From 65-84 years	16	16	
85 years and over	0	0	
Gender categorical Units: Subjects			
Female	0	0	
Male	16	16	

Subject analysis sets

Subject analysis set title	brachial artery blood flow during maximal handgrip
Subject analysis set type	Intention-to-treat

Subject analysis set description:
brachial artery blood flow during maximal handgrip

Reporting group values	brachial artery blood flow during maximal handgrip		
Number of subjects	16		
Age categorical Units: Subjects			
In utero	0		
Preterm newborn infants (gestational age < 37 wks)	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)	0		
From 65-84 years	16		
85 years and over	0		

Gender categorical Units: Subjects			
Female	0		
Male	16		

End points

End points reporting groups

Reporting group title	Active 1
Reporting group description: -	
Reporting group title	Active 2
Reporting group description: -	
Subject analysis set title	brachial artery blood flow during maximal handgrip
Subject analysis set type	Intention-to-treat
Subject analysis set description: brachial artery blood flow during maximal handgrip	

Primary: brachial artery blood flow during maximal handgrip

End point title	brachial artery blood flow during maximal handgrip
End point description: brachial artery blood flow during maximal handgrip	
End point type	Primary
End point timeframe: 4W	

End point values	Active 1	Active 2	brachial artery blood flow during maximal handgrip	
Subject group type	Reporting group	Reporting group	Subject analysis set	
Number of subjects analysed	16	16	16	
Units: ml/min				
number (not applicable)	16	16	16	

Statistical analyses

Statistical analysis title	one-way ANOVA for repeated measures
Comparison groups	Active 1 v Active 2 v brachial artery blood flow during maximal handgrip
Number of subjects included in analysis	48
Analysis specification	Pre-specified
Analysis type	other
P-value	< 0.05
Method	ANOVA

Adverse events

Adverse events information[1]	
Timeframe for reporting adverse events: 12W	
Assessment type	Systematic

Dictionary used	
Dictionary name	None
Dictionary version	0

Reporting groups	
Reporting group title	period 2

Reporting group description: -

Serious adverse events	period 2		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 16 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events			

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

Non-serious adverse events	period 2		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	0 / 16 (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: Please see published paper attached

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