ABSTRACT

MODIFIED FACTOR IX, AND COMPOSITIONS, METHODS AND USES FOR GENE TRANSFER TO CELLS, ORGANS AND TISSUES

The invention relates to modified Factor IX coding sequence, expression cassette, vectors such as viral (e.g., lenti- or adeno-associated viral) vectors, and gene transfer methods and uses. In particular, to target Factor IX nucleic acid to cells, tissues or organs for expression (transcription) of Factor IX.

(Figure 19)