



The Audiology Pipeline: An overlooked yet advancing corner of biopharma

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For decades, hearing loss management has required hearing aids to amplify sound and cochlear implants to bypass damaged internal structures. Disease-modifying treatments have been elusive.

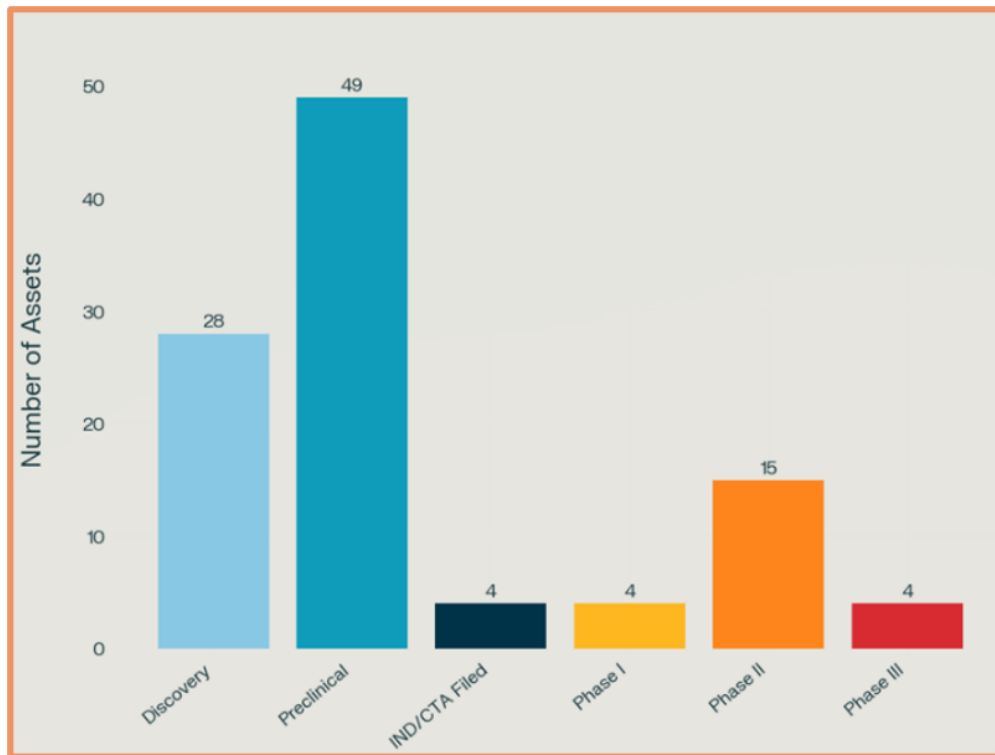
Audiology care is now entering a transformational phase, with the FDA recently approving Regeneron's Otarmeni (lunsotogene parvec), the first gene therapy to restore a neurosensory function in humans. Otarmeni is intended to treat a rare cause of congenital deafness in a pediatric population with otoferlin (OTOF) gene mutations. While this is a small patient population, the clinical proof of concept and regulatory success provides exemplary evidence that biological, disease-modifying approaches to hearing restoration are no longer theoretical.

A Lower-Profile Field, Now Gaining Momentum

The audiology field has made incremental progress for years, but has generally lacked the visibility, capital and dealmaking activity that fields such as ophthalmology and neuroscience generate. According to *Global Data*, the audiology industry pipeline is comprised of 104 hearing loss and hearing disorder candidates, spanning discovery through Phase 3. Three-quarters (~74%) of these research candidates

are in Discovery or Preclinical stage while ~26% are in clinical stage, from IND filing through Phase III.

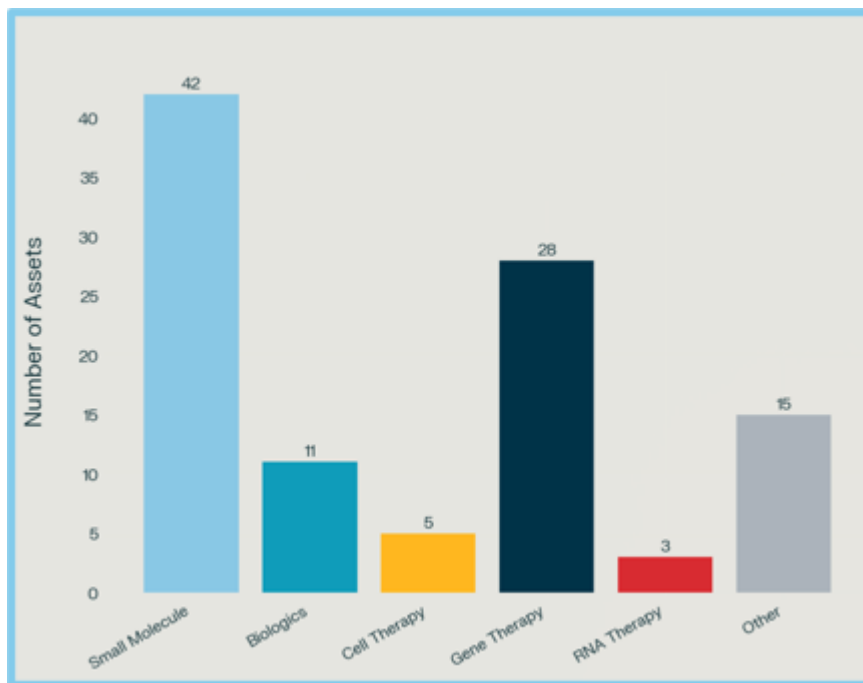
Audiology Pipeline By Development Stage



Two Prominent Modalities: Small Molecules and Gene Therapy

Small molecule development represents ~40% of active industry-wide programs (excluding discontinued/inactive programs). A testament to decades of accumulated knowledge in otoprotection, anti-inflammatory approaches and ion-channel biology. Gene therapy represents ~27% of the pipeline and is almost entirely built on AAV-vector delivery. Cell therapy and RNA-based approaches each comprise a much smaller modality cohort, together making up roughly 8% of programs.

Audiology Pipeline By Modality



Biologics, including monoclonal antibodies, comprise about a tenth of the pipeline. This is largely a function of the cochlea's unique physiology as a small, fluid-filled, immune-sensitive structure that is difficult to access and does not lend itself easily to antibody-based approaches. [Neuracle's NS101](#) is a notable standout - this monoclonal antibody has reached phase 2 for Sudden Sensorineural Hearing Loss.

The table below lists **Phase 2** development programs ongoing or completed.

Phase 2 therapeutic candidates by company, indication, target and modality

Drug	Company	Indication (Phase II)	Target	Modality
AC-102	AudioCure Pharma GmbH	Acute SNHL	Neurotrophic Factor	Small Molecule
AK-OTOF	Akouos Inc	Acute SNHL	Otoferlin	Gene Therapy
DB-041	Regeneron Pharmaceuticals Inc	Hearing Disorders	Undisclosed	Small Molecule
Dexamethasone-Loaded Exosomes	Kafr El Sheikh University	Acute SNHL	Glucocorticoid Receptor	Small Molecule
GJB2-Related Hearing Loss	Skylark Bio Inc	Hearing Disorders	GJB2 (Connexin 26)	Gene Therapy
MA-5	Tohoku University	Hearing Disorders	Mitofilin (MIC60) / Mitochondrial ATP Synthase	Small Molecule
NS-101	Neuracle Science Co Ltd	Acute SNHL	TAFA-5 / FAM19A5	Monoclonal Antibody
anakinra	Swedish Orphan Biovitrum AB	Autoimmune Inner Ear Disease (AIED)	IL-1 Receptor Type 1	Recombinant Protein
azasetron	Sensorion SA	Acute SNHL; Hearing Disorders	5-HT3 & Histamine H4 Receptors	Small Molecule
bimokalmir (ACOU-085)	Acousia Therapeutics GmbH	Acute SNHL (cisplatin-induced)	KCNQ4 (Kv7.4)	Small Molecule
ebselen	Sound Pharmaceuticals Inc	Hearing Disorders	Glutathione Peroxidase	Small Molecule
sirolimus	Nobelpharma Co Ltd	Acute SNHL	mTOR	Small Molecule
sodium thiosulfate	Fennec Pharmaceuticals Inc	Hearing Loss - Adults (cisplatin-induced)	Antioxidant	Small Molecule
telE	Bodor Laboratories Inc	Unspecified Ear Nose Throat Disorders	Undisclosed / Retrometabolic	Small Molecule

Mechanisms in Focus: A Diverse Target Landscape

There are five distinct programs targeting **Chemokine-Like Protein TAFA-5** (also called FAM19A5), all being advanced by South Korea's Neuracle Science. Neuracle is pursuing the same validated target (synapse-loss inhibition via FAM19A5) through three different modality approaches in parallel: monoclonal antibodies, a small molecule and a peptide program.

GJB2 (which encodes Connexin 26) is the central target focus of four independent gene therapy developers, including Sensorion, Skylark Bio, Akouos, and Gap Junction Inc. These companies aim to restore the potassium-recycling function GJB2 controls. The heightened interest in this target reflects the scale of the opportunity: GJB2

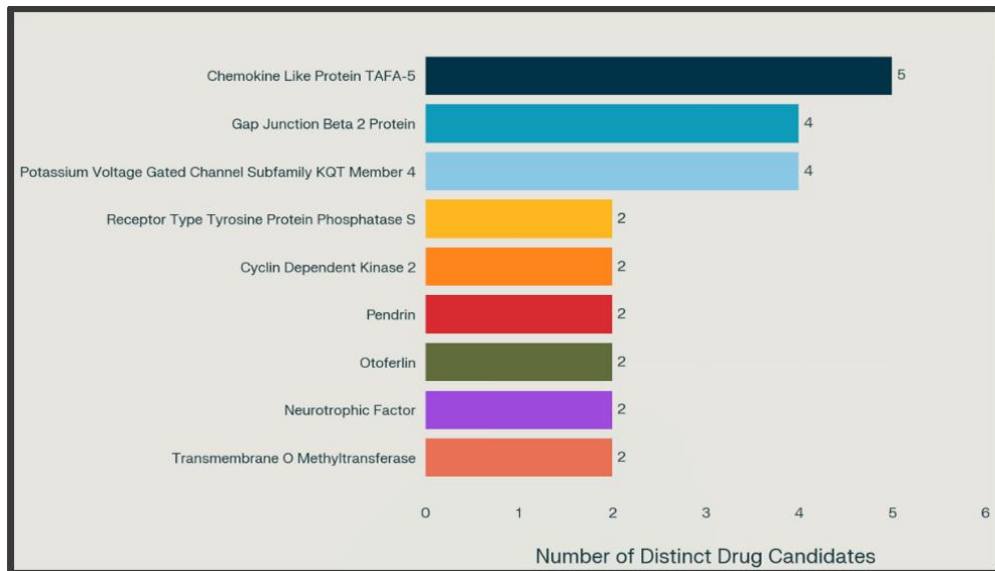
mutations account for ~ 50% of genetic congenital deafness cases, making it the largest addressable target in genetic hearing loss.

Elsewhere in the audiology landscape, four small molecule developers have homed on **KCNQ4** (the Kv7.4 potassium channel). [Acousia Therapeutics' bimokalner](#) (ACOU085), has a substantial clinical lead amongst programs focused on this target, having completed a successful Phase 2 PROHEAR study evaluating prevention of cisplatin-induced ototoxicity. Bimokalner has further development potential in Sudden Sensorineural Hearing Loss (SSNHL) and other hearing preservation indications.

Otoferlin (OTOF) is the target behind Regeneron's approved Otarmeni and Eli Lilly's AK-OTOF.

Pendrin, Cyclin-Dependent Kinase 2, Neurotrophic Factor, Transmembrane O Methyltransferase and **Receptor Type Tyrosine Protein Phosphatase S** all have two distinct drug candidates each.

Top Targets in the Audiology Industry Pipeline



The majority of gene therapy programs in active development are aimed at a congenital or monogenic cause of hearing loss. The core value proposition of gene therapy – to correct a specific genetic mutation – promises transformational improvement in addressing mutations occurring in OTOF, GJB2, Pendrin, and Stereocilin, for example. A unique exception, however, is Sensorion’s GJB2 program, involving SENS-601, which has been extended beyond congenital deafness into adult-onset presbycusis (age-related hearing loss). The validation for this bold pipeline extension comes from fairly recent science showing that Connexin 26 (the protein GJB2 encodes) plays a role in age-related hearing loss, as well as causing deafness from birth when entirely absent. Age-related decline in Cx26 expression appears to independently accelerate hearing loss later in life. [Sensorion is conducting a natural history study in adults aged 30 to 55 to characterize this early onset presbycusis population.](#) If this thesis holds up in the clinic, it would meaningfully expand gene therapy’s addressable market well beyond the rare-disease congenital population to capture a larger slice of everyday age-related hearing loss.

Why the Pipeline Looks Fragmented: Multiple Causes of Hearing Loss

Part of what explains the pipeline's mechanistic spread is that hearing loss is a heterogeneous condition. The scientific literature identifies several distinct and largely non-overlapping causes, each of which points toward a different therapeutic strategy.

Age-related hearing loss (presbycusis) is the largest single contributor by volume, affecting roughly half the population in their 70s, with ~719 million prevalent cases globally among those 60 and older, as of 2021. Noise exposure, occupational and recreational causes account for an estimated 16% of adult disabling hearing loss worldwide.

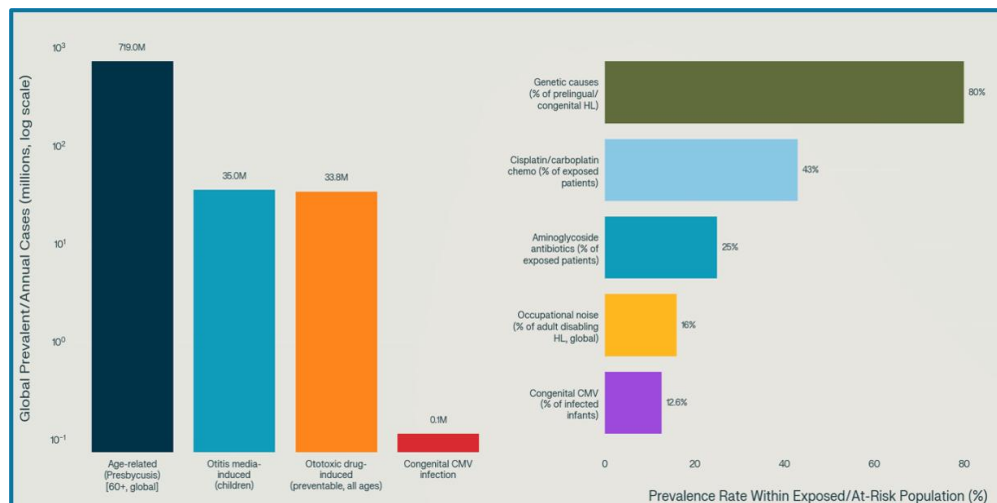
Otitis media (ear infections), is the leading cause in young children, responsible for nearly 69% of hearing loss in children under five. Ototoxic medications, including platinum-based chemotherapies and aminoglycoside antibiotics, cause an estimated 33.8 million new cases of hearing loss annually, with [cisplatin and carboplatin linked to hearing loss in a pooled 43% of treated patients](#).

At the **genetic** end of the spectrum, ~ 80% of prelingual, congenital hearing loss is attributable to inherited mutations. This fact underscores why GJB2 and OTOF programs occupy such an outsized share of gene therapy development in audiology.

The global burden of hearing loss (mild, moderate, severe) is anticipated to [grow from 1.5 billion to 2.5 billion by 2050](#), a substantial market opportunity by any measure. Overall, the causal diversity in hearing loss drives the expansive range in modality and targets being pursued across the industry.

Causes and Prevalence of Hearing Loss

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Dealmaking: Smaller Scale, Building Momentum

A modest but telling set of transactions suggests prescient pharmaceutical and medtech companies are seizing the opportunity to gain industry leadership in the relatively overlooked audiology space. The sector's overall deal volume has been historically thin next to fields like ophthalmology, oncology, or other CNS indications, where licensing and M&A activity has been more frequent and generally larger.

On the M&A side,

- Eli Lilly's acquisition of Akouos in October 2022 was valued at ~ \$487 million upfront, plus a contingent value right of up to \$3.00 per share.
- Less than a year later, Regeneron acquired Decibel Therapeutics for \$109 million upfront, with total potential value of up to \$213 million including milestones.

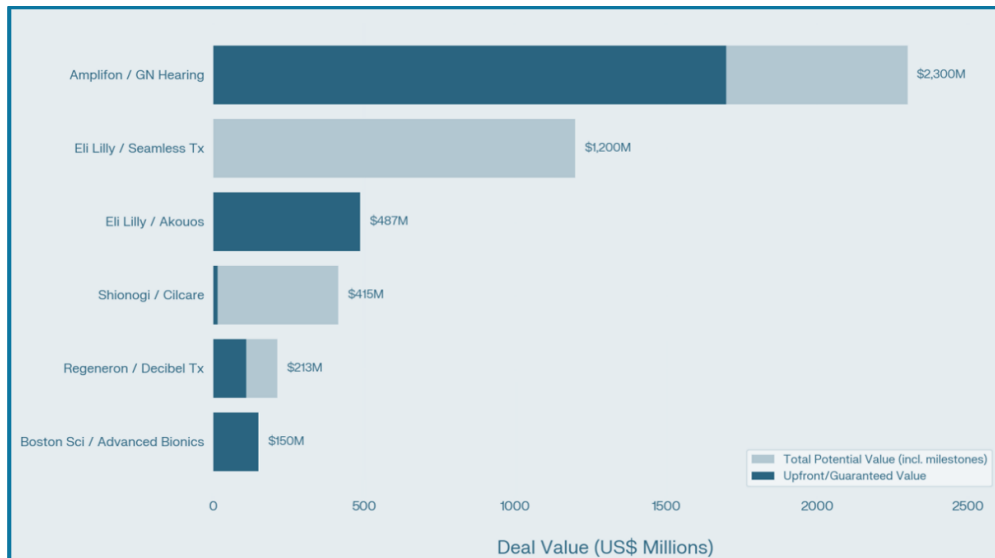
These two transactions were arguably the turning point in audiology dealmaking - evidence that big pharma recognized tangible commercial upside in genetic hearing loss therapies, no longer relegating them as niche and too small with which to bother.

On the licensing and collaboration side,

- Shionogi's option agreement with Cilcare for small molecule hearing loss candidates could be worth up to \$415 million including an upfront payment, milestones, and royalties.
- Eli Lilly's January 2026 collaboration with Seamless Therapeutics focuses on programmable recombinase-based treatments and carries a potential value of up to \$1.2 billion for Seamless.
- Separately, Eli Lilly also struck a \$1.3 billion collaboration with Rznomics in 2025 for RNA-editing therapies targeting rare hearing loss diseases.

Clearly big pharma's interest now extends beyond AAV gene therapy into a broader range of genetic medicine platforms as well as small molecule delivery.

Transactions in the Hearing Loss Landscape



Financing activity in the audiology space mirrors the building momentum witnessed on the M&A/licensing side.

In January 2026, Sensorion closed a \$60 million round that included a \$20 million strategic investment from Sanofi, marking the first direct stake by a major pharmaceutical company in Sensorion. More recently, Sensorion has filed for a U.S. IPO.

Meanwhile, consolidation is picking up in the more established, commercial corners of device manufacturing and hearing clinic services. Amplifon's ~\$2.3 billion agreement to acquire GN Store Nord's hearing business, while outside the prescription therapeutics pipeline itself, points to an ecosystem attracting capital and dealmaking on multiple fronts.

The Bottom Line

Audiology is at **an inflection point**. A first regulatory approval has proven that biological, disease-modifying treatments for hearing loss are achievable. A well-characterized set of distinct underlying causes provides developers with multiple credible paths forward, whether through gene therapy, small molecules, RNA editing or other modalities. Validated, high-value targets have attracted a broader range of competitive R&D developers, expanding the audiology landscape meaningfully.

There are also preliminary signs the addressable market for some of these approaches could expand well beyond initial projections. Emerging science suggests that Connexin 26, the protein generated by the leading genetic target GJB2, may play a role in age-related hearing loss as well as congenital deafness. Similarly, on the small molecule side, several of the otoprotective compounds now in development for ototoxicity, including KCNQ4 activators and antioxidant-based agents, have shown preclinical activity against more than one type of cochlear damage. These earlier data sets hint that some of today's narrowly targeted preventive therapies may find **use in broader hearing loss populations** - an encouraging thought for all of us in the aging population!