

Title (en)

RECOMBINANT ADENO ASSOCIATED VIRUS (RAAV) ENCODING GJB2 AND USES THEREOF

Title (de)

FÜR GJB2 CODIERENDES REKOMBINANTES ADENO-ASSOZIIERTES VIRUS (RAAV) UND VERWENDUNGEN DAVON

Title (fr)

VIRUS ADÉNO-ASSOCIÉ RECOMBINANT (RVAA) CODANT GJB2 ET SES UTILISATIONS

Publication

EP 4211151 A1 20230719 (EN)

Application

EP 21867807 A 20210914

Priority

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Abstract (en)

[origin: WO2022056444A1] The present disclosure, at least in part, relates to compositions (e.g., isolated nucleic acid and rAAVs) and methods for treating Non-syndromic hearing loss and deafness (DFNB1) by delivering gap junction beta 2 (GJB2) protein to inner ear cells that normally express GJB2 (e.g., fibrocytes and supporting cells of the organ of Corti and nearby regions). The isolated nucleic acid of the present disclosure comprises an expression cassette, wherein the expression cassette comprises a gap junction beta 2 (GJB2) gene regulatory element (GRE) (e.g., GJB2 enhancers, GJB2 promoters, GJB2 5' UTR, and/or GJB2 3' UTR), and a nucleotide sequence encoding a GJB2 protein.

IPC 8 full level

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