Supplementary Information

Cochlear gene therapy with ancestral AAV in adult mice: complete transduction of inner hair cells without cochlear dysfunction

Jun Suzuki^{1,2,3}, Ken Hashimoto^{1,2,3}, Ru Xiao^{4,5}, Luk H. Vandenberghe^{4,5,6} and M. Charles Liberman^{1,2}

¹Department of Otology and Laryngology, Harvard Medical School, Boston, MA 02115, USA

² Eaton-Peabody Laboratories, Massachusetts Eye & Ear Infirmary, Boston, MA 02114, USA

³ Department of Otorhinolaryngology-Head and Neck Surgery, Tohoku University Graduate School of Medicine, Sendai, Miyagi 980-8574, Japan

⁴ Grousbeck Gene Therapy Center, Schepens Eye Research Institute and Massachusetts Eye & Ear Infirmary, Boston, MA 02114, USA

Ocular Genomics Institute, Department of Ophthalmology, Harvard Medical School, Boston, MA 02114, USA

⁶ Harvard Stem Cell Institute, Harvard University, Cambridge, MA 02138, USA

Supplementary Figure Legends

Supplemental Figure 1: Transfection efficiency in OHCs can be close to 100%. The percentage of EGFP positive OHCs as a function of cochlear frequency/location in each of the 13 individual cases studied.

Supplemental Figure 2: The surgical approach to the posterior semicircular canal. **A:** View of the left side of the skull after separating and scraping the overlying muscle and fascia. Dashed box is shown at higher magnification in the next panel. **B:** Final view of canalostomy, showing its position relative to the lateral and posterior canals, which are visible through the exposed skull. **C:** Insertion of a polyimide tube into the posterior canal. **D:** Horizontal, plastic section through an un-operated mouse of the same strain and age, showing the posterior canal where it enters the ampulla. The polyimide tube (red; schematized) is drawn to scale: O.D. = $124 \mu m$.

Supplementary video S1: Surgery video (avi file, 51.6 MB)



