

Illuminate leaves Proqr in darkness



[Elizabeth Cairns](#)

Proqr [had hoped that both doses](#) of its gene therapy seprofarsen might prove effective in its [pivotal phase 2/3 Illuminate trial](#) in the rare eye disorder CEP290-mediated Leber’s congenital amaurosis 10 (LCA10). Today it admitted that neither was. The washout was total, with no significant difference between either dose, or between the doses and sham treatment, on the primary or any of the secondary endpoints, and the company’s stock plummeted 74%. On a call today Proqr did not rule out subgroup dredges, but it would be wildly optimistic to hope for anything here. The question for the company is what the failure means for the rest of its pipeline, and while it insisted that there was limited readthrough – and that it has cash to last until 2024 – it will have a nervous wait for readouts of its trials. Topline data from the phase 1/2 trial of QR-504a in Fuchs endothelial corneal dystrophy, as well as results of the phase 1 Aurora study of QR-1123 in retinitis pigmentosa, are expected this year. The remaining LCA10 pipeline is led by Editas with EDIT-101, but this has [already proven a bit of a disappointment](#).

Leber’s congenital amaurosis projects in the clinic

Project	Company	Mechanism of action	Details
<i>Phase 3</i>			
Sepofarsen	Proqr	CEP290 gene therapy	Failed pivotal Ph2/3 Illuminate trial Feb 2022
<i>Phase 2</i>			
EDIT-101	Editas	Crispr Cas9 CEP290 gene therapy	Interim data from Ph1/2 Brilliance trial disappointed Sep 2021
SAR439483	Atsena/Sanofi	Guanylate cyclase receptor inhibitor	Ph1/2 ongoing ; primary completion Feb 2022
HORA-RPE65	Coave	Retinoid isomerohydrolase gene therapy	Only active development is in retinitis pigmentosa

CEP290=centrosomal protein 290. Source: Evaluate Pharma

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