

Clinical trial results:

An Open-Label, Single Arm, Multiple Dose, Dose Escalation Study to Evaluate the Safety and Tolerability of QR-110 in Subjects with Leber's Congenital Amaurosis (LCA) due to c.2991+1655A>G Mutation (p. Cys998X) in the CEP290 Gene

Summary

Results information	

Trial information

Trial identification

Additional study identifiers

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Sponsors

Paediatric regulatory details	

Results analysis stage	

General information about the trial

Population of trial subjects	
Subjects enrolled per country	

Subjects enrolled per age group	

Recruitment

Pre-assignment

Period 1

Arms	
Arm title	

Arm title	

Number of subjects in period 1	

Baseline characteristics

Reporting groups

Reporting group values		

End points reporting groups	

End point values		

Primary: Frequency of Ocular AEs in Contralateral Eyes (CE)		

End point values		

Secondary: Frequency of non-ocular AEs		

End point values		

Secondary: Severity of non-ocular AEs		

End point values		

Secondary: BCVA change from baseline in Treatment Eye (TE)		

End point values		

Secondary: Red FST Change from	Baseline in Treatment Eye (TE)

End point values		

Secondary: Blue FST Change from Baseline in Treatment Eye (TE)		

End point values		

Secondary: Mobility Course Composite Score Change from Baseline in Treatment Eye (TE)

End point values		

Secondary: Red FST Change from Baseline in Contralateral E	iye (CE	.)
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End point values		

Statistical analyses

Secondary: BCVA change from baseline in Contralateral Eye (CE)

End point values		

Secondary: Blue FST Change from Baseline in Contralateral Eye (CE)	

End point values		

Secondary: Mobility Course Composite Score Change from Baseline in Co	ntralateral
Eye (CE)	

End point values		

Secondary: Serum pharmacokinetics		

End point values		

Adverse events information

Dictionary used

 Bictionary used

 Reporting groups

Serious adverse events		
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Non-serious adverse events		
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More information

Substantial protocol amendments (globally)

Date	Amendment

Interruptions (globally)

Limitations and caveats

Online references