

○ AAV-based Gene Therapy Platform Technology

Title	Development of AAV-Based Gene Therapy Platform for Neuromuscular and Central Nervous System Disorders
Definition	<ul style="list-style-type: none"> <li>○ A next-generation gene therapy platform that utilizes adeno associated virus (AAV) vectors to correct pathogenic genes or deliver normal genes for the treatment of neuromuscular and central nervous system disorders</li> <li>- A precision therapeutic approach targeting rare inherited diseases such as muscular atrophy disorders and metabolic myopathies, incorporating AAV viral vectors or CRISPR-based gene-editing technologies to restore normal gene expression in muscle cells and recover the function of defective genes</li> <li>- The platform integrates capsid engineering, tissue-specific expression control, immune-response mitigation, and high-efficiency vector manufacturing with QbD-based quality management to achieve accurate and durable in vivo therapeutic outcomes</li> </ul>
R&D Plan	<ul style="list-style-type: none"> <li>○ (Step 1) Design and preclinical validation of therapeutic AAV vectors for target rare diseases</li> <li>- Construct AAV vectors incorporating muscle-tissue-specific promoters and evaluate therapeutic efficacy in patient-derived cells and disease animal models</li> <li>- Design AAV vectors by selectively integrating key technologies such as CRISPR-based gene editing and capsid engineering to verify gene correction and expression recovery efficiency</li> <li>- Conduct preliminary toxicity and immune-response assessments and the IND-enabling GLP study plan (biodistribution, immunogenicity, etc.)</li> <li>○ (Step 2) GMP-grade AAV Manufacturing, IND-enabling Package, and IND Approval</li> <li>- Establish a GMP-compliant manufacturing process for clinical-grade AAV gene therapy, integrating QbD-based QC and optimization of vector yield and purification efficiency</li> <li>- Advance comprehensive vector characterization including particle stability, infectivity, and capsid integrity and standardize CMC data packages for regulatory submission</li> <li>- Execute IND-enabling non-clinical and CMC documentation, submit the IND and secure approval to establish a framework for entering Phase I clinical entry</li> </ul>

Need for Support	<ul style="list-style-type: none"> <li>○ (Policy) AAV-based gene therapy is a key focus of the government's advanced regenerative-bioindustry and K-Bio global expansion strategies; international joint research can enhance access to treatments for rare and intractable diseases.</li> <li>○ (Technical) The development integrates high-complexity technologies such as AAV vector manufacturing, capsid engineering, and CRISPR gene editing; collaboration with global pharmaceutical companies and support for GMP and QbD-based production systems are essential.</li> <li>○ (Market) With few approved therapies for rare neuromuscular diseases, successful commercialization could allow domestic biopharma SMEs to secure an early entry into the global gene-therapy market.</li> <li>○ (Social) Improves treatment accessibility for patients with rare genetic disorders and strengthens the national infrastructure and skilled workforce for gene-therapy R&amp;D and biomanufacturing.</li> </ul>
Performance Target	<ul style="list-style-type: none"> <li>○ (Step 1) Complete design and construction of AAV-based gene-therapy vectors (<math>\geq 1</math>); verify therapeutic efficacy in patient-derived cells and animal models (<math>\geq 1</math>); finalize preclinical toxicity and immune-response evaluations to assemble the IND submission data package</li> <li>○ (Step 2) Establish GMP-grade manufacturing processes for clinical AAV vectors (QbD-based); develop CMC and QC systems and complete vector characterization (including particle stability and infectivity); submit the IND and secure approval (<math>\geq 1</math>) through supplementary non-clinical and CMC documentation</li> <li>○ (After completion) Execution of global technology transfer or initiate clinical trial and first patient dosing; pursue joint clinical development or technology-transfer agreements with global pharmaceutical partners (<math>\geq 1</math>)</li> </ul>