

# Discovery of Non-clinical Development Candidate Molecule for a Novel Universal Retinitis Pigmentosa Therapeutics

Olix Pharmaceuticals



OPHTHALMOLOGY	Candidate
Product Type	Small interfering RNA (siRNA)
Indication	Retinitis Pigmentosa
Target	Retinoid-related Orphan Nuclear Receptor Beta (ROR $\beta$ )
MoA(Mechanism of Action)	Protection of retinal degeneration by regulating gene expression of key transcriptional factors in photoreceptor cells
Competitiveness	<p>a. High unmet need: Wide application for various types of rod mutant RP patients (1.5 million globally)</p> <p>b. Cost: Cost-effective compared to Luxturna<sup>®</sup>, an approved treatment with limited application (used for patients with RPE65 gene mutations (&lt;1% of total RP patients))</p> <p>c. Technology: Leveraging proprietary cell-penetrating asymmetric siRNA platform technology</p> <ul style="list-style-type: none"> <li>• Less off-target effect, reduced immune response &amp; efficient delivery</li> <li>• Patient compliance due to less frequent injections</li> </ul>
Development Stage	Candidate
Route of Administration	Intravitreal injection ( $\geq$ Q3M)