

Title (en)
FACTOR VIII MUTATION REPAIR AND TOLERANCE INDUCTION AND RELATED CDNAs, COMPOSITIONS, METHODS AND SYSTEMS

Title (de)
FAKTOR-VIII-MUTATIONSREPARATUR UND TOLERANZINDUKTION SOWIE ENTSPRECHENDE CDNA, ZUSAMMENSETZUNGEN, VERFAHREN UND SYSTEME

Title (fr)
RÉPARATION DE MUTATIONS ET INDUCTION DE TOLÉRANCE DU FACTEUR VIII ET ADNC, COMPOSITIONS, PROCÉDÉS ET SYSTÈMES ASSOCIÉS

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Abstract (en)
[origin: WO2015191899A1] The present disclosure relates to methods, systems, and compositions to repair one or more mutations in a Factor VIII gene sequence of a subject by introducing into a cell of the subject one or more polynucleotides encoding a DNA scission enzyme (DNA-SE) and one or more repair vehicles (RVs) containing at least a cDNA-repair sequence (RS) such that insertion of the cDNA-RS through homologous recombination with the F8 gene of the subject (sF8) provides a repaired F8 gene (rF8), the repaired F8 gene (rF8) upon expression forming a functional FVIII conferring improved coagulation functionality to the FVIII protein encoded by the sF8. The present disclosure also relates to cells derived using the methods, systems and compositions described.

IPC 8 full level
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