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Development of First-in-Class mutant transcript-selective ASO therapeutic candidates for the treatment of Avellino corneal dystrophy

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OPHTHALMOLOGY | **Candidate**

Product Type	Antisence oligonucleotide (ASO)
Indication	Avellino Corneal Dystrophy
Target	Mutant TGFBI protein
MoA(Mechanism of Action)	Selectively inhibiting the expression of the R124H mutant TGFBI with ASOs while minimizing the impact on wild-type TGFBI
Competitiveness	First-in-Class ASO therapeutics with characteristics of superior mutation selectivity and efficacy, and outstanding safety including minimized off-target effects - Competitor's siRNA lacks high mutant selectivity and CRISPR is limited to have 43.1% of the correction efficiency in corneal cells
Development Stage	Candidate
Route of Administration	Intravitreal injection or Eye drop

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