
BRIDGE-BDNF - Brain-derived neurotrophic factor restoration via intelligent delivery for gene enhancement

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The novel adeno-associated virus (AAV) capsid variant, developed by the project team, allows for the first time an efficient transduction of spiral ganglion neurons (neurons of the cochlea) and supporting cells in all cochlear turns at low vector dosage. With this novel vector, the researcher could prove in a mouse model a protective effect on spiral ganglion neurons after induced hair cells loss.

The aim of the project is to optimize the expression of the factor which is considered crucial for the long-term preservation of spiral ganglion neurons. Therefore, various promoters and their influence on the expression of the factor are being tested in vitro. This initial testing is complemented by in vivo testing in an established cochlear implant model. In preparation for translation, the scientists will then validate their approach in a relevant large animal model. This project describes the essential next steps for transferring the therapeutic approach into a clinical trial phase. In addition, the existing production protocol for the optimized vector will be transferred to a GMP-compliant manufacturing process.
