

STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-202)

ETRANACOGENE DEZAPARVOVEC

PRONUNCIATION

e" tra nak' oh jeen dez" a par' voe vek

THERAPEUTIC USE

Treatment of Coagulation factor IX deficiency
(Hemophilia B)

DESCRIPTION

DNA (synthetic adeno-associated virus 5 vector AMT-061 human
blood-coagulation factor IX padua variant-specifying) (CA INDEX NAME)

SEQUENCE

DNA sequence of the AAV cassette

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SEQ      1 gggccactcc ctctctgctg gctcgcctgc tcaactgaggc cgggagacca
      51 aaggctgccc gacgcccggg ctttgcccgg gcggcctcag tgagcgagcg
     101 agcgcgcaga gagggagtgg ccaactccat cactaggggt tcctggaggg
     151 gtggagtctg gaccctaaa atgggcaaac attgcaagca gcaaacagca
     201 aacacacagc cctccctgcc tgctgacctt ggagctgggg cagaggtcag
     251 agacctctct gggcccatgc cacctccaac atccactcga ccccttgtaa
     301 tttcggtgga gaggagcaga ggttgcctct gcgtggttta ggtagtgtga
     351 gaggggaatg actcctttcg gtaagtgcag tggagctgtg aactgcccga
     401 ggcaaagcgt ccgggcagcg taggcggggc actcagatcc cagccagtgg
     451 acttagcccc tgtttgctcc tccgataact ggggtgacct tggttaatat
     501 tcaccagcag cctccccctg tgcccctctg gatccactgc ttaaatacgg
     551 acgaggacag ggccctgtct cctcagcttc aggcaccacc actgacctgg
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     651 gtgttaaaact actgattcta attgtttctc tcttttagat tccaaccttt
     701 ggaactgaat tctagaccac catgcagagg gtgaacatga tcatggctga
     751 gagccctggc ctgatcacca tctgcctgct gggctacctg ctgtctgctg
     801 agtgacactgt gttcctggac catgagaatg ccaacaagat cctgaacagg
     851 cccaagagat acaactctgg caagctggag gagtttgcgc agggcaacct
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    1001 ggggaccagt gtgagagcaa cccctgcctg aatgggggca gctgcaagga
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    1101 actgtgagct ggatgtgacc tgcaacatca agaatggcag atgtgagcag
    1151 ttctgcaaga actctgctga caacaagggt gtgtgcagct gcaactgagg
    1201 ctacaggctg gctgagaacc agaagagctg tgagcctgct gtgccattcc
    1251 catgtggcag agtgtctgtg agccagacca gcaagctgac cagggtctgag
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    1351 cctggacaac atcaccagga gcaccagag cttcaatgac ttcaccaggg
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    1451 ctgaatggca aggtggatgc cttctgtggg ggcagcattg tgaatgagaa
    1501 gtggattgtg actgctgccc actgtgtgga gactgggggt aagatcactg
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    1601 aggaatgtga tcaggatcat cccccaccac aactacaatg ctgccatcaa
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    1951 ctctgggggc ccccatgtga ctgaggtgga gggcaccagc ttcctgactg
    2001 gcatcatcag ctgggggggag gagtgtgcca tgaagggcaa gtatggcatc
    2051 tacaccaaag tctccagata tgtgaactgg atcaaggaga agaccaagct
    2101 gacctgactc gatgctttat ttgtgaaatt tgtgatgcta ttgctttatt
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2151 tgtaaccatt ataagctgca ataaacaagt taacaacaac aattgcattc
2201 attttatggt tcaggttcag ggggaggtgt gggaggtttt ttaaactagg
2251 tcacgactcc acccctccag gaaccctag tgatggagtt ggccactccc
2301 tctctgcgcg ctcgctcgct cactgaggcc gcccgggcaa agcccgggcg
2351 tcgggcgacc tttggtcgcc cggcctcagt gagcgagcga gcgcgcagag
2401 agggagtggc cc

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Amino Acid Sequence of the Human FIX-Padua Protein Encoded by the hFIXco-Padua Expression Cassette:

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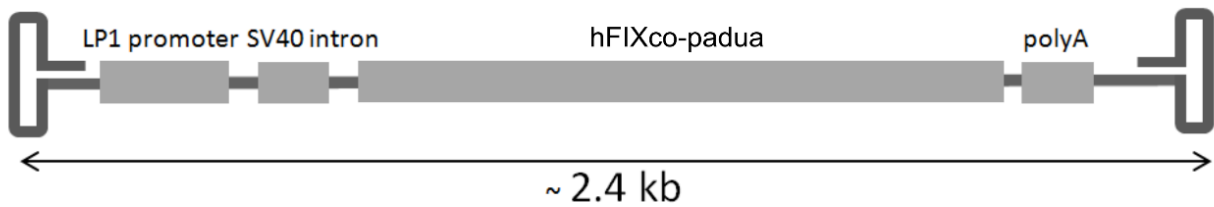
MQRVNMIMAE SPGLITICLL GYLLSAECTV FLDHENANKI LNRPKRYNSG 50
KLEEFVQGNL ERECMEEKCS FEEAREVFEN TERTTEFWKQ YVDGDQCESN 100
PCLNGGSKD DINSYECWCP FGFEGKNCEL DVTCNIKNGR CEQFCKNSAD 150
NKVVCSTEG YRLAENQKSC EPAVPFPCGR VSVSQTSKLT RAEAVFPDVD 200
YVNSTEAETI LDNITQSTQS FNDFTRVVG EDAKPGQFPW QVVLNGKVDA 250
FCGGSIVNEK WIVTAAHCVE TGVKITVVG EHNIEETEHT EQKRNVIIRI 300
PHHNYNAAIN KYNHDIALLE LDEPLVLNSY VTPICIIDKE YTNIFLKFSG 350
GYVSGWGRVF HKGRSALVLQ YLRVPLVDRA TLLSTKFTI YNNMFCAGFH 400
EGGRDSCQGD SGGPHVTEVE GTSFLTGIIS WGEECAMKGG YGIYTKVSRY 450
VNWIKEKTKL T 461

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TABLE OF FEATURES

Feature	Description	Position
AAV ITR	Left AAV-2 inverted terminal repeat	1-144
LP1 promoter	Liver specific promoter	163-611
SV40 intron	SV40 intron	616-708
hFIXco-Padua	Codon optimized human Factor IX-Padua	722-2107
polyA	Late polyA signal	2113-2245
AAV ITR	Right AAV-2 inverted terminal repeat	2269-2412

SCHEMATIC MAP



TRADEMARK None

SPONSOR uniQure biopharma B.V.

CODE DESIGNATIONS AMT-061; AAV5-hFIXco-Padua

CAS REGISTRY NUMBER 2156583-26-3

UNII Z5XCD5Q9RL

WHO NUMBER 10963

SCS