

# STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (JK-252)

BEVUFENOGENE NOFEPARVOVEC

PRONUNCIATION

be" vue fen' oh jeen noe" fe par' voe vec

THERAPEUTIC USE

Treatment phenylalanine hydroxylase deficiency

## DESCRIPTION

DNA (recombinant human hematopoietic stem cell-derived adeno-associated virus vector serotype AAVHSC15 human liver-specific expression cassette including human phenylalanine 4-hydroxylase codon-optimized cDNA plus adeno-associated virus 2 inverted terminal repeat flanks) One ITR contains a deleted terminal resolution site ( $\Delta$ trs) resulting in the formation of a self-complimentary AAV genome (CA INDEX NAME)

## SEQUENCE

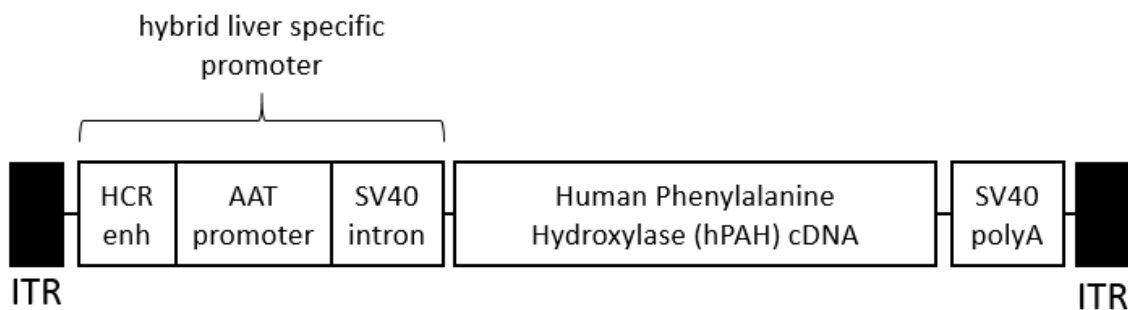
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CTGCGCGCTC GCTCGCTCAC TGAGGCCGCC CGGGCAAAGC CCGGGCGTCG 50
GGCGACCTTT GGTCGCCCGG CCTCAGTGAG CGAGCGAGCG CGCAGAGAGG 100
GAGTGGAATT CACGCGTGGA TCTGAATTCA ATTCACGCGT GGTACCTCCC 150
TAAAATGGGC AAACATTGCA AGCAGCAAAC AGCAAACACA CAGCCCTCCC 200
TGCCTGCTGA CCTTGGAGCT GGGGCAGAGG TCAGAGACCT CTCTGGGCCC 250
ATGCCACCTC CAACATCCAC TCGACCCCTT GGAATTTTCGG TGGAGAGGAG 300
CAGAGGTTGT CCTGGCGTGG TTTAGGTAGT GTGAGAGGGG AATGACTCCT 350
TTCGGTAAAGT GCAGTGGAAG CTGTACTACTG CCCAGGCAA GCGTCCGGGC 400
AGCGTAGGCG GGCGACTCAG ATCCCAGCCA GTGGACTTAG CCCCTGTTTG 450
CTCCTCCGAT AACTGGGGTG ACCTTGTTTA ATATTCACCA GCAGCCTCCC 500
CCGTTGCCCC TCTGGATCCA CTGCTTAAAT ACGGACGAGG ACAGGGCCCT 550
GTCTCCTCAG CTTCAGGCAC CACCACTGAC CTGGGACAGT GAATCCTCTA 600
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CCACCGCTGT GCTGGAGAAC CCTGGGCTGG GGAGGAAACT GTCAGACTTC 750
GGGCAGGAGA CTTCATAACAT TGAGGATAAC TGTAACCAGA ATGGCGCCAT 800
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TGCGCCTGTT TGAGGAGAAC GACGTGAATC TGACCCACAT CGAGTCCCGG 900
CCTTCTAGAC TGAAGAAGGA CGAGTACGAG TTCTTTACCC ACCTGGATAA 950
GCGGTCCCTG CCAGCCCTGA CAAACATCAT CAAGATCCTG AGGCACGACA 1000
TCGGAGCAAC CGTGCACGAG CTGTCTCGGG ACAAGAAGAA GGATACCGTG 1050
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CCTGTCTTAC GGAGCAGAGC TGGACGCAGA TCACCCTGGC TTCAAGGACC 1150
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GACCTGGGGC ACAGTGTTC AAGACCCTGAA GAGCCTGTAC AAGACACACG 1300
CCTGCTACGA GTATAACCAC ATCTTCCCC TGCTGGAGAA GTATTGTGGC 1350
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GACCTGCACA GGCTTTAGGC TGAGGCCAGT GGCAGGACTG CTGAGCTCCC 1450
GGGACTTCCT GGGAGGACTG GCCTTCAGAG TGTTCCTACTG CACCCAGTAC 1500
ATCAGGCACG GCTCCAAGCC AATGTATACA CCAGAGCCCG ACATCTGTCA 1550
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TTTCCCAGGA GATCGGACTG GCATCTCTGG GAGCACCTGA CGAGTACATC 1650
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TCGCCGCCAC AATCCCTAGG CCCTTCAGCG TGCGGTACGA CCCTTATAAC 1950
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CTCAATCAAT AGCGAAATCG GAATCCTGTG CTCCGCCCTG CAGAAAATCA 2050
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TGTTTCAGGT TCAGGGGGAG GTGTGGGAGG TTTTTTAAAG CATGCTGGGG 2200
AGAGATCGAT CTGAGGAACC CCTAGTGATG GAGTTGGCCA CTCCTCTCT 2250
GCGCGCTCGC TCGCTCACTG AGGCCGGGCG ACCAAAGGTC GCCCGACGCC 2300
CGGGCTTTGC CCGGGCGGCC TCAGTGAGCG AGCGAGCGCG CAGAGAGGGA 2350
GTGGCC 2356

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## SCHEMATIC MAP



Feature	Description	Position
ITR	5' inverted terminal repeat	1-106
HCR	Human Hepatic control region (HCR-1) enhancer element	148-339
AAT promoter	Human alpha-1-antitrypsin (hAAT) gene promoter	341-545
SV40 intron	SV40 small T antigen	596-688
hPAH cDNA	Human phenylalanine hydroxylase (PAH) gene, codon optimized	697-2055
SV40 polyA	SV40 late polyA	2057-2189
ITR	3' inverted terminal repeat	2214-2356

TRADEMARK None yet

SPONSOR Homology Medicines, Inc.

CODE DESIGNATION HMI-102

CAS REGISTRY NUMBER 2374772-30-0

UNII W6GL3Z8KWT

WHO NUMBER 11521

SCS