RS-2026-25503251

AAV Gene Therapy for Pendrin Mutation–Associated Sensorineural Hearing Loss

ICM CO.,LTD.

OTHERS | **Candidate**

Product Type	rProtein
Indication	DFNB4/PDS (Sensorineural Hearing Loss)
Target	SLC26A4 (Pendrin)
MoA(Mechanism of Action)	• AAV-mediated wildtype pendrin gene delivery to recuperate inner ear defects caused by Pendrin gene mutations ✓ Re-establishing endolymph balance ✓ Restoring inner ear structural integrity ✓ Preventing progressive sensory hair cell degeneration ✓ Preserving auditory function
Competitiveness	• DFNB4 mutations - the 3rd most common genetic cause of hearing loss • Variant-agnostic strategy by AAV-mediated wildtype pendrin gene replacement • Proprietary AAV platform specifically targeting inner ear • Proprietary preclinical disease models recapitulating DFNB4 phenotypes, enabling therapeutic validation of the drug candidates • First-in-class development potential for Pendrin-associated sensorineural hearing loss
Development Stage	Candidate
Route of Administration	Round Window Membrane (RWM) Intracochlear Injection