

Does your child have sensorineural hearing loss?

Learn more about a clinical research study for children with hearing loss caused by specific genetic changes, or mutations, called otoferlin gene (*OTOF*) mutations.

What is the AK-OTOF-101 study?

The goal of the study is to find out if the study drug, administered using the study device, is safe and improves hearing in children with sensorineural hearing loss (SNHL) due to otoferlin gene mutations.

About genes and hearing loss

- Our genes provide instructions to our cells to make proteins that help us live and grow. The otoferlin gene provides instructions for the body to make otoferlin, a protein necessary for hearing.
- When there are changes, or mutations, in the otoferlin gene, the body may not be able to make functional otoferlin protein.
- Without functional otoferlin protein, sounds cannot be communicated from the ear to the brain, resulting in hearing loss.
- Children with otoferlin gene mutations typically have a type of SNHL called auditory neuropathy. Auditory neuropathy is a type of SNHL where the inner ear detects sound but cannot send the sound signal to the brain.

What are the study drug and study device?

The study drug (AAVAnc80-hOTOF, or AK-OTOF) is a **gene therapy**. This gene therapy is designed to deliver a working copy of the otoferlin gene to cells in the ear. With a working copy of the gene in the cells of the cochlea, the organ of hearing, the body may be able to make functional otoferlin protein and may improve hearing.

The study drug will be administered to the inner ear during a surgical procedure using the study device.

Both the study drug and the study device are **investigational**, which means they can only be used in research trials. They have not been approved by regulatory authorities like the US Food and Drug Administration (FDA) or European Medicines Agency (EMA). This is the first time the study drug and the device are being used in humans.

What will happen during the study?

▪ Screening Period

During the Screening period, your child will receive assessments to confirm they qualify to join the study.

▪ Study drug administration

If qualified, your child will have surgery to receive one dose of the study drug (using the study device) in one ear. Your child will stay overnight at the site after surgery so the study staff can monitor their health.

▪ Follow-up visits

You and your child will attend 11 follow-up visits at the site over the course of 2 years. You and your child will also be expected to participate in a Long Term Follow-up Study (with annual visits) for an additional 3 years.

You may be eligible for travel reimbursement.

Who can join the study?

To join the study, your child must meet the following requirements*:

- Between age 7 to 17 years old (inclusive, Group 1a); between age 2 to 17 years old (inclusive, Groups 1b and 2) with sensorineural hearing loss
- Otoferlin gene mutations confirmed by genetic testing (genetic testing will be provided, as needed)
- Does not have cochlear implants in both ears

*Other study requirements will apply.

What is a clinical research study?

Clinical research studies help scientists and doctors explore whether a medical strategy, drug, or device is safe and effective for people. Before a new study drug or study device can be approved and made available to the public, it must go through phases of clinical research. Each phase helps researchers learn more and relies on volunteer participants.

For more information about the AK-OTOF-101 study, visit www.otofclinicaltrial.com.