

Focus Area: Targeted Therapeutics for Auditory Regeneration

Reducing social isolation by improving and restoring lost hearing

Our Goal

Our goal for Focus Area Targeted Therapeutics for Auditory Regeneration is to identify, develop and deliver treatments for patients with **sensorineural hearing loss** through **regenerative medicine** and **targeted drug delivery**. We are aiming high, seeking **restoration of auditory function**, a goal yet to be achieved in medicine.

Background



Approximately one in three adults aged 65 to 74 suffer from hearing loss rising to half of adults over 75.¹ Current treatment options are limited, meaning **hearing loss significantly impacts quality of life** including social isolation and reduced communication with loved ones. We are developing **potentially revolutionary medicines** to improve or even restore hearing for patients with sensorineural hearing loss.

Strategic Approach

We are investing in drug candidates combining targeted delivery technology and the potential for auditory function regeneration:



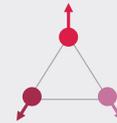
FOCUS

Prioritizing novel therapeutic options for sensorineural hearing loss caused by cochlear damage.



ENRICH

Leveraging our R&D platform to build a unique and differentiating pipeline, targeting key inner ear cells central to disease pathophysiology.



EXPAND

Exploring next-generation technologies and modalities in-house and with external partners including antisense oligonucleotides and gene therapies.

Our differentiated platform technologies include:



Otology research and development capability and unique pipeline



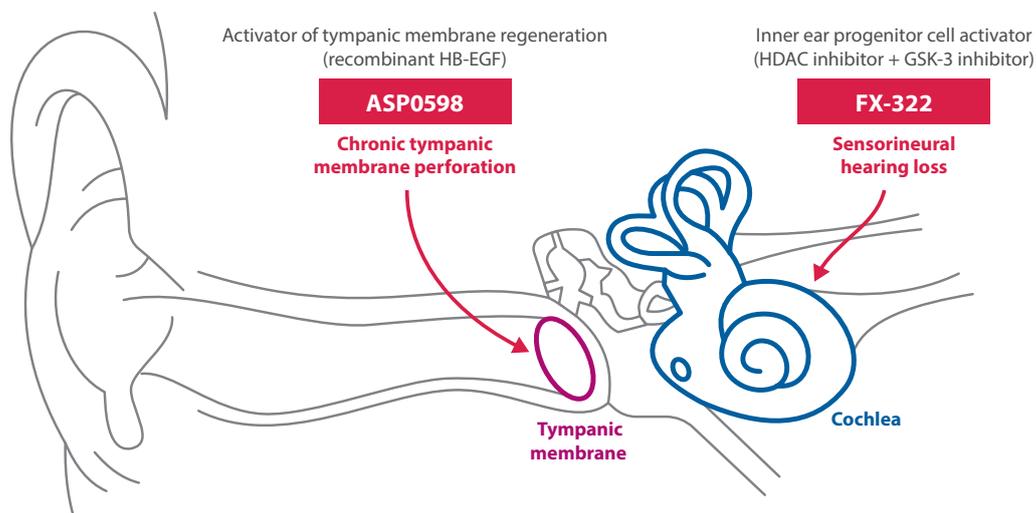
Safe, efficient and user-friendly delivery of treatment based on multiple modalities



Platform and capability to yield innovative drug candidates with regenerative potential

Pipeline

We are exploring innovative modalities to restore hearing.



GSK-3: Glycogen synthase kinase-3, HDAC: Histone deacetylase, HB-EGF: Heparin-binding epidermal growth factor-like growth factor

Spotlight: Delivering regenerative medicine directly into the ear

Astellas is rapidly becoming a leading center of regenerative medicine worldwide. In hearing loss, the potential for regenerative medicine is immense, offering hope for life-changing improvement through targeted, restorative treatment for patients who have no current treatment options.

Key to the development of transformative therapies for hearing loss is an efficient and safe drug delivery technology for transporting regenerative cells into the cochlea and middle ear.

A series of programs for otologic indications are planned, aiming for the recovery of auditory function. Our lead program, FX-322, an inner ear progenitor cell activator for sensorineural hearing loss, is currently in phase 2 clinical trials.

Current Status†

We are building a competitive pipeline in collaboration with expert partners and academia:

| Compound | Modality | Mechanism | Origin/partner | Indication | Current stage | | |
|---------------|---------------------|--|----------------|---------------------------------------|-----------------------|------------------|------------------|
| | | | | | Pre-clinical/research | Clinical phase 1 | Clinical phase 2 |
| FX-322 | Small molecule | Inner ear progenitor cell activator (GSK-3 inhibitor + HDAC inhibitor) | * | Sensorineural hearing loss | | | |
| ASP0598 | Recombinant protein | Recombinant HB-EGF for regeneration of tympanic membrane | * | Chronic tympanic membrane perforation | | | |
| Not disclosed | Not disclosed | Hair cell regeneration | Not disclosed* | Sensorineural hearing loss | | | |
| Not disclosed | Not disclosed | Multiple | In-house | Sensorineural hearing loss | | | |

GSK-3: Glycogen synthase kinase-3, HDAC: Histone deacetylase, HB-EGF: Heparin-binding epidermal growth factor-like growth factor.

† Accurate as of June 2020, * Programs developed under joint research.

REFERENCES

1. Data from the National Institute on Deafness and Other Communication Disorders (NIDCD) Fact Sheet on Age-Related Hearing Loss, March 2016.



In this material, statements made with respect to current plans, estimates, strategies and beliefs and other statements that are not historical facts are forward-looking statements about the future performance of Astellas Pharma. These statements are based on management's current assumptions and beliefs in light of the information currently available to it and involve known and unknown risks and uncertainties. A number of factors could cause actual results to differ materially from those discussed in the forward-looking statements. Such factors include, but are not limited to: (i) changes in general economic conditions and in laws and regulations, relating to pharmaceutical markets, (ii) currency exchange rate fluctuations, (iii) delays in new product launches, (iv) the inability of Astellas to market existing and new products effectively, (v) the inability of Astellas to continue to effectively research and develop products accepted by customers in highly competitive markets, and (vi) infringements of Astellas' intellectual property rights by third parties. Information about pharmaceutical products (including products currently in development) which is included in this material is not intended to constitute an advertisement or medical advice.