

Gene Therapy Trial for Otoferlin Gene-mediated Hearing Loss

Status: RECRUITING

Eligibility Criteria

Sex: ALL

Age:

Healthy Volunteers: This study is NOT accepting healthy volunteers

Criteria for Inclusion:

1. Participants may be of any age, based on Cohort Criteria
2. At least two mutations in the otoferlin gene
3. Clinical presentation of Profound bilateral sensorineural hearing loss as assessed by ABR
4. Preserved distortion product otoacoustic emissions (DPOAEs)
5. Able and willing to comply with all study requirements, including willingness to participate in a separate long term follow-up study after completion of this trial

Criteria for Exclusion:

1. Persistent ear infections, anatomic or other abnormalities of the ear, and/or medical conditions that would contraindicate undergoing surgery, anesthesia, and/or administration of investigational gene therapy
2. Cochlear Implant(s) in the ear(s) to receive AAVAnc80-hOTOF
3. Prior participation in a clinical trial with an investigational drug, within six months prior to administration, or any prior participation in a gene therapy clinical trial

Conditions & Interventions

Interventions:

COMBINATION_PRODUCT: AAVAnc80-hOTOF via Akouos Delivery Device, COMBINATION_PRODUCT: AAVAnc80-hOTOF via Akouos Delivery Device and Precision Delivery Mechanism

Conditions:

Sensorineural Hearing Loss, Bilateral

Keywords:

Otoferlin, Auditory neuropathy (AN), Deafness, autosomal recessive 9 (DFNB9), Hearing loss, Hearing loss, bilateral, Hearing loss, sensorineural

More Information

Description:

Part A of this trial will evaluate the safety and tolerability of a single surgical administration procedure in one or both ear(s) with one of two dose levels of AAVAnc80-hOTOF and will evaluate the Akouos Delivery Device, together with the Precision Delivery Mechanism, to safely achieve the intended product performance.

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Principal Investigator:

Principal Investigator ID:

Phase: PHASE1

IRB Number:

System ID: NCT05821959

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