

# A Study of DB-OTO, an Adeno-Associated Virus (AAV) Based Gene Therapy, in Children/Infants With Hearing Loss Due to Otoferlin Mutations

NCT05788536

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<b>Status</b>	RECRUITING
<b>Phase</b>	Phase 1, Phase 2
<b>Sponsor</b>	Regeneron Pharmaceuticals
<b>Enrollment</b>	30 participants

## Key Eligibility Criteria

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### Inclusion (9)

- Willingness of at least 1 parent/legal guardian to provide written informed consent (and patient to provide assent, when applicable) and willingness to comply with trial protocol; to consent to genetic testing for the patient (and patient to provide assent, when applicable) in order to evaluate a panel of hearing loss-related genes; and to consent to vaccinations for the patient (and patient to provide assent, when applicable) in accordance with the country-specific pediatric immunization schedule as described in the protocol
- Patient is aged <18 years and able to perform all necessary assessments to qualify for enrollment and dosing in the corresponding cohort at the time the parent/legal guardian signing the informed consent form (and patient providing assent, when applicable)
- Presence of biallelic, likely pathogenic or pathogenic OTOF variants
- No clinically significant laboratory findings on clinical laboratory tests at time of Screening as described in the protocol
- Audiological Criteria:
  - ... and 4 more (see full listing online)

### Exclusion (7)

- History of prior treatment with gene therapy
- Surgical anatomy that would preclude or meaningfully impact the planned surgical approach as indicated by medical imaging (eg, computed tomography [CT] or magnetic resonance imaging [MRI]) in the ear(s) to be injected with DB-OTO
- History or presence of other permanent or untreatable hearing loss conditions
- Prior or current history of malignancies
- Prior or current history of meningitis
- ... and 2 more (see full listing online)

## Locations (15 total)

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University of California Los Angeles Medical Center, Los Angeles, California, United States  
Rady Children's Hospital, San Diego, California, United States  
Nemours Children's Clinic, Jacksonville, Florida, United States  
... and 12 more locations

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<https://clinicaltrials.gov/study/NCT05788536>

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