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(54) Title: VECTORS

(57) Abstract: The present invention provides a dual adeno-associated virus (AAV) vector system for expressing a COL4A3, COL4A4 or COL4A5 polypeptide comprising: (a) a first AAV vector comprising a promoter and a 5' coding sequence (CDS), wherein the 5' CDS encodes an N-terminal part of the COL4A3, COL4A4 or COL4A5 polypeptide; and (b) a second AAV vector comprising a 3' CDS, wherein the 3' CDS encodes a C-terminal part of the COL4A3, COL4A4 or COL4A5 polypeptide. The present invention also provides cells and pharmaceutical compositions comprising said dual AAV vector system and uses thereof, for example for preventing and/or treating Alport Syndrome and related conditions (e.g. any condition in a subject who has a pathogenic variant of a COL4A3, COL4A4 or COL4A5 gene).



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