

# Gene Therapy Restores Balance and Auditory Functions in a Mouse Model of Usher Syndrome

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Following the publication of the article, the authors realized that the Whirlin cDNA (NCBI accession no. AY739114) used in the study contains two synonymous variants (Leu476Leu (CTC to CTT), and Thr537Thr (ACA to ACG)) and one non-synonymous variant (Gly550Arg (GGA to AGA)). The authors regret this error. We corrected all three variants to conform to the wild type mouse sequence (accession number AY739114) and repeated the inner ear gene therapy experiments in the whirler mutant mice (*Whrn*<sup>wi/wi</sup>) as published in the original study. The corrected AAV8-whirlin was delivered via the posterior canal approach and caused a partial improvement in the auditory function in the *Whrn*<sup>wi/wi</sup> mice (mean ABR thresholds of  $97.5 \pm 1.64$ ,  $81.9 \pm 5.08$ ,  $95.0 \pm 2.67$ , and  $95.7 \pm 2.59$  at stimulus frequencies of 4 kHz, 8 kHz, 16 kHz, and 32 kHz, respectively, n=5). The magnitudes of ABR threshold improvement are similar to our previously published results (p = 0.27, ANOVA). The corrected AAV8-whirlin delivered via the posterior canal approach also led to an improvement in the vestibular function in the *Whrn*<sup>wi/wi</sup> mice, as indicated by a reduction in circling behavior ( $7.20 \pm 0.43$  rotations per 2 min). This improvement in vestibular function is also similar to our previously published results (p = 0.41, t-test). Thus, correction of the variants that were present in the original whirlin cDNA did not alter the conclusions of the study.