Clinical trial results:
Study of the preliminary efficacy and safety of topical cysteamine formulated in viscous solution in cystinosis patients

Summary

EudraCT number	2013-003228-35
Trial protocol	ES
Global end of trial date	23 July 2015

Results information

Result version number	v1 (current)
This version publication date	02 July 2021
First version publication date	02 July 2021

Trial information

Trial identification

Sponsor protocol code	18072013

Additional study identifiers

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

Notes:

Sponsors

Sponsor organisation name	VHIR
Sponsor organisation address	Passeig Vall Hebron 119-129, Barcelona, Spain, 08035
Public contact	Study coordinator, VHIR, +34 934893166, nmartin@vhebron.net
Scientific contact	Study coordinator, VHIR, +34 934893166, joaquin.lopez.soriano@vhir.org

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	23 July 2015
Is this the analysis of the primary completion data?	Yes
Primary completion date	23 July 2015
Global end of trial reached?	Yes
Global end of trial date	23 July 2015
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

The main objective of the
study is to assess the efficacy of 0.55% cysteamine formulated in viscous solution compared with saline solution at the same dose, in corneal crystal deposition.

Protection of trial subjects:

Patients and parents were trained and informed about drop administration and possible adverse effects. Since there was no changes in the administered drug, no other measures were needed to implement.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	15 November 2013
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	Spain: 5
Worldwide total number of subjects	5
EEA total number of subjects	5

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)	2
Adolescents (12-17 years)	3
Adults (18-64 years)	0
From 65 to 84 years	0
85 years and over	0

Subject disposition

Recruitment	
Recruitment details: -	
Pre-assignment	
Screening details: -	
Pre-assignment period milestones	
Number of subjects started	5
Number of subjects completed	5

Period 1	
Period 1 title	Cysteamine viscous solutions (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded
Arms	
Arm title	Viscous eyedrops

Arm description:

Comparison of cysteamine saline solution versus viscous solution

Arm type	Experimental
Investigational medicinal product name	Mercaptamine
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Ear/eye/nasal drops, solution
Routes of administration	Ophthalmic use

Dosage and administration details:

0.55% viscous solution of cysteamine according to previous prescription for 3 months. Followed by 0.55% viscous solution of cysteamine four times a day for 3 months

Number of subjects in period 1	Viscous eyedrops
Started	5
Completed	5

Baseline characteristics

Reporting groups

Reporting group title	Cysteamine viscous solutions

Reporting group description: -

Reporting group values	Cysteamine viscous solutions	Total	
Number of subjects	5	5	
Age categorical Units: Subjects			
children 5-17y	5	5	
Gender categorical Units: Subjects			
Female	1	1	
Male	4	4	
Cystinosis disease			
Cystinosis diagnostic			
Units: Subjects			
Disease	5	5	

Subject analysis sets

Subject analysis set title	Efficiency and safety of cysteamine drops
Subject analysis set type	Full analysis

Subject analysis set description:
Children with cystinosis

Reporting group values	Efficiency and safety of cysteamine drops		
Number of subjects	5		
Age categorical Units: Subjects			
children 5-17y	5		
Gender categorical Units: Subjects			
Female	1		
Male	4		
Cystinosis disease			
Cystinosis diagnostic			
Units: Subjects			
Disease	5		

End points

End points reporting groups

Reporting group title	Viscous eyedrops
Reporting group description:	
Comparison of cysteamine saline solution versus viscous solution	
Subject analysis set title	Efficiency and safety of cysteamine drops
Subject analysis set type	Full analysis
Subject analysis set description:	
Children with cystinosis	

Primary: Corneal cystine crystal deposits

End point title	Corneal cystine crystal deposits[1]
End point description:	
End point type	Primary
End point timeframe:	
At the end of 6 months treatment	

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: 0.55% cysteamine viscous solution showed similar effects on corneal cistine crystals than saline solution. No changes were observed by modyfing frequency of instillations. Low number of enrolled patients limits the conclusions of the study. Collaboration of patients was a problem to obtain objective results

End point values	Viscous eyedrops			
Subject group type	Reporting group			
Number of subjects analysed	5			
Units: Number of crystals	5			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: During the extension of the study	
Assessment type	Systematic
Dictionary used	
Dictionary name	MedDRA
Dictionary version	2
Reporting groups	
Reporting group title	Total adverse events

Reporting group description: -

Serious adverse events	Total adverse events		
Total subjects affected by serious adverse events			
subjects affected / exposed	2 / 5 (40.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		
Nervous system disorders			
Intracranial pressure increased			
subjects affected / exposed	2 / 5 (40.00%)		
occurrences causally related to treatment / all	0 / 2		
deaths causally related to treatment / all	0 / 0		
Renal and urinary disorders			
Transplant dysfunction			
subjects affected / exposed	1 / 5 (20.00%)		
occurrences causally related to treatment / all	0 / 1		
deaths causally related to treatment / all	0 / 0		

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

Non-serious adverse events	Total adverse events		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	5 / 5 (100.00%)		
Eye disorders			
Itching			
subjects affected / exposed	5 / 5 (100.00%)		
occurrences (all)	5		

Vision blurred subjects affected / exposed occurrences (all)	3 / 5 (60.00%) 3		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
05 January 2015	Dr Marisol Lopez Moreno was included as researcher in the study
12 January 2015	Confocal microscopy was done at Hospital Clínic (barcelona) instead of Casa Maternitat because the equipment was moved

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported