RS-2024-00337778

Study on the Discovery of Lead Compounds for Lesch-Nyhan Syndrome Treatment Using Cell-Derived Vesicles Carrying HPRT1 mRNA and Proteins

MDimune Inc.

OTHERS | Lead

Product Type	Cell-derived vesicles carrying HPRT1 mRNA and proteins
Indication	Lesch-Nyhan syndrome
Target	Deficiency of hypoxanthine-guanine phosphoribosyltransferase activity
MoA(Mechanism of Action)	Delivery of HPRT1 mRNA and proteins leads to the restoration of HGPRT enzyme activity, which in turn restores the purine metabolism and cellular function.
Competitiveness	• Co-Delivery of mRNA & Protein - The therapeutic agent contains both mRNA and protein forms of the active ingredient. The two forms of the active ingredient are co-encapsulated into vesicles through overexpression in cells, enabling simultaneous delivery. The mRNA derived from cellular transcription eliminates the need for separate in vitro transcritpion (IVT) and purification steps, and reduces immunogenicity. • High Efficiency of mRNA Encapsulation - The CDV engineering platform allows for the incorporation of specific RNA-binding proteins, significantly improving the efficiency of mRNA encapsulation within the vesicles. • Superior Safety Profile - Biocompatible CDVs make them ideal for repeated systematic administration, offering a significant advantage for therapeutic applications. • High Productivity utilizing Innovative Technology - CDVs producing through extrusion is a unique approach that can overcome the production limitations of naturally secreted extracellular vesicles. The technological innovation was internationally recognized, earning a Bronze Medal at the 2023 Edison Awards.
Development Stage	Lead
Route of Administration	i.v.

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