

## ○ Neurology Therapeutics

Title	Co-development of Innovative Drug Candidates for Neurological Diseases Based on Glial Cell and Neuroinflammation Biology
Definition	<ul style="list-style-type: none"> <li>○ Development of First-in-class novel-target drug candidates for pain, epilepsy, neurodegenerative diseases, and psychiatric disorders that go beyond the limitations of existing treatments, such as insufficient efficacy and drug resistance, and aim at suppression of disease progression and disease-modifying therapy (DMT) / fundamental treatment approaches for neurological disorders.</li> <li>- Regulation of disease-specific glial cell function, suppression of neuroinflammation, and modulation of immune interactions in the peripheral/central nervous system (particularly BBB-related mechanisms)</li> <li>- Fundamental therapeutic approaches that alter disease progression or achieve cure through modulation of epigenetics, epitranscriptomics, and DNA repair mechanisms</li> <li>- Dual target modulation to address clinical therapeutic challenges</li> <li>○ Not limited to a specific modality, and includes BBB-permeable small molecules, antibodies, antibody mimetics, peptides, cell therapy, genome editing / RNA-based nucleic acids, and others.</li> <li>- Including target validation (including assay development) and drug development research; however, diagnostics, drug repurposing, and screening equipment development are excluded</li> </ul>
R&D Plan	<ul style="list-style-type: none"> <li>○ <b>(Step 1)</b> Candidate target validation and securing preclinical candidates</li> <li>- Verification of the mechanistic validity of candidate targets through human-derived systems such as human induced pluripotent stem cells (hiPSCs), 3D brain organoids, and human brain slice/sample-based analyses</li> <li>- Establishment of core assays targeting disease progression axes, including modulation of neuro-immune/glial cell functions and epigenetic/DNA repair mechanisms</li> <li>- Final selection of 1–2 preclinical candidates after confirming efficacy PoC, preliminary ADME/PK, BBB distribution, and early</li> </ul>

	<p>safety findings (including CNS adverse effects)</p> <ul style="list-style-type: none"> <li>○ <b>(Step 2)</b> Completion of the IND data package and IND approval <ul style="list-style-type: none"> <li>- Completion of the IND-enabling package including GLP toxicology, safety pharmacology (including CNS), ADME/PK (considering CNS distribution and permeability), and, if needed, long-term toxicology and behavioral/cognitive evaluations</li> <li>- Establishment of CMC (process / analytical methods / specifications / stability), GMP manufacturing, and QC systems</li> <li>- Preparation, submission, and approval of the IND dossier</li> </ul> </li> </ul>
Need for Support	<ul style="list-style-type: none"> <li>○ (Policy) Neurological disorders are directly linked to national healthcare and caregiving costs (e.g., the estimated national dementia management cost is KRW 27 trillion in 2025, KRW 39 trillion in 2030, and KRW 75 trillion in 2040), and resolving high-burden neurological disorders through therapeutic innovation is aligned with public policy priorities.</li> <li>○ (Technical) The treatment paradigm for neurological disorders is shifting toward improving the fundamental neural environment itself, including glial cells and neuroinflammation, and this requires high-difficulty bundled technologies across candidate–disease model–biomarker development and long-term safety verification. Therefore, it is difficult for SMEs/ventures to advance independently, and collaboration with global pharmaceutical companies is essential.</li> <li>○ (Market) Due to population aging, the global Alzheimer’s disease therapeutics market is projected to grow at 61.2% annually (from USD 650 million in 2023 to USD 18.22 billion in 2030, equivalent to approximately KRW 27 trillion). Therefore, discovery of neurological disease therapeutic candidates and entry into the IND stage can contribute to expanded technology transfer and global commercialization opportunities for domestic companies.</li> <li>○ (Social) Neurological disorders cause deterioration in patients’ quality of life, family caregiving burden, and socioeconomic losses (medical/care costs and productivity loss) over a long disease course. Expansion of treatment options for neurological disorders can improve quality of life and productivity, while also strengthening the drug development capabilities and global networks of domestic biotech ventures.</li> </ul>
Performance Target	<ul style="list-style-type: none"> <li>○ <b>(Step 1)</b> Validate targets using human-derived CNS models, secure 1–2 candidate compounds, obtain efficacy PoC (including glial cell / neuroinflammation indicators), BBB distribution, preliminary ADME/PK,</li> </ul>

	<p>and early safety data, and finalize the IND-enabling study roadmap</p> <ul style="list-style-type: none"><li>○ <b>(Step 2)</b> Secure the IND-enabling package, including GLP toxicology, as well as CMC/GMP readiness, and complete IND submission and approval</li><li>○ <b>(After completion)</b> Conclude at least one global co-development or technology transfer agreement</li></ul>
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