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Identification of Lead Candidates for Tissue-Specific Gene Editing Therapeutics for DM1

NES Biotechnology

OTHERS | **Lead**

Product Type	Gene & Nucleic acids
Indication	Myotonic dystrophy type1, DM1
Target	Mutant DMPK transcripts harboring expanded CUG repeats in the 3' untranslated region.
MoA(Mechanism of Action)	CRISPR-Cas9 system is delivered via a gold nanoparticle (AuNP)-aptamer conjugate that targets transferrin receptor (TfR)-expressing cells. The modality aims to: 1) Edit, degrade, or suppress toxic DMPK RNA in muscle and neuronal tissues, 2) Restore MBNL function by releasing sequestered RNA-binding proteins, and 3) Reduce downstream RNA foci and rescue mis-splicing events.
Competitiveness	• First-in-class or best-in-class DM1 gene-editing candidate using a non-viral, muscle-targeted delivery platform • Overcomes limitations of AAV-based gene therapy (payload size, immunogenicity, repeat dosing) • Demonstrated selective editing, low immunogenicity, and scalable manufacturability • Potential to be superior to systemic ASOs (e.g., IONIS-DMPKRx) in tissue penetration and durability • Leverages TfR aptamer-guided delivery, enhancing muscle uptake while minimizing off-target exposure
Development Stage	Lead
Route of Administration	Intravenous (IV) injection