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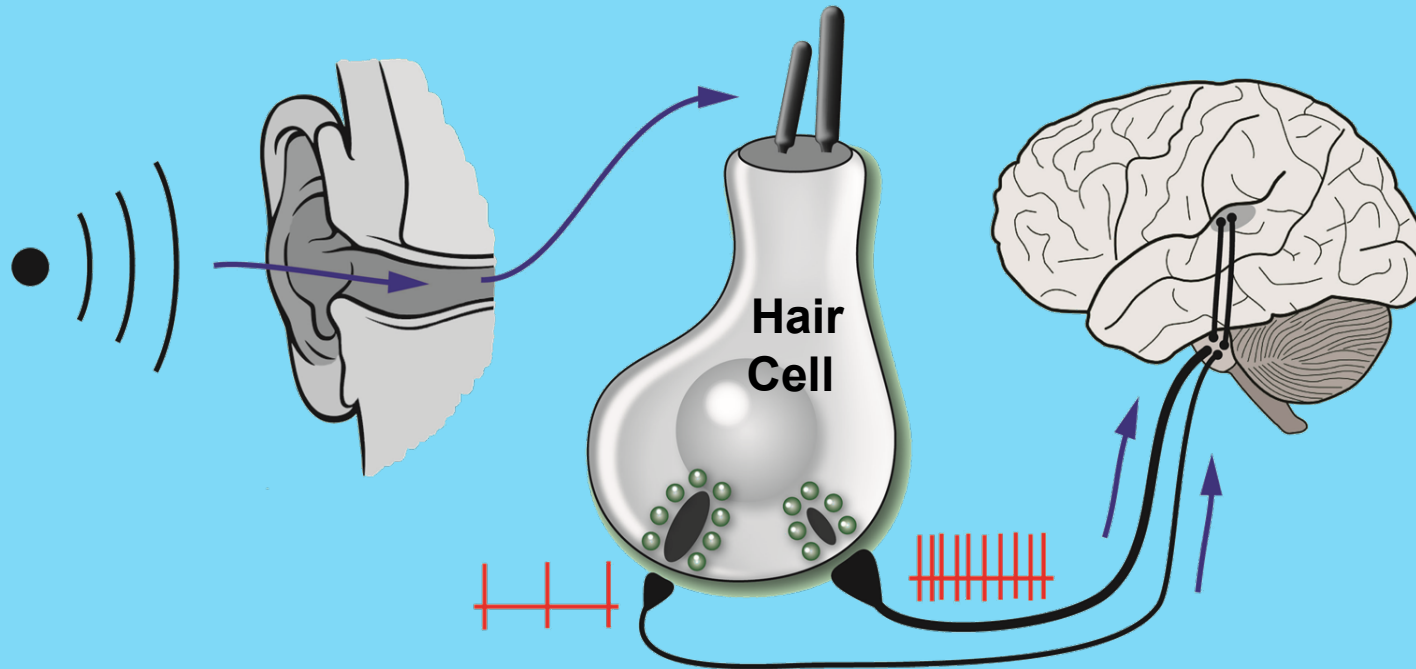
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Gene-based therapy for Deafness and Age-related Hearing Loss

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Research Interests - WM Lab.

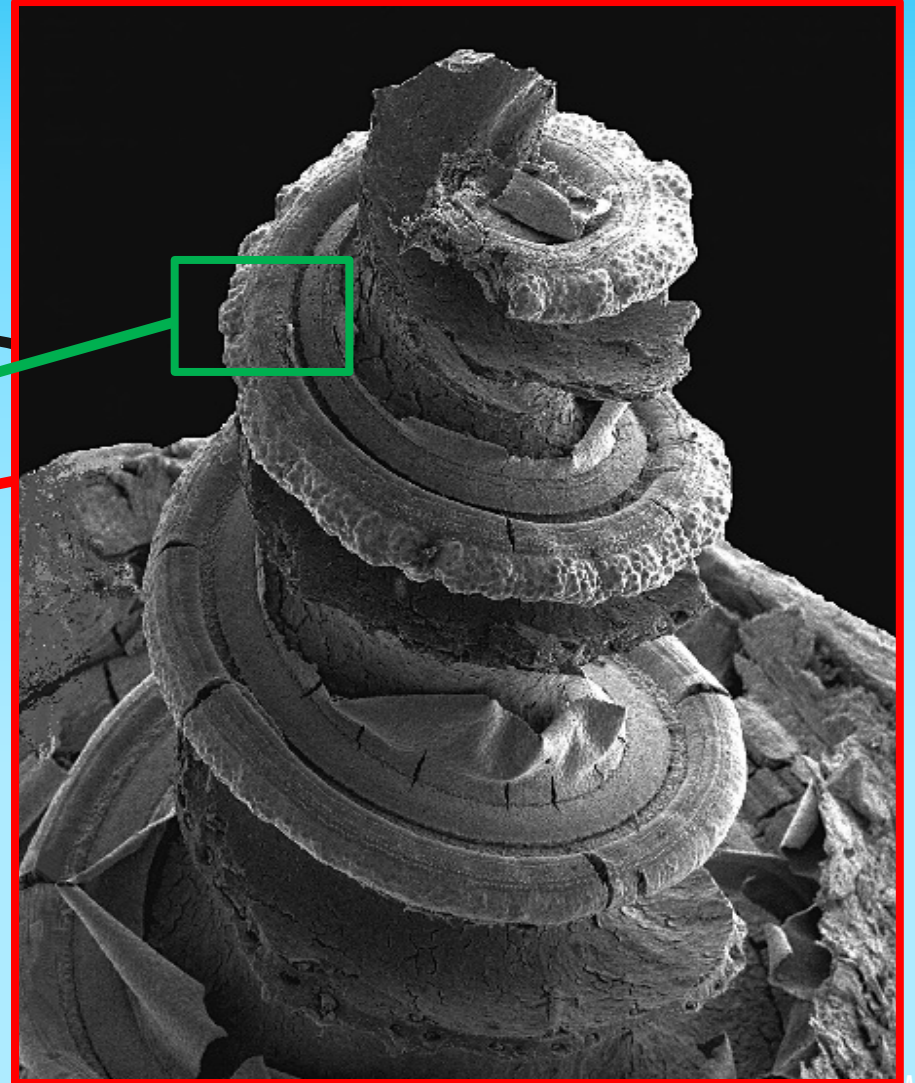
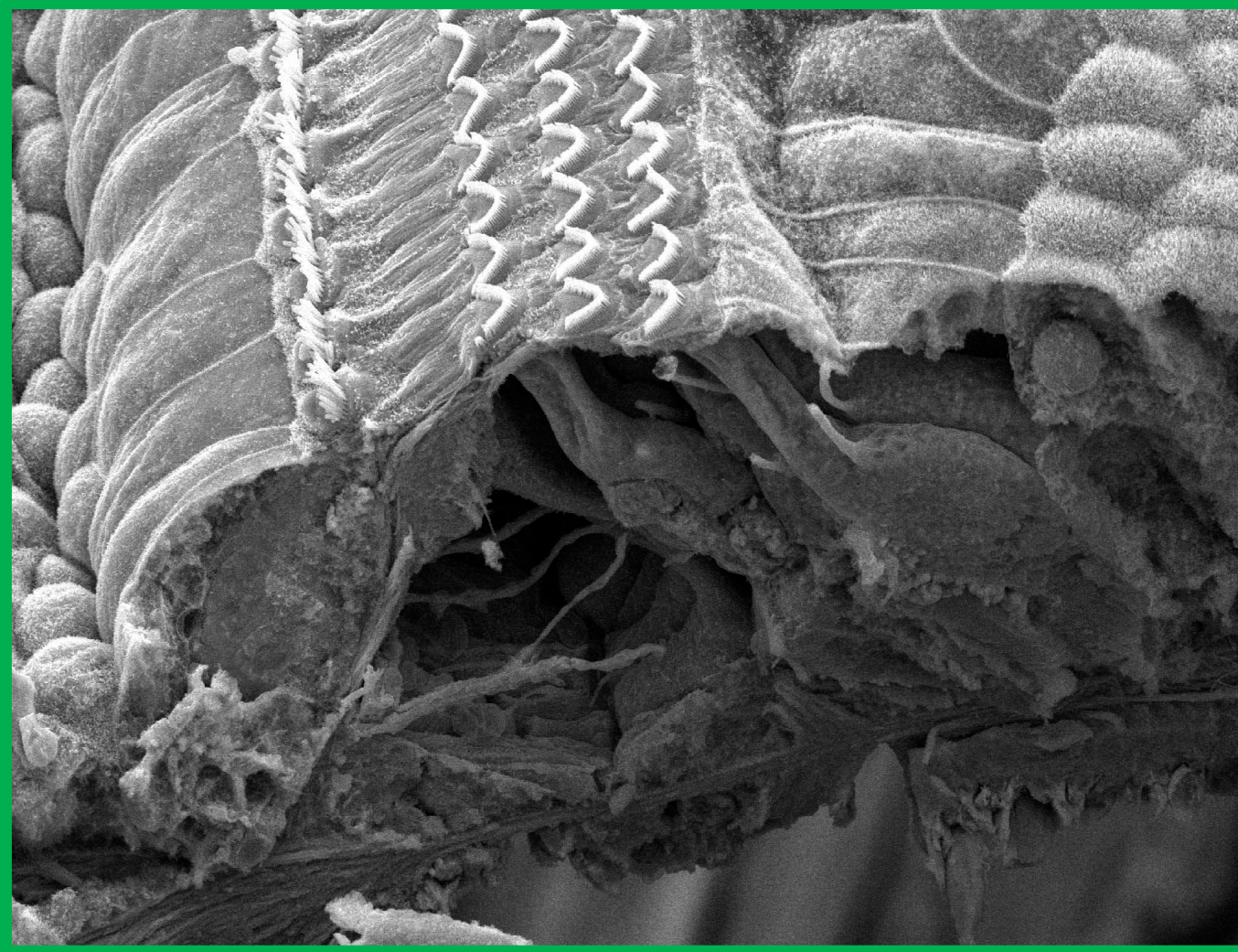


- Understanding the mechanisms underpinning the development, function, and ageing of the mammalian auditory system.
- Understanding the mechanisms underpinning hearing loss and deafness.
- *In vivo* gene-based therapies to prevent and cure hearing loss and deafness.



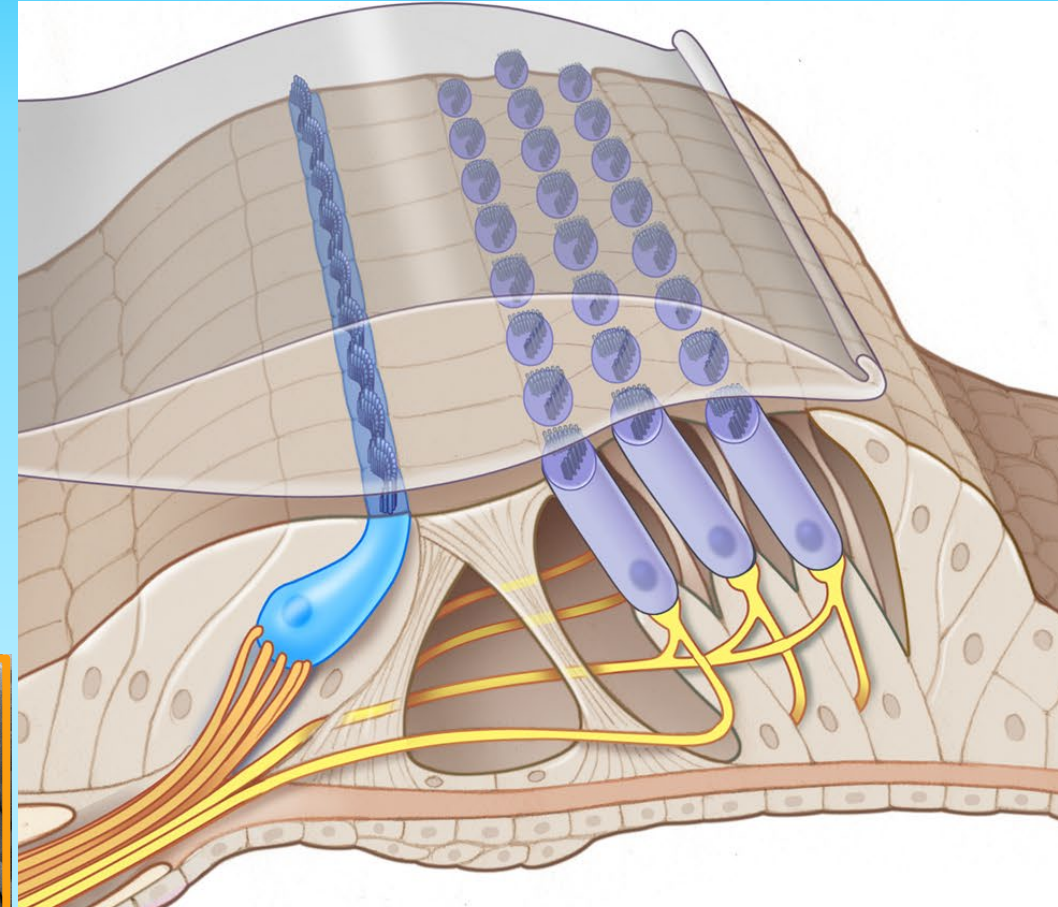
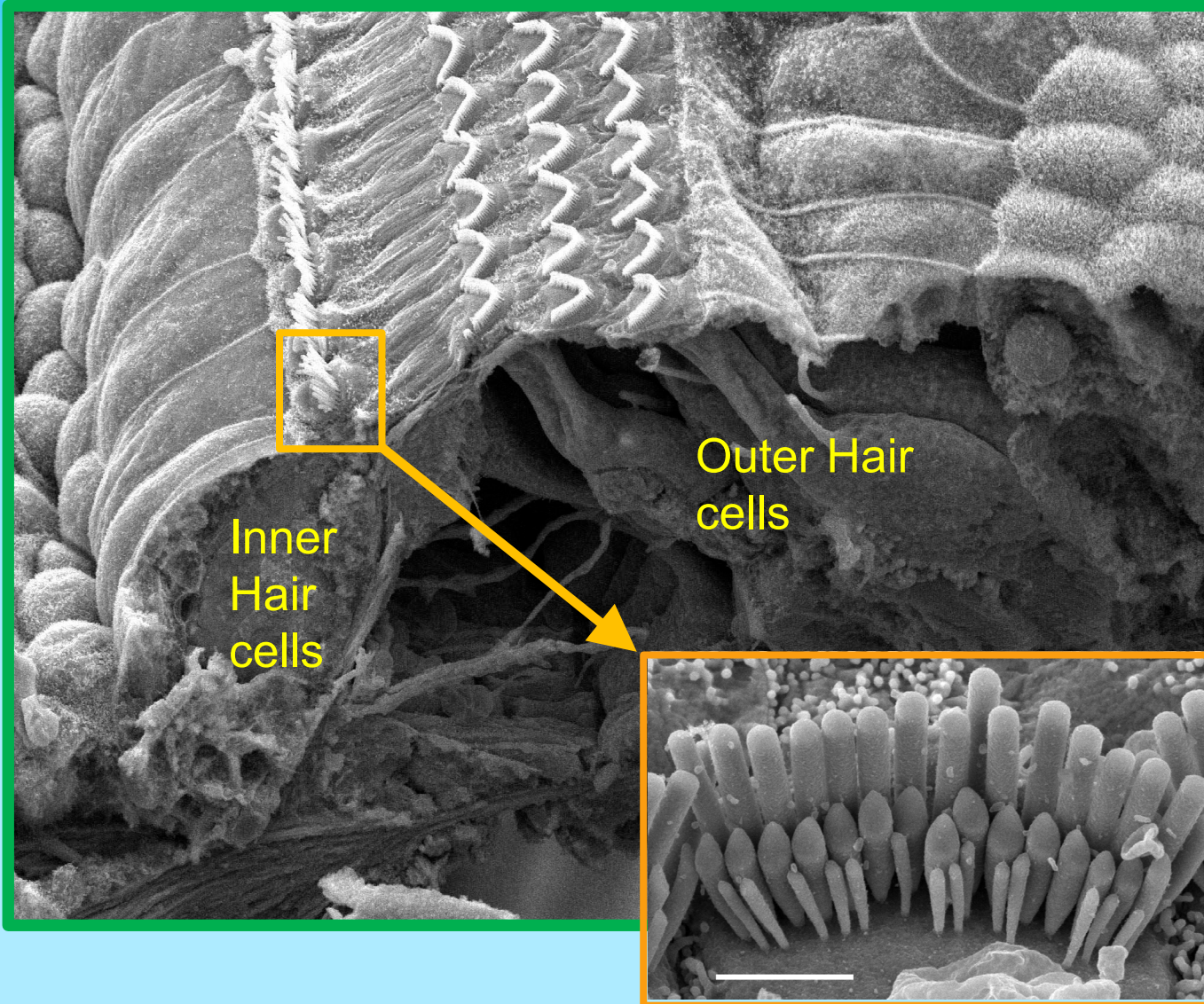
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How do we hear?



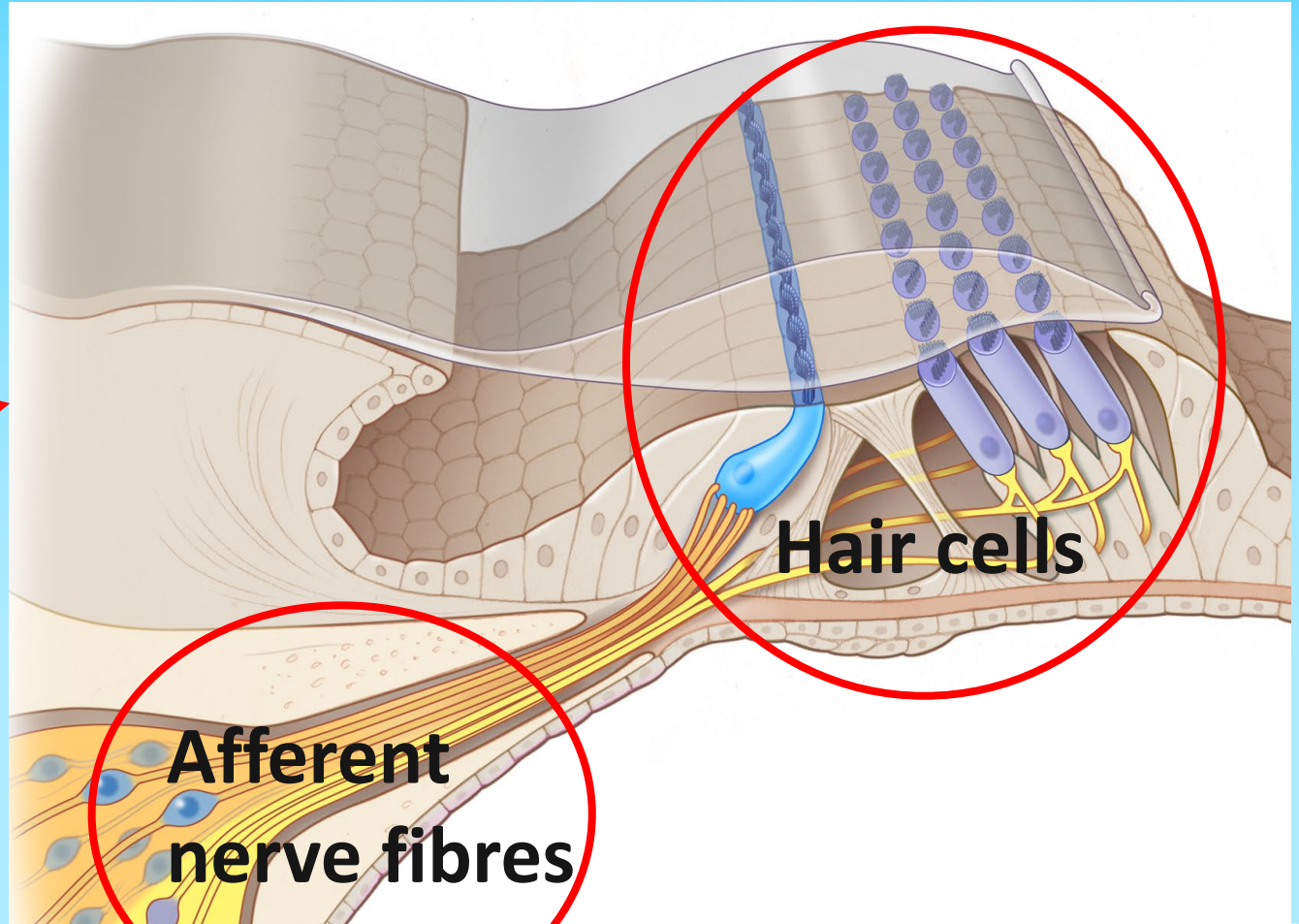
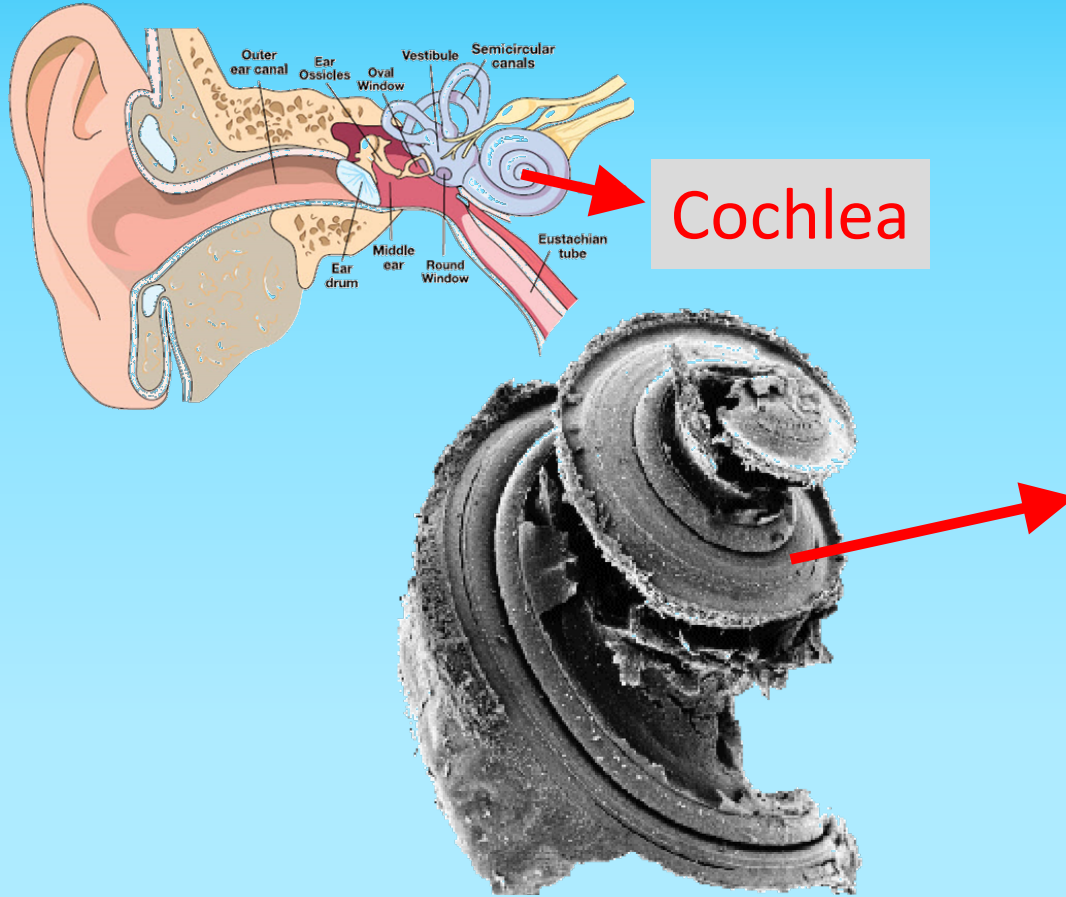


How do we hear?





Hearing loss and deafness targets





What is hearing impairment?

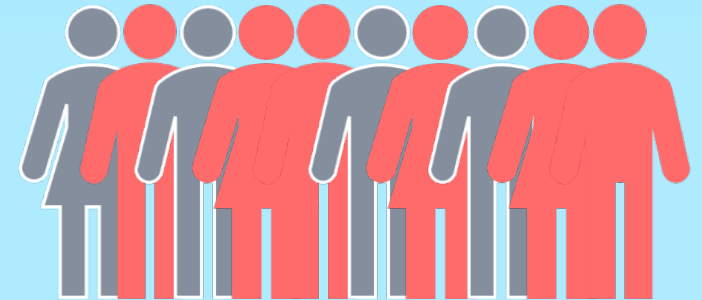


Dysfunction (e.g., tinnitus) or loss of hearing from one or both ears.

Most common sensory disorder in humans.

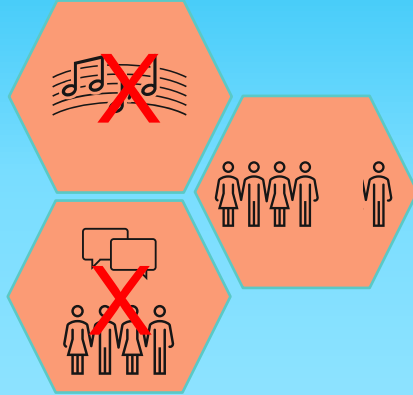
- There are ~50K children with hearing loss in the UK.
- One of the most common health conditions experienced by older adults (age-related hearing loss).

25% of people over 45 &
60% of people over 70
have hearing loss
(>2.5 Billion people in the world by 2050).

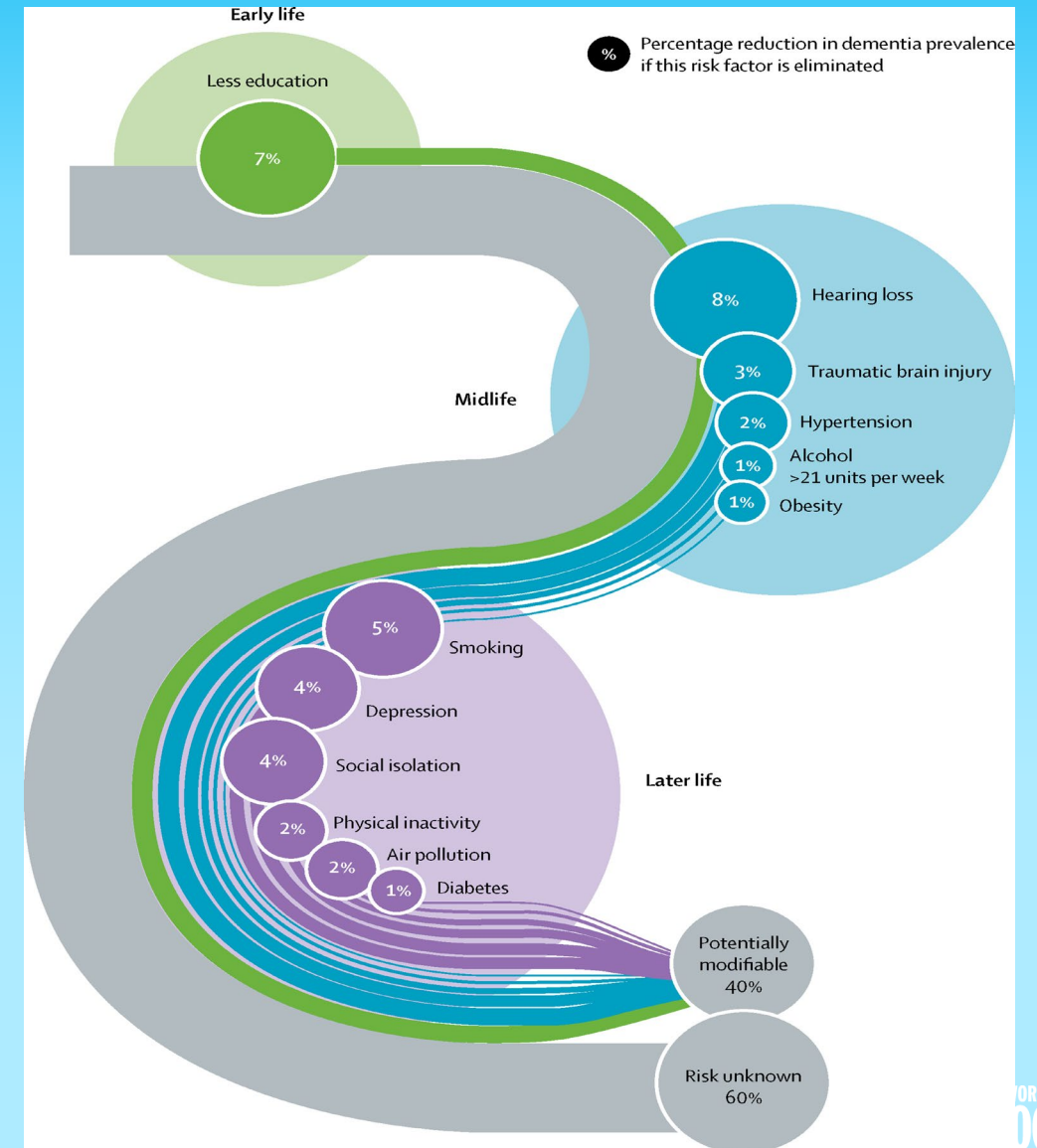




Prevalence of hearing impairment



- Associated with social isolation and depression.
- Linked with cognitive decline and increased risk for dementia diagnosis.





Genetic-based deafness and hearing loss

Despite the clinical prevalence of deafness and age-related hearing loss in the population.....

There are no available Treatments for Deafness and Age-Related Hearing Loss (ARHL) nor Diagnostic tools for an early detection of Hearing loss.



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Our Mission

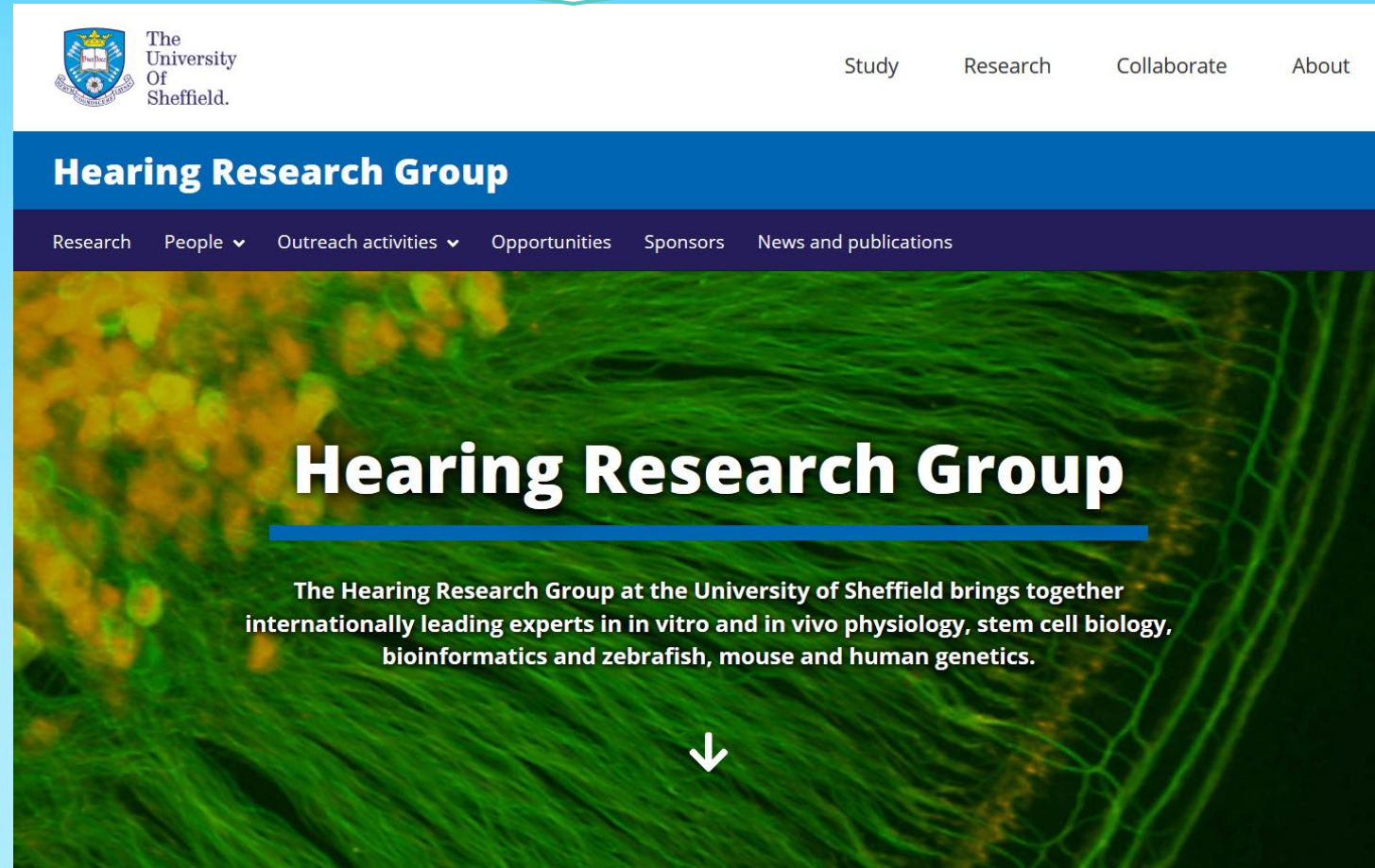
Our Mission

- To advance the discovery of the mechanisms leading to deafness and age-related hearing loss (ARHL).
- To develop diagnostic tools and therapeutic interventions to prevent and cure deafness and ARHL.

Gene therapy

- Replacing defective gene with a functional one.

HEAR US tweet! @HearingShef



The screenshot shows the website for the Hearing Research Group at the University of Sheffield. The page features a blue header with the university logo and navigation links: Study, Research, Collaborate, and About. Below the header is a dark blue banner with the text "Hearing Research Group". A purple navigation bar contains links for Research, People, Outreach activities, Opportunities, Sponsors, and News and publications. The main content area has a green and yellow background with the text "Hearing Research Group" and a description: "The Hearing Research Group at the University of Sheffield brings together internationally leading experts in in vitro and in vivo physiology, stem cell biology, bioinformatics and zebrafish, mouse and human genetics." A white arrow points downwards.

<https://www.sheffield.ac.uk/hearing>

A WORLD
TOP 100
UNIVERSITY

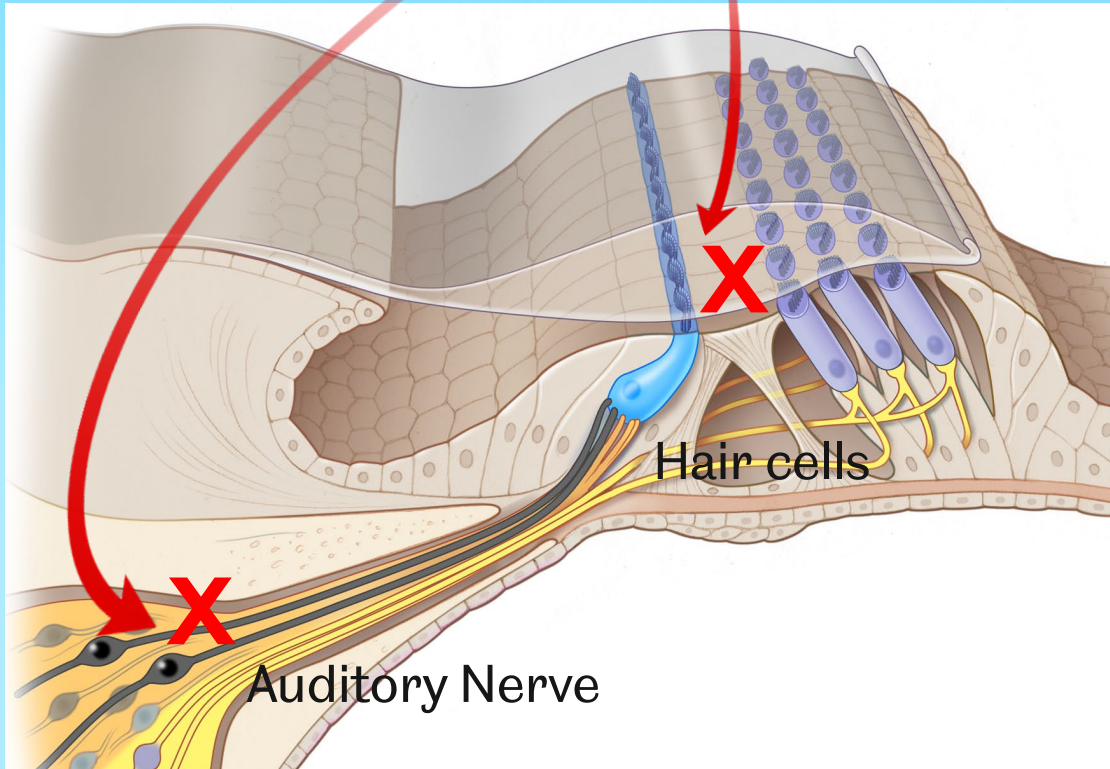


Loss of hair cells and nerve fibres

Genetic mutations

Deafness

Progressive hearing loss
(Age-related hearing loss)



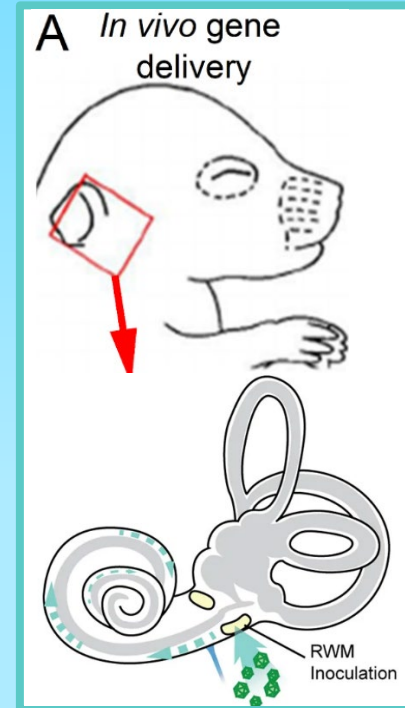
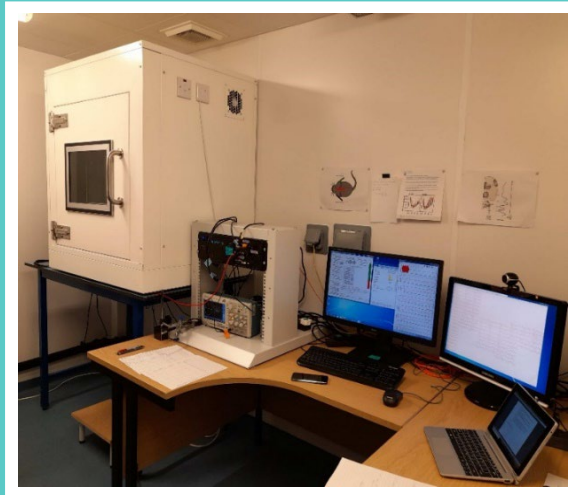
-Highly reduced functioning of
cochlear implants and hearing
aids



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Establish a gene-based therapy for hearing loss

GENE-BASED THERAPY suite for HEARING LOSS @ SHEFFIELD/UK



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Hearing Research Group

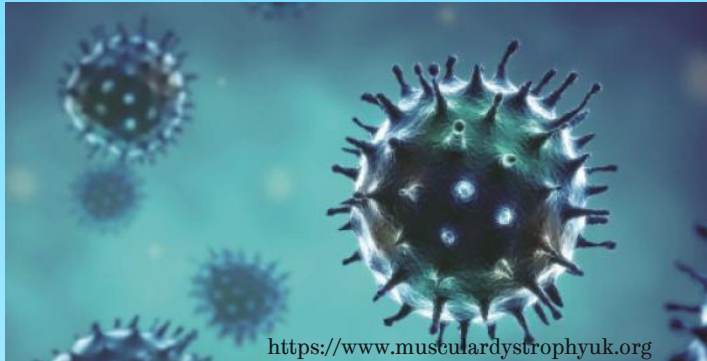
Gene Therapy Suite
funded by

DUDLEY & GEOFFREY
COX CHARITABLE
TRUST

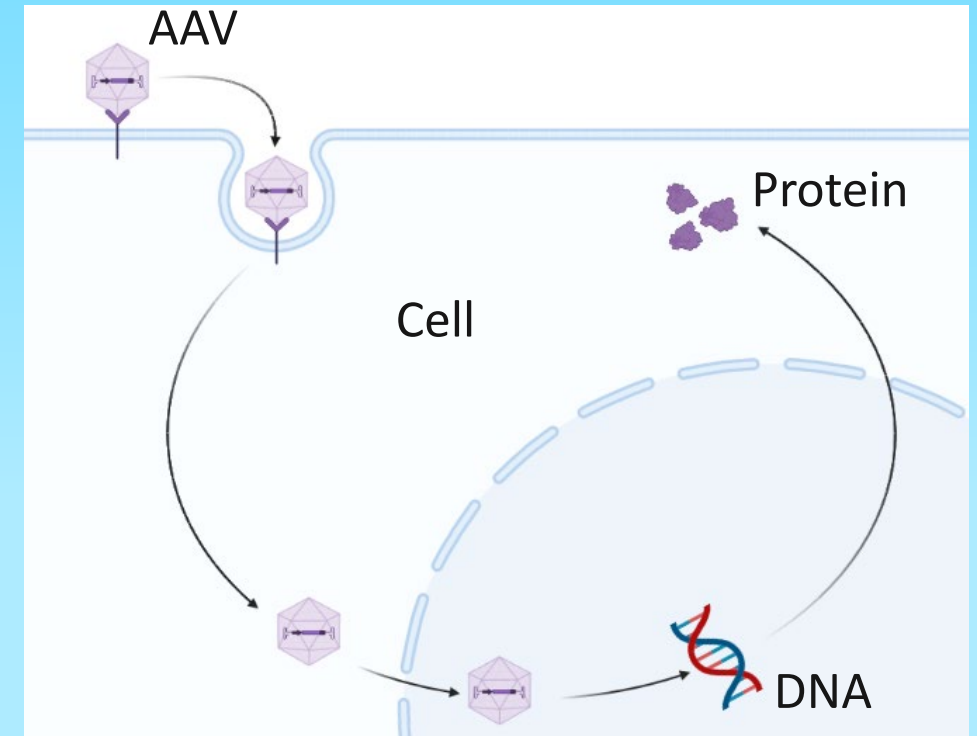


How to deliver “repaired” genes to the dysfunctional hair cells or neurons

Adeno-associated viruses (AAVs)



- Already used in clinical trial because they are safe.
- They are extremely efficient in infecting cells.
- The AAV's own DNA is replaced with the gene of interest.
- They act as a carrier.
- “Infected” cells will express the gene of interest.

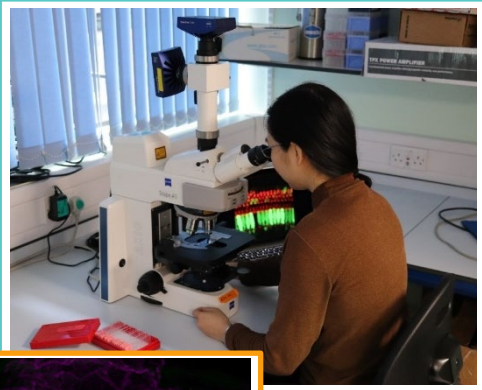




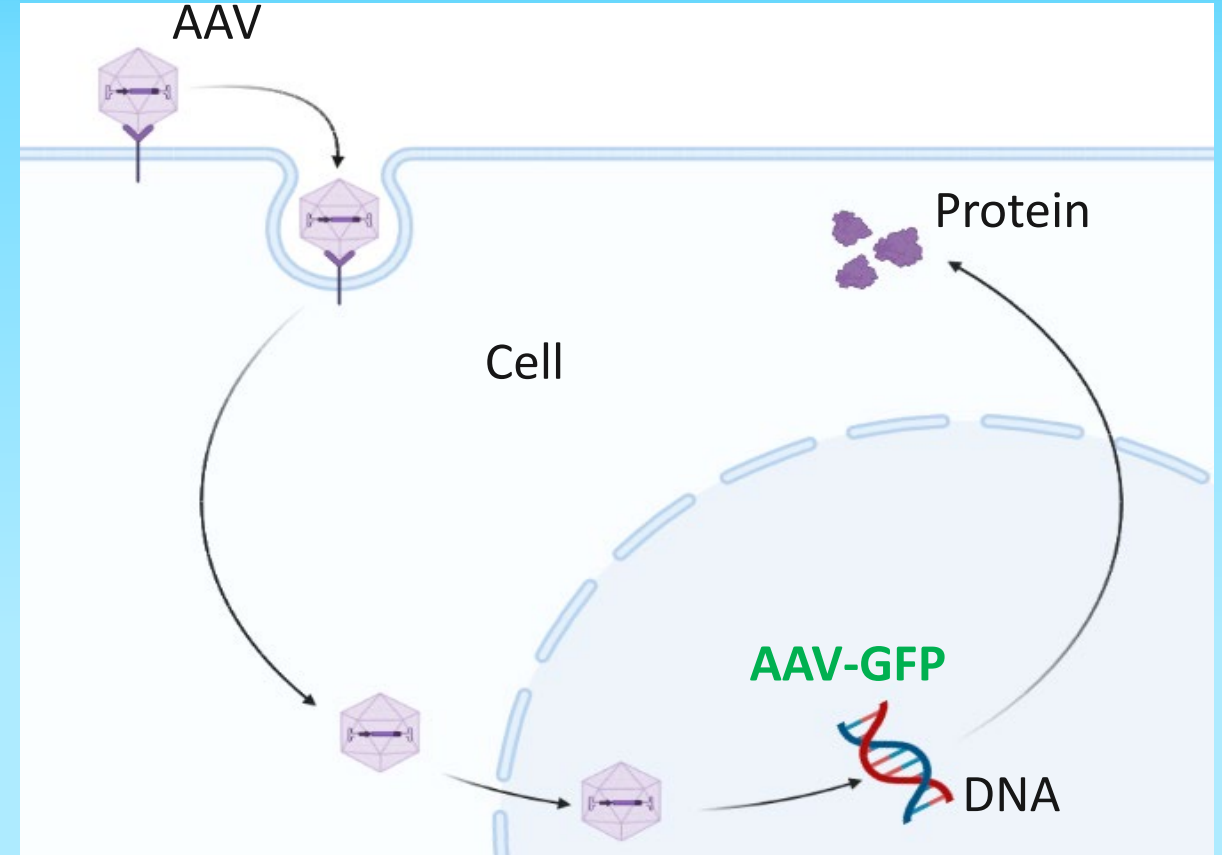
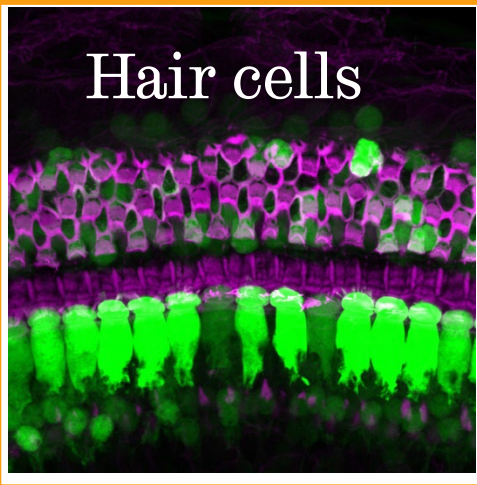
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Delivering “repaired” genes to the hair cells

A *In vivo* gene
delivery



Hair cells





Developing gene-based therapy for hearing loss

Targeting different forms of hearing loss in humans

Age-Related Hearing Loss

Profound deafness



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Research group and Funding

Thank you for listening

HEAR US tweet! @HearingShef



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<https://www.sheffield.ac.uk/hearing>