

○ Therapeutics for Kidney Diseases

Title	Global Co-development of Targeted Therapeutics for Glomerular Diseases and Chronic Kidney Disease
Definition	<ul style="list-style-type: none"> ○ Development of innovative therapeutics that suppress inflammation and fibrosis by targeting the pathogenic mechanisms underlying major glomerular diseases—including IgA nephropathy (IgAN), focal segmental glomerulosclerosis (FSGS), and autosomal dominant polycystic kidney disease (ADPKD)—as well as chronic kidney disease (CKD) in the advanced fibrotic stage ○ The project focuses on key molecular targets such as the APOL1 signaling pathways, immune-complex clearance mechanisms, PKD1 functional restoration, kidney fibrosis reduction, glomerular and tubular epithelial cells protection and promotes global collaborative research and development centered on antibody-based and small-molecule therapeutics (excluding gene and cell therapies)
R&D Plan	<ul style="list-style-type: none"> ○ (Step 1) Mechanistic Elucidation and Target-based Candidate Discovery <ul style="list-style-type: none"> - Verify key pathogenic mechanisms involved in glomerular and renal injury, including APOL1 signaling, immune-complex clearance, and PKD1 functional restoration - Identify and evaluate antibody and small-molecule candidates using patient-derived cells, disease-relevant animal models to confirm therapeutic efficacy and establish translational biomarkers - Conduct ADME/PK studies, preliminary toxicity, and immunogenicity assessments to establish initial safety profiles and define the IND-enabling study plan ○ (Step 2) IND-enabling Package Completion and IND approval <ul style="list-style-type: none"> - Establish a GMP-compliant production system and CMC documentation for clinical-grade candidates and perform GLP toxicology studies to complete the IND-enabling nonclinical data package - Compile and submit the IND dossier, and obtain IND approval

Need for Support	<ul style="list-style-type: none"> ○ (Policy) Chronic kidney disease (CKD) and glomerular disorders are major chronic illnesses in aging societies. This project aligns with the Korean government's National Chronic Disease Management Policy and its initiative to strengthen global cooperation in advanced biopharmaceutical research. ○ (Technical) Therapeutics targeting novel pathways such as APOL1 and kidney fibrosis reduction involve high technical complexity and scientific risk, making it difficult for small and medium-sized enterprises to pursue independently. Collaboration with global pharmaceutical partners and governmental support are essential for GMP-grade manufacturing, clinical trial design, and successful regulatory progression. ○ (Market) The global therapeutic market for CKD and glomerular diseases exceeds KRW 50 trillion annually, leaving significant room for entry by first-in-class therapies with innovative mechanisms. Successful drug development could substantially reduce dialysis and transplantation demand, thereby alleviating national healthcare expenditures. ○ (Social) Reducing dialysis and transplantation rates will help lower healthcare costs while expanding the global networks of domestic biotech ventures and fostering human resource development in the biomedical sector.
Performance Target	<ul style="list-style-type: none"> ○ (Step 1) Secure novel target-based drug candidates, demonstrate preclinical proof-of-concept efficacy and complete preliminary ADME/PK and safety profiling to prepare for IND submission. ○ (Step 2) Complete IND-enabling studies(including GLP toxicology) and achieve CMC/GMP readiness. Submit the IND and obtain IND approval to initiate domestic and international Phase I clinical trials and verifying initial safety and tolerability. ○ (After completion) Execute global technology transfer agreements or establish clinical development partnerships.