STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

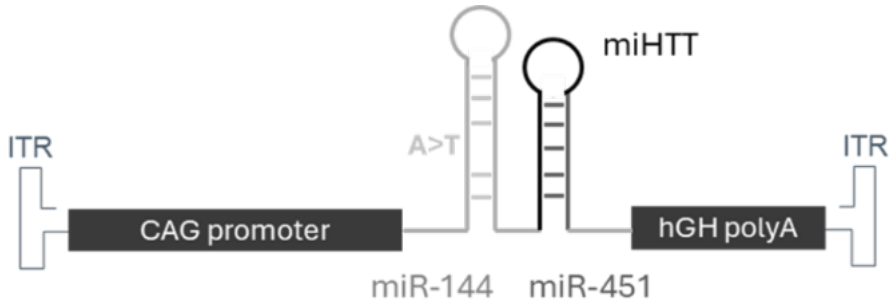
USAN (OP-103)	IFEZUNTIRGENE INILPARVOVEC
PRONOUNCIATION	eye fe" zun tir' jeen in" il par' voe vek
THERAPEUTIC CLAIM	Treatment of Huntington's Disease

DESCRIPTION

DNA (recombinant replication-deficient adeno-associated virus 5 vector AMT-130 chicken β-actin promoter plus human huntingtin mRNA-targeting microRNA miHTT-specifying)

STRUCTURAL FORMULA

Genetic Map of the entire product: Schematic representation of the vector genome (not at scale), consisting of the synthetic CAG promoter, the miHTT expression cassette and the human growth hormone (hGH) polyA sequence, flanked by inverted terminal repeats (ITRs). The miRNA expression cassette comprises the miR-144 helper hairpin, which is not expressed due to the A>T mutation, and the engineered miR-451 scaffold encoding the therapeutic miHTT.



Linear DNA sequence (bp): Predicted hairpin structure of the 72-nt primary miRNA (pri-miRNA-451) that is generated for miHTT. The 21-nt that were designed to be homologous to the target site in the *HTT* mRNA is highlighted.

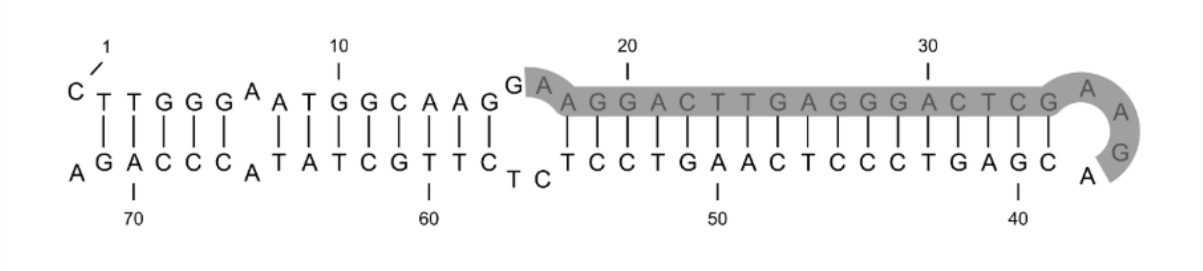


Table of features:

Sequence Element	Position
AAV ITR -Left AAV2 inverted terminal repeat	1 - 145
Cloning Sequence	146 - 279

CAG promoter, composed of CMV Enhancer Promoter, first exon and first intron of chicken beta actin gene Splice acceptor of the rabbit beta-globin gene	280 - 1949
Cloning region	1950 - 2054
Flanking region of miR-451 (contains the inactivated miR144 sequence)	2055 - 2251
Engineered miRNA targeting human huntingtin coding sequence, incorporated in the cellular pri-mir-451 backbone	2252 - 2323
Flanking region of miR451	2324 - 2525
Cloning region	2526 - 2554
Human growth hormone polyA	2555 - 2651
Cloning region	2652 - 2699
AAV ITR - Right AAV-2 inverted terminal repeat	2700 - 2844

Full Genetic Sequence

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GGGGCCACTC  CCTCTCTGCG  CGCTCGCTCG  CTCACTGAGG  CCGCCCGGGC        50
AAAGCCCGGG  CGTCGGGCGA  CCTTTGGTCG  CCCGGCCTCA  GTGAGCGAGC       100
GAGCGCGCAG  AGAGGGAGTG  GCCAACTCCA  TCACTAGGGG  TTCCTCAGAT       150
CCGGTGCGGG  CCTCTTCGCT  ATTACGCCAG  CTGGCGAAAG  GGGGATGTGC       200
TGCAAGGCGA  TTAAGTTGGG  TAACGCCAGG  GTTTTCCCAG  TCACGACGTT       250
GTAAAACGAC  GGCCAGTGAA  TTCGCCCTTA  ATTCGGTACC  TAGTTATTAA       300
TAGTAATCAA  TTACGGGGTC  ATTAGTTCAT  AGCCCATATA  TGGAGTTCCG       350
CGTTACATAA  CTTACGGTAA  ATGGCCCGCC  TGGCTGACCG  CCCAACGACC       400
CCCGCCCATT  GACGTCAATA  ATGACGTATG  TTCCCATAGT  AACGCCAATA       450
GGGACTTTCC  ATTGACGTCA  ATGGGTGGAG  TATTTACGGT  AAACTGCCCA       500
CTTGGCAGTA  CATCAAGTGT  ATCATATGCC  AAGTACGCCC  CCTATTGACG       550
TCAATGACGG  TAAATGGCCC  GCCTGGCATT  ATGCCCAGTA  CATGACCTTA       600
TGGGACTTTC  CTACTTGGCA  GTACATCTAC  GTATTAGTCA  TCGCTATTAC       650
CATGGTCGAG  GTGAGCCCCA  CGTTCTGCTT  CACTCTCCCC  ATCTCCCCCC       700
CCTCCCCACC  CCCAATTTTG  TATTTATTTA  TTTTTTAATT  ATTTTGTGCA       750
GCGATGGGGG  CGGGGGGGGG  GGGGGGGCGC  GCGCCAGGCG  GGGCGGGGCG       800
GGGCGAGGGG  CGGGGCGGGG  CGAGGCGGAG  AGGTGCGGCG  GCAGCCAATC       850
AGAGCGGCGC  GCTCCGAAAG  TTTCCTTTTA  TGGCGAGGCG  GCGGCGGCGG       900
CGGCCCTATA  AAAAGCGAAG  CGCGCGGCGG  GCGGGAGTCG  CTGCGCGCTG       950
CCTTCGCCCC  GTGCCCCGCT  CCGCCGCCGC  CTCGCGCCGC  CCGCCCCGGC      1000
TCTGACTGAC  CGCGTTACTC  CCACAGGTGA  GCGGGCGGGA  CGGCCCTTCT      1050
CCTCCGGGCT  GTAATTAGCG  CTTGGTTTAA  TGACGGCTTG  TTTCTTTTCT      1100
GTGGCTGCGT  GAAAGCCTTG  AGGGGCTCCG  GGAGGGCCCT  TTGTGCGGGG      1150
GGAGCGGCTC  GGGGGGTGCG  TGCGTGTGTG  TGTGCGTGGG  GAGCGCCGCG      1200
TGCGGCTCCG  CGCTGCCCGG  CGGCTGTGAG  CGCTGCGGGC  GCGGCGCGGG      1250
GCTTTGTGCG  CTCCGCAGTG  TGCGCGAGGG  GAGCGCGGCC  GGGGGCGGTG      1300
CCCCGCGGTG  CGGGGGGGGC  TGCGAGGGGA  ACAAAGGCTG  CGTGCGGGGT      1350
GTGTGCGTGG  GGGGGTGAGC  AGGGGGTGTG  GGCGCGTCGG  TCGGGCTGCA      1400
ACCCCCCCTG  CACCCCCCTC  CCCGAGTTGC  TGAGCACGGC  CCGGCTTCGG      1450
GTGCGGGGCT  CCGTACGGGG  CGTGGCGCGG  GGCTCGCCGT  GCCGGGCGGG      1500
GGGTGGCGGC  AGGTGGGGGT  GCCGGGCGGG  GCGGGGCCGC  CTCGGGCCGG      1550
GGAGGGCTCG  GGGGAGGGGC  GCGGCGGCCC  CCGGAGCGCC  GGCGGCTGTC      1600
GAGGCGCGGC  GAGCCGCAGC  CATTGCCTTT  TATGGTAATC  GTGCGAGAGG      1650
GCGCAGGGAC  TTCCTTTGTC  CCAAATCTGT  GCGGAGCCGA  AATCTGGGAG      1700
GCGCCGCCGC  ACCCCCTCTA  GCGGGCGCGG  GGCGAAGCGG  TGCGGCGCCG      1750
GCAGGAAGGA  AATGGGCGGG  GAGGGCCTTC  GTGCGTCGCC  GCGCCGCCGT      1800
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CCCCTTCTCC  CTCTCCAGCC  TCGGGGCTGT  CCGCGGGGGG  ACGGCTGCCT        1850
TCGGGGGGGA  CGGGGCAGGG  CGGGGTTCGG  CTTCTGGCGT  GTGACCGGCG        1900
GCTCTAGAGC  CTCTGCTAAC  CATGTTCATG  CCTTCTTCTT  TTTCCTACAG        1950
CTCCTGGGCA  ACGTGCTGGT  TATTGTGCTG  TCTCATCATT  TTGGCAAAGA        2000
ATTAAGGGCG  AATTCGAGCT  CGGTACCTCG  CGAATGCATC  TAGATATCGG        2050
CGCTATGCTT  CCTGTGCCCC  CAGTGGGGCC  CTGGCTGGGA  TTTCATCATA        2100
TACTGTAAGT  TTGCGATGAG  ACACTACAGT  ATAGATGATG  TACTAGTCCG        2150
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GCCCAAGAAG  CTCTCTGCTC  AGCCTGTCAC  AACCTACTGA  CTGCCAGGGC        2250
ACTTGGGAAT  GGCAAGGAAG  GACTTGAGGG  ACTCGAAGAC  GAGTCCCTCA        2300
AGTCCTCTCT  TGCTATACCC  AGAAAACGTG  CCAGGAAGAG  AACTCAGGAC        2350
CCTGAAGCAG  ACTACTGGAA  GGGAGACTCC  AGCTCAAACA  AGGCAGGGGT        2400
GGGGGCGTGG  GATTGGGGGT  AGGGGAGGGA  ATAGATACAT  TTTCTCTTTC        2450
CTGTTGTAAA  GAAATAAAGA  TAAGCCAGGC  ACAGTGGCTC  ACGCCTGTAA        2500
TCCCACCACT  TTCAGAGGCC  AAGGCGCTGG  ATCCAGATCT  CGAGCGGCCG        2550
CCCGTGGCAT  CCCTGTGACC  CCTCCCCAGT  GCCTCTCCTG  GCCCTGGAAG        2600
TTGCCACTCC  AGTGCCCACC  AGCCTTGTCC  TAATAAAATT  AAGTTGCATC        2650
AAGATCGACG  GGCCCGTCGA  CTGCAGAGGC  CTGCATGCAA  GCTGATCTGA        2700
GGAACCCCTA  GTGATGGAGT  TGGCCACTCC  CTCTCTGCGC  GCTCGCTCGC        2750
TCACTGAGGC  CGGGCGACCA  AAGGTCGCCC  GACGCCCGGG  CTTTGCCCGG        2800
GCGGCCTCAG  TGAGCGAGCG  AGCGCGCAGA  GAGGGAGTGG  CCCC             2844
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TRADEMARKS	None
SPONSOR	uniQure Biopharma B.V.
DESIGNATION CODES	AMT-130, AAV5-miHTT, rAAV5-miHTT, AAV5(765)-miHTT
CAS REGISTRY NUMBER	2407582-19-6
UNII	VB2ZC1P6P4
WHO NUMBER	11711

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