

Gene Therapy Trial for Otoferlin Gene-mediated Hearing Loss

NCT05821959

Status	RECRUITING
Phase	Phase 1, Phase 2
Sponsor	Akouos, Inc.
Enrollment	22 participants

Key Eligibility Criteria

Inclusion (5)

- Participants may be of any age, based on Cohort Criteria
- At least two mutations in the otoferlin gene
- Clinical presentation of Profound bilateral sensorineural hearing loss as assessed by ABR
- Preserved distortion product otoacoustic emissions (DPOAEs)
- 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

Exclusion (3)

- 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
- Cochlear Implant(s) in the ear(s) to receive AAVAnc80-hOTOF
- 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

Locations (9 total)

University of California, San Francisco Benioff Children's Hospital, San Francisco, California, United States
University of Iowa, Iowa City, Iowa, United States
Boston Children's Hospital, Boston, Massachusetts, United States
... and 6 more locations