Clinical trial results:
Follow-up studie: Metformin therapie bij kinderen en adolescenten met neurogene of neuromusculaire aandoeningen

Summary

EudraCT number	2009-009278-29
Trial protocol	BE
Global end of trial date	27 August 2009

Results information

Result version number	v1 (current)
This version publication date	23 July 2023
First version publication date	23 July 2023
Summary attachment (see zip file)	metformin in children with motor deficit (fulltextmetformin.pdf)

Trial information

Trial identification

Sponsor protocol code	S51477

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	UZ LEuven
Sponsor organisation address	herestraat 49, Leuven, Belgium, 3000
Public contact	Kristina.casteels@uzleuven.be, Kristina.casteels@uzleuven.be, 32 16343801, kristina.casteels@uzleuven.be
Scientific contact	Kristina.casteels@uzleuven.be, Kristina.casteels@uzleuven.be, 0032 16343801, kristina.casteels@uzleuven.be

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	30 December 2008
Is this the analysis of the primary completion data?	Yes
Primary completion date	30 December 2008
Global end of trial reached?	Yes
Global end of trial date	27 August 2009
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

Effect of metformin on body composition and insulin resistance

Protection of trial subjects:

Clinical examination and blood test

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	07 February 2005
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	Belgium: 42
Worldwide total number of subjects	42
EEA total number of subjects	42

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)	12
Adolescents (12-17 years)	30
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment

Recruitment details:

Patients were recruited in the neurological deparmtent of our hospital

Pre-assignment

Screening details:

Children with Duchenne Muscular Dystrophy and spina bifida.

Period 1

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

Blinding implementation details:

Randomization was stratified by diagnostic subgroup

Arms

Are arms mutually exclusive?	Yes
Arm title	Metformin

Arm description:

Metformin

Arm type	Active comparator
Investigational medicinal product name	Metformine
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Coated tablet
Routes of administration	Oral use

Dosage and administration details:

850 mg 1/day

Arm title	placebo

Arm description:

placebo

Arm type	Placebo
Investigational medicinal product name	placebo
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Coated tablet
Routes of administration	Oral use

Dosage and administration details:

1/day

Number of subjects in period 1	Metformin	placebo
Started	23	19
Completed	21	14
Not completed	2	5
Consent withdrawn by subject	1	-
Adverse event, non-fatal	1	1
social reasons	-	3
Lost to follow-up	-	1

Baseline characteristics

Reporting groups	
Reporting group title	overall trial

Reporting group description: -

Reporting group values	overall trial	Total	
Number of subjects	42	42	
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		0	
85 years and over		0	
Age continuous			
The mean age of the participants was 15,5 +/- 6,2 years			
Units: years			
geometric mean	15.5		
standard deviation	± 6.2	-	
Gender categorical Units: Subjects			
Female	23	23	
Male	19	19	

End points

End points reporting groups

Reporting group title	Metformin
Reporting group description: Metformin	
Reporting group title	placebo
Reporting group description: placebo	

Primary: weight loss by metformin

End point title	weight loss by metformin
End point description: mean difference of 2 kg within 6 months (p =0.007) in metformin group versus placebo	
End point type	Primary
End point timeframe: over twelve months	

End point values	Metformin	placebo		
Subject group type	Reporting group	Reporting group		
Number of subjects analysed	21	19		
Units: kilogram(s)				
geometric mean (confidence interval 100%)				
weight loss	0.32 (-0.54 to 1.19)	1.92 (0.98 to 2.87)		

Statistical analyses

Statistical analysis title	multivariate regression models
Comparison groups	Metformin v placebo
Number of subjects included in analysis	40
Analysis specification	Pre-specified
Analysis type	superiority
P-value	< 0.05
Method	Mixed models analysis
Parameter estimate	Mean difference (net)

Adverse events

Adverse events information	
Timeframe for reporting adverse events: 12 months of study	
Adverse event reporting additional description: nausea	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	2

Reporting groups	
Reporting group title	metformin
Reporting group description: 1 patient stopped medication due to nausea, diarrhea	
Reporting group title	placebo
Reporting group description: -	

Serious adverse events	metformin	placebo	
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 19 (0.00%)	0 / 23 (0.00%)	
number of deaths (all causes)	0	0	
number of deaths resulting from adverse events	0	0	

Frequency threshold for reporting non-serious adverse events: 5 %

Non-serious adverse events	metformin	placebo	
Total subjects affected by non-serious adverse events			
subjects affected / exposed	1 / 19 (5.26%)	0 / 23 (0.00%)	
Gastrointestinal disorders			
Nausea	Additional description: nausea and diarrhea		
subjects affected / exposed	1 / 19 (5.26%)	0 / 23 (0.00%)	
occurrences (all)	1	0	

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