Clinical trial results:
A Phase 2, open-label, multi-centre study to assess the efficacy and safety of intravitreal THR-317 for the treatment of macular telangiectasia Type 1 (MacTel 1)

Summary

EudraCT number	2017-004010-26
Trial protocol	FR
Global end of trial date	22 November 2019

Results information

Result version number	v1 (current)
This version publication date	04 December 2020
First version publication date	04 December 2020

Trial information

Trial identification

Sponsor protocol code	THR-317-003

Additional study identifiers

ISRCTN number	-
ClinicalTrials.gov id (NCT number)	NCT03669393
WHO universal trial number (UTN)	-

Notes:

Sponsors

Sponsor organisation name	Oxurion NV
Sponsor organisation address	Gaston Geenslaan 1, Leuven, Belgium, 3001
Public contact	Global Clinical Development, Oxurion NV, 32 16751310, info@oxurion.com
Scientific contact	Global Clinical Development, Oxurion NV, 32 16751310, info@oxurion.com

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	16 January 2020
Is this the analysis of the primary completion data?	Yes
Primary completion date	22 November 2019
Global end of trial reached?	Yes
Global end of trial date	22 November 2019
Was the trial ended prematurely?	No

Notes:

General information about the trial

Main objective of the trial:

To evaluate the efficacy and safety of THR-317 for the treatment of MacTel 1

Protection of trial subjects:

All study procedures, including the intravitreal injections, were performed by qualified and trained personnel. Only eligible subjects were enrolled in the study and only subjects who did not meet any withdrawal criteria received repeat injections. All subjects were supervised in the immediate post-injection period with appropriate medical treatment readily available. Subjects were followed for 3 months after the last injection. Adverse events were recorded throughout the study period. At each study visit, a full ophthalmic examination, BCVA assessment and SD-OCT imaging were performed.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	18 September 2018
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	France: 3
Country: Number of subjects enrolled	Switzerland: 5
Worldwide total number of subjects	8
EEA total number of subjects	3

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)	6
From 65 to 84 years	2

85 years and over	0

Subject disposition

Recruitment

Recruitment details: -

Pre-assignment

Screening details:

The study included a Screening during which in- and exclusion criteria were checked

Period 1

Period 1 title	Overall Trial (overall period)
Is this the baseline period?	Yes
Allocation method	Not applicable
Blinding used	Not blinded

Arms

Arm title	THR-317

Arm description:

Subjects received 3 intravitreal injections of THR-317 8mg, approximately 1 month apart

Arm type	Experimental
Investigational medicinal product name	THR-317 8mg
Investigational medicinal product code	THR-317
Other name	
Pharmaceutical forms	Solution for injection
Routes of administration	Intravitreal use

Dosage and administration details:

intravitreal THR-317 8mg at Day 0, Month 1 and Month 2

Number of subjects in period 1	THR-317
Started	8
Completed	8

Baseline characteristics

Reporting groups	
Reporting group title	THR-317
Reporting group description:	
Subjects received 3 intravitreal injections of THR-317 8mg, approximately 1 month apart	

Reporting group values	THR-317	Total	
Number of subjects	8	8	
Age categorical Units: Subjects			
Adults (18-64 years)	6	6	
From 65-84 years	2	2	
Age continuous Units: years			
arithmetic mean	59.9		
standard deviation	± 7.61	-	
Gender categorical Units: Subjects			
Female	0	0	
Male	8	8	

End points

End points reporting groups

Reporting group title	THR-317
Reporting group description:	
Subjects received 3 intravitreal injections of THR-317 8mg, approximately 1 month apart	

Primary: Change from Baseline in CST, based on SD-OCT

End point title	Change from Baseline in CST, based on SD-OCT[1]
End point description:	
End point type	Primary
End point timeframe:	
Month 3	

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: This was an open-label, exploratory study with a single treatment arm and a limited number of subjects. This endpoint was evaluated only descriptively. No statistical hypothesis testing was performed.

End point values	THR-317			
Subject group type	Reporting group			
Number of subjects analysed	8			
Units: µm				
arithmetic mean (standard deviation)	23.9 (± 41.29)			

Statistical analyses

No statistical analyses for this end point

Secondary: Change from Baseline in BCVA

End point title	Change from Baseline in BCVA
End point description:	
End point type	Secondary
End point timeframe:	
Month 3	

End point values	THR-317			
Subject group type	Reporting group			
Number of subjects analysed	8			
Units: ETDRS letters				
arithmetic mean (standard deviation)	-2.8 (± 5.18)			

Statistical analyses

No statistical analyses for this end point

Secondary: Incidence of systemic and ocular (serious) adverse events ([S]AEs), from first study treatment up to the end of the study

End point title	Incidence of systemic and ocular (serious) adverse events ([S]AEs), from first study treatment up to the end of the study

End point description:

End point type	Secondary

End point timeframe:

From first study treatment up to end of the study

End point values	THR-317			
Subject group type	Reporting group			
Number of subjects analysed	8			
Units: subjects	3			

Statistical analyses

No statistical analyses for this end point

Adverse events

Adverse events information	
Timeframe for reporting adverse events: From first administration of study treatment up to end of study	
Assessment type	Systematic

Dictionary used	
Dictionary name	MedDRA
Dictionary version	22.0

Reporting groups	
Reporting group title	THR-317

Reporting group description: -

Serious adverse events	THR-317		
Total subjects affected by serious adverse events			
subjects affected / exposed	0 / 8 (0.00%)		
number of deaths (all causes)	0		
number of deaths resulting from adverse events	0		

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

Non-serious adverse events	THR-317		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	3 / 8 (37.50%)		
Injury, poisoning and procedural complications			
Post Procedural Complication			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Nervous system disorders			
Headache			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	2		
Eye disorders			
Visual Acuity Reduced			
subjects affected / exposed	1 / 8 (12.50%)		
occurrences (all)	1		
Vitritis			

subjects affected / exposed occurrences (all) Eye Pain subjects affected / exposed occurrences (all)	1 / 8 (12.50%) 1 1 / 8 (12.50%) 1		
Musculoskeletal and connective tissue disorders Musculoskeletal Pain subjects affected / exposed occurrences (all)	 1 / 8 (12.50%) 1		

More information

Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? Yes

Date	Amendment
11 October 2018	Addition of blood sampling for immunogenicity assessment.

Notes:

Interruptions (globally)

Were there any global interruptions to the trial? No

Limitations and caveats

None reported