



## Clinical trial results:

## Raxone® treatment for patients with dominant optic atrophy due to OPA1 gene mutation

### Summary

EudraCT number	2019-001493-28
Trial protocol	AT
Global end of trial date	25 April 2023

### Results information

Result version number	v1 (current)
This version publication date	27 April 2024
First version publication date	27 April 2024

### Trial information

#### Trial identification

Sponsor protocol code	OPA1
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#### Additional study identifiers

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

Notes:

#### Sponsors

Sponsor organisation name	Medical University of Graz
Sponsor organisation address	Neue Stiftingtalstraße 6, Graz, Austria, 8010
Public contact	Clinical trials information, Medizinische Universität Graz, Univ.-Augenklinik, 0043 0316385 82899, katharina.valentin@medunigraz.at
Scientific contact	Clinical trials information, Medizinische Universität Graz, Univ.-Augenklinik, 0043 0316385 82899, katharina.valentin@medunigraz.at

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:

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**Results analysis stage**

Analysis stage	Final
Date of interim/final analysis	26 April 2022
Is this the analysis of the primary completion data?	Yes
Primary completion date	26 April 2022
Global end of trial reached?	Yes
Global end of trial date	25 April 2023
Was the trial ended prematurely?	No

Notes:

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**General information about the trial**

Main objective of the trial:

Evaluation of the therapeutic effect of 900mg Raxone® per day regarding visual acuity in ADOA patients with OPA1 mutation within a 12 month period.

Protection of trial subjects:

The study was conducted according to GCP and local regulations.

Background therapy: -

Evidence for comparator: -

Actual start date of recruitment	01 October 2020
Long term follow-up planned	No
Independent data monitoring committee (IDMC) involvement?	No

Notes:

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**Population of trial subjects**

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**Subjects enrolled per country**

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

Notes:

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**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)	2
Adults (18-64 years)	14
From 65 to 84 years	0
85 years and over	0

## Subject disposition

### Recruitment

Recruitment details:

Recruitment period was between 10/2020 and 05/2021

### Pre-assignment

Screening details:

16 patients have signed the informed consent, 1 patient was lost to follow-up. There were no screening failures.

### Period 1

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

### Arms

<b>Arm title</b>	Raxone
Arm description: -	
Arm type	Experimental
Investigational medicinal product name	Raxone 150 mg film-coated tablets
Investigational medicinal product code	
Other name	
Pharmaceutical forms	Film-coated tablet
Routes of administration	Oral use

Dosage and administration details:

150 mg 3x2 daily for 12 months

Number of subjects in period 1	Raxone
Started	16
Completed	15
Not completed	1
Lost to follow-up	1

## Baseline characteristics

## End points

### End points reporting groups

Reporting group title	Raxone
Reporting group description: -	

### Primary: Best recovery/ least deterioration of visual acuity from baseline to 12 months measured with ETDRS charts on the right eye

End point title	Best recovery/ least deterioration of visual acuity from baseline to 12 months measured with ETDRS charts on the right eye <sup>[1]</sup>
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End point description:

End point type	Primary
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End point timeframe:

Baseline and follow-up

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: single arm study - was not possible to enter data in the systeme

End point values	Raxone			
Subject group type	Reporting group			
Number of subjects analysed	16			
Units: logMAR				
arithmetic mean (standard deviation)				
Baseline	0.52 (± 0.32)			
Follow-up	0.44 (± 0.32)			

### Statistical analyses

No statistical analyses for this end point

### Primary: Best recovery/ least deterioration of visual acuity from baseline to 12 months measured with ETDRS charts on the left eye

End point title	Best recovery/ least deterioration of visual acuity from baseline to 12 months measured with ETDRS charts on the left eye <sup>[2]</sup>
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End point description:

End point type	Primary
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End point timeframe:

Baseline and follow-up

Notes:

[2] - 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: single arm study - was not possible to enter data in the systeme

End point values	Raxone			
Subject group type	Reporting group			
Number of subjects analysed	16			
Units: logMAR				
arithmetic mean (standard deviation)				
Baseline	0.54 (± 0.36)			
Follow-up	0.48 (± 0.38)			

## Statistical analyses

No statistical analyses for this end point

## Primary: Best recovery/ least deterioration of visual acuity from baseline to 12 months measured with ETDRS charts on the better-seeing eye

End point title	Best recovery/ least deterioration of visual acuity from baseline to 12 months measured with ETDRS charts on the better-seeing eye <sup>[3]</sup>
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End point description:

End point type	Primary
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End point timeframe:

Baseline  
Follow-up

Notes:

[3] - 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: single arm study - was not possible to enter data in the systeme

End point values	Raxone			
Subject group type	Reporting group			
Number of subjects analysed	16			
Units: logMAR				
arithmetic mean (standard deviation)				
Baseline	0.46 (± 0.32)			
Follow-up	0.41 (± 0.35)			

## Statistical analyses

No statistical analyses for this end point

## Adverse events

### Adverse events information

Timeframe for reporting adverse events:

12 months

Assessment type	Systematic
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### Dictionary used

Dictionary name	MedDRA
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Dictionary version	26.1
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### Reporting groups

Reporting group title	Raxone
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Reporting group description: -

Serious adverse events	Raxone		
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	0		

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

Non-serious adverse events	Raxone		
Total subjects affected by non-serious adverse events			
subjects affected / exposed	9 / 16 (56.25%)		
General disorders and administration site conditions			
Headache			
subjects affected / exposed	2 / 16 (12.50%)		
occurrences (all)	2		
Anorexia			
subjects affected / exposed	1 / 16 (6.25%)		
occurrences (all)	1		
Sore throat			
subjects affected / exposed	1 / 16 (6.25%)		
occurrences (all)	1		
Blood and lymphatic system disorders			

anemia			
subjects affected / exposed	2 / 16 (12.50%)		
occurrences (all)	2		
Elevation of liver parameters			
subjects affected / exposed	2 / 16 (12.50%)		
occurrences (all)	2		
Gastrointestinal disorders			
Pyrosis			
subjects affected / exposed	1 / 16 (6.25%)		
occurrences (all)	1		



## More information

### Substantial protocol amendments (globally)

Were there any global substantial amendments to the protocol? No

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### Interruptions (globally)

Were there any global interruptions to the trial? No

### Limitations and caveats

None reported