

○ Therapeutics for Retinal Diseases

Title	Development of Cell and Gene Therapies for Posterior Retinal Diseases
Definition	<ul style="list-style-type: none"> <li>○ The project aims to develop cell and gene therapies targeting posterior retinal diseases to restore or improve vision and compensate for blindness through retinal cell replacement or genetic correction</li> <li>- The program integrates universal donor cell technology and disease-specific gene editing (including prime editing) to establish retinal cell-specific and gene-delivery therapeutic candidates</li> </ul>
R&D Plan	<ul style="list-style-type: none"> <li>○ (Step 1) Design and Preclinical Validation <ul style="list-style-type: none"> <li>- Design cell and gene therapies for posterior retinal diseases and validate therapeutic efficacy in clinically translatable models (e.g., patient-derived retinal organoids and relevant animal models)</li> <li>- Evaluate the efficacy and safety of retinal organoid and retinal progenitor/stem cell transplantation using universal donor cell platforms optimized to overcome retinal microenvironment adaptation</li> <li>- Design and functionally validate immune-tolerant gene therapy vectors (e.g., AAV, dual-vector systems) and optimize delivery and dosing strategies. Identify key translational biomarkers for functional vision improvement</li> <li>- Establish a consistent CMC framework and QC/QA strategy (incl. potency/identity assays), and leverage patient-derived retinal organoids to generate early toxicity, immunogenicity, and long-term safety data to inform the IND-enabling program</li> </ul> </li> <li>○ (Step 2) IND-enabling Package Completion and Clinical-Translational Readness <ul style="list-style-type: none"> <li>- Complete IND-enabling nonclinical studies(including GLP toxicology biodistribution/shedding and immunogenicity assessments as applicable) and finalize CMC documentation for regulatory filling to support IND submission and obtain IND approval</li> <li>- Establish clinical study designs focused on initial safety and tolerability</li> </ul> </li> </ul>
Need for Support	<ul style="list-style-type: none"> <li>○ (Policy) Cell and gene therapies for retinal diseases represent a core area of national advanced regenerative medicine policy. The project aims to contribute to regulatory and policy framework development through international collaborative research</li> </ul>

	<ul style="list-style-type: none"> <li>○ (Technical) Utilize global pharmaceutical partners' expertise in clinical design, CMC, quality assurance, and analytical infrastructure to enhance reliability and efficiency in IND submission and demonstration of therapeutic safety and efficacy.</li> <li>○ (Market) Leverage international co-development models for drug candidates targeting blinding retinal diseases to sustain investment, accelerate commercialization, and expand innovative drug business models.</li> <li>○ (Social) Address unmet medical needs in patients with intractable retinal diseases, strengthen national competitiveness through global research networks and patient registries, and ensure long-term verification of safety and clinical efficacy.</li> </ul>
Performance Target	<ul style="list-style-type: none"> <li>○ (Step 1) Identify candidate cell and gene therapies for target retinal diseases, demonstrate preclinical efficacy and preliminary safety, and establish IND-enabling CMC/QC frameworks and translational biomarkers to prepare for IND submission</li> <li>○ (Step 2) Complete IND-enabling packages (including GLP toxicology and CMC documentation) and obtain the IND approval</li> <li>○ (After completion) Execute global technology transfer or clinical development agreement</li> </ul>