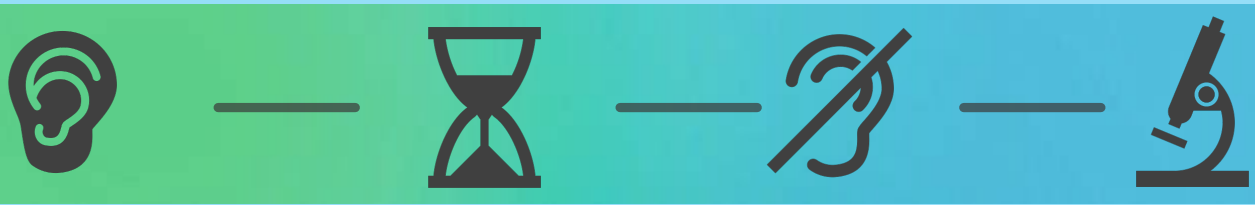
The University Of Sheffield. Neuroscience Institute.

Hearing Research Group

GENE THERAPY RESEARCH

Aiding the development of gene therapies into life-changing treatment for hearing loss



Why do we need a new therapy?

The available therapeutic options to ameliorate hearing loss, hearing aids and cochlear implants, are extremely beneficial for many patients; yet they are unable to restore important features of hearing, such as the ability to understand speech in a noisy environment.
Therefore, we need to develop new therapeutic intervention to treat hearing loss.

What is Gene Therapy?

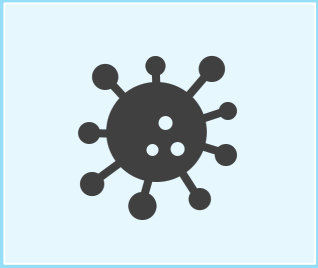
Genes are made of DNA, which is the code that contains all the information required for making molecules that are essential for the development and function of our body. When a gene is missing or does not work properly, essential molecules are either not produced or malfunctioning, potentially causing diseases.

Gene Therapy is a technique used to deliver a normal gene into specific cells to replace the missing or dysfunctional gene causing the disease. The normal gene, when incorporated into the target cells, will restore the normal function and correct the disease.

How does Gene Therapy work?

There are many ways to repair genetic modifications that cause diseases. One of them is using modified viruses to carry and delivering normal genes into the target cells.

These viruses, called adeno-associated viruses (AAVs), are modified so they cannot replicate (i.e. make more of themselves) in the infected cells. This is because DNA of the AAV is replaced with the DNA of a normal copy of the gene that is missing or does not work in diseases, such as hearing loss.



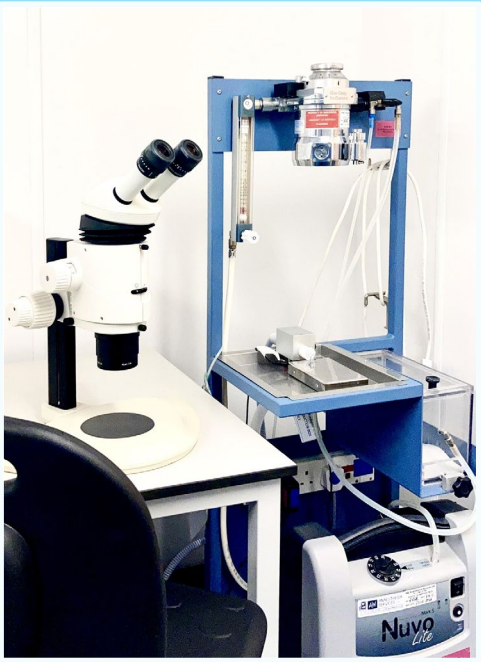
Gene Therapy for hearing loss?

Many forms of hearing loss and deafness are caused by genetic modifications in specific cells in the ear, preventing their normal function. Delivering normal copies of the genes in the affected cells may reinstate the functionality of the cells and restore hearing. Therefore, Gene Therapy is a promising therapeutic strategy to cure genetic forms of hearing loss and deafness.

Gene Therapy at Sheffield?

Since Gene Therapy approaches are currently being developed worldwide, our group at the University of Sheffield is now researching the applicability of this approach to cure hearing loss and deafness. We carefully select our delivery method and AAV subtypes to target specifically the cells in the ear that are affected by the genetic modification.

We are using top-notch equipment for our experimental procedures, including newly established state-of-the-art Gene Therapy suites. This helps us assess the expression and functionality of the healthy genes once inserted back into the auditory system.



The University Of Sheffield. Neuroscience Institute.

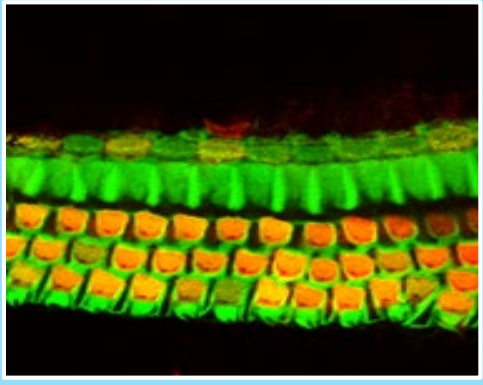
Hearing Research Group

We HEAR YOU – our Promise

Currently, about 12 million people in the UK are affected by hearing loss, with age-related hearing loss (ARHL) being the single biggest cause.

The World Health Organization has also estimated that more than 900 million people worldwide will be affected by some forms of hearing loss by 2050.

Therefore, our aim to evaluate the effectiveness of Gene Therapy for hearing loss and to determine whether our work can have a positive impact on patients. Our wish is to transform the lives of those who are or will be affected by hearing loss.



The University Of Sheffield. Neuroscience Institute.

Hearing Research Group

For further information:

Professor Walter Marcotti
School of Bioscience
Neuroscience Institute
University of Sheffield,
Western Bank, Sheffield S10 2TN

w.marcotti@sheffield.ac.uk

www.sheffield.ac.uk/neuroscience-institute
www.sheffield.ac.uk/hearing

@HearingShef