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GCT
National Strategy
Gene- and Cell-Based Therapies
Project Funding

FusigenX - A novel, non-viral vector for Gene Editor delivery into muscle and heart tissue in Duchenne muscular dystrophy

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The aim of the project FusigenX is to develop a novel, non-viral gene delivery system to treat Duchenne muscular dystrophy (DMD), one of the most severe inherited muscle diseases. Current gene therapy approaches rely on viral vectors, which pose significant risks including toxicity, immune reactions, and limited re-application. FusigenX introduces engineered proteinbased carriers that efficiently and specifically deliver therapeutic RNA to heart and muscle cells. This new technology promises higher transfer efficiency than lipid nanoparticles while avoiding the safety concerns associated with adeno-associated viruses (AAVs).

The project will conduct proof-of-concept studies in pig models, using CRISPR/Cas9-based genome editors such as the adenine base editor to correct DMD mutations and restore dystrophin expression without detectable side effects. Over the next two years, the consortium will refine muscle- and heart-targeted RNA carriers, validate efficacy in advanced preclinical models, and prepare for clinical translation. With its unique combination of efficiency, safety, and scalability, FusigenX aims to deliver the first nonviral gene-editing therapy for DMD, paving the way for future applications in genetic medicine.