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AI in Medicine
Future of Healthcare by AI

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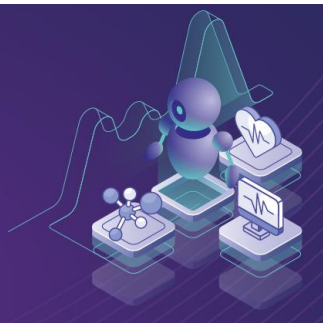
Speech Title	Gene Therapy for Hereditary Hearing Loss: From Molecular Insights to First-in-Human Trials	



Abstract(200 words)：

Hereditary hearing loss—the most common congenital sensory disorder, affecting 1–2 per 1,000 newborns—presents a unique opportunity for precision therapeutics. The cochlea is surgically accessible, fluid-sealed, and over half of cases arise from single-gene mutations. Recent breakthroughs in inner-ear biology, combined with advances in gene delivery and editing, are rapidly reshaping the therapeutic landscape. Engineered adeno-associated virus (AAV) capsids, such as Anc80L65 and next-generation AAV9 variants, now enable >80% inner-hair-cell transduction in rodents and non-human primates, while hybrid nanoparticle systems accommodate oversized or dual-vector payloads. Beyond classical gene replacement, CRISPR-based gene editing, together with precision base and prime editors, is expanding the therapeutic repertoire for mutation-specific correction.

These innovations are moving toward clinical application. Phase I/II trials targeting OTOF-related auditory synaptopathy are underway, with preliminary reports showing encouraging safety and no dose-limiting ototoxicity. Our group has contributed key preclinical milestones, including proof-of-concept studies for some of the most prevalent forms of genetic deafness. Looking ahead, universal newborn genomic screening, rational vector design, and scalable GMP manufacturing will be critical for clinical translation. By integrating molecular genetics, vectorology, and clinical otology, gene therapy is poised to redefine the