STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (LM-146)	ZOCAGLUSAGENE NUZAPARVOVEC
PRONUNCIATION	zoe'' kah gloo’ sah jeen noo zah par’ voe vek
THERAPEUTIC CLAIM	Treatment of Pompe disease

CHEMICAL NAME

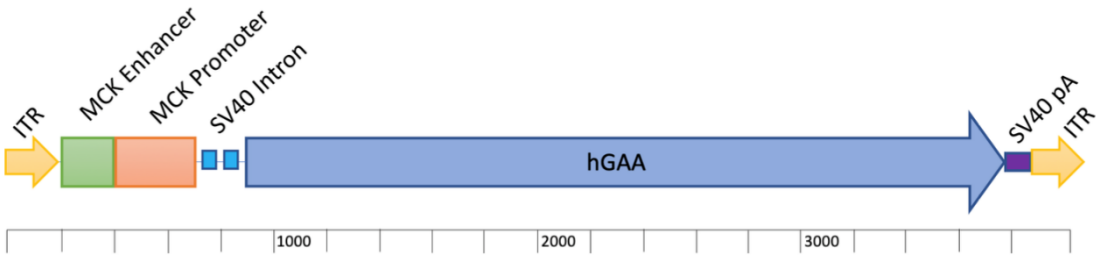
DNA (recombinant adeno-associated virus vector AT845 mouse muscle-type creatine kinase enhancer plus mouse muscle-type creatine kinase promoter plus human lysosomal α-glucosidase precursor codon-optimized transgene plus simian virus 40 polyadenylation signal plus adeno-associated virus 2 inverted terminal repeat flanks-specifying)

DESCRIPTION

recombinant, non-replicating adeno-associated virus serotype 8 (rAAV8) vector encoding codon-optimised human acid alpha-glucosidase (GAA), also called lysosomal α-glucosidase or acid maltase, under control of a mouse muscle creatine kinase (MCK) enhancer/promoter followed by a simian virus 40 (SV40) intron, terminated by the SV40 late polyadenylation sequence, and flanked by AAV2 inverted terminal repeats (ITRs). (INN proposed List #127)

STRUCTURE

AT845 schematic map of vector with inserted hGAA gene:



Scale is approximate; ITR = AAV inverted terminal repeat; MCK enhancer = murine MCK enhancer element; MCK promoter = murine MCK promoter; hGAA = human acid alpha-glucosidase cDNA; SV40 late poly A = SV40 late poly A signal sequence

Full nucleotide sequence (single strand) of AT845 (rAAV8-eMCK-hGAA) vector genome(bp): 3994

TTGGCCACTC	CCTCTCTGCG	CGCTCGCTCG	CTCACTGAGG	CCGGGCGACC	50
AAAGGTCGCC	CGACGCCCGG	GCTTTGCCCG	GGCGGCCTCA	GTGAGCGAGC	100
GAGCGCGCAG	AGAGGGAGTG	GCCAACTCCA	TCACTAGGGG	TTCCTCCTAG	150
CACGCGTCCA	CTACGGGTCT	AGGCTGCCCA	TGTAAGGAGG	CAAGGCCTGG	200
GGACACCCGA	GATGCCTGGT	TATAATTAAC	CCAGACATGT	GGCTGCCCCC	250
CCCCCCCCAA	CACCTGCTGC	CTGAGCCTCA	CCCCCACCCC	GGTGCCTGGG	300
TCTTAGGCTC	TGTACACCAT	GGAGGAGAAG	CTCGCTCTAA	AAATAACCCT	350
GTCCCTGGTG	GATCCCCTGC	ATGCCCAATC	AAGGCTGTGG	GGGACTGAGG	400
GCAGGCTGTA	ACAGGCTTGG	GGGCCAGGGC	TTATACGTGC	CTGGGACTCC	450
CAAAGTATTA	CTGTTCCATG	TTCCCGGCGA	AGGGCCAGCT	GTCCCCCGCC	500
AGCTAGACTC	AGCACTTAGT	TTAGGAACCA	GTGAGCAAGT	CAGCCCTTGG	550

GGCAGCCCAT	ACAAGGCCAT	GGGGCTGGGC	AAGCTGCACG	CCTGGGTCCG	600
GGGTGGGCAC	GGTGCCCGGG	CAACGAGCTG	AAAGCTCATC	TGCTCTCAGG	650
GGCCCCTCCC	TGGGGACAGC	CCCTCCTGGC	TAGTCACACC	CTGTAGGCTC	700
CTCTATATAA	CCCAGGGGCA	CAGGGGCTGC	CCCCGGGTCA	CCTCTAAGGT	750
AAATATAAAA	TTTTTAAGTG	TATAATGTGT	TAAACTACTG	ATTCTAATTG	800
TTTCTCTCTT	TTAGATTCCA	ACCTTTAGAA	CTGACCACCA	TGGGGGTGAG	850
GCACCCCCCC	TGCAGCCACA	GGCTGCTGGC	TGTGTGTGCC	CTGGTCAGCC	900
TGGCCACTGC	TGCCCTGCTG	GGCCACATCC	TGCTGCATGA	CTTCCTGCTG	950
GTGCCCAGAG	AGCTGTCTGG	CAGCAGCCCT	GTGCTGGAGG	AAACCCACCC	1000
TGCCCACCAG	CAGGGGGCCA	GCAGGCCTGG	CCCCAGGGAT	GCCCAGGCCC	1050
ACCCTGGCAG	GCCCAGGGCT	GTGCCCACCC	AGTGTGATGT	GCCCCCCAAC	1100
AGCAGGTTTG	ACTGTGCCCC	TGACAAGGCC	ATCACCCAGG	AGCAGTGTGA	1150
GGCCAGGGGC	TGCTGCTACA	TCCCTGCCAA	GCAGGGCCTG	CAGGGGGCCC	1200
AGATGGGCCA	GCCCTGGTGC	TTCTTCCCCC	CCTCATACCC	CTCCTACAAG	1250
CTGGAGAACC	TGAGCAGCTC	TGAGATGGGC	TACACTGCCA	CCCTGACCAG	1300
GACCACCCCG	ACGTTCTTCC	CCAAGGACAT	CCTGACCCTG	AGGCTGGATG	1350
TGATGATGGA	GACTGAGAAC	AGGCTGCACT	TCACCATCAA	GGACCCTGCC	1400
AACAGGAGAT	ATGAGGTGCC	CCTGGAAACC	CCCCATGTGC	ACAGCAGGGC	1450
CCCCAGCCCC	CTGTACTCTG	TGGAGTTCTC	TGAGGAGCCC	TTTGGGGTGA	1500
TTGTGAGGAG	GCAGCTGGAT	GGCAGGGTGC	TGCTGAACAC	CACTGTGGCC	1550
CCCCTGTTCT	TTGCTGACCA	GTTCCTGCAG	CTGAGCACCA	GCCTGCCCAG	1600
CCAGTACATC	ACTGGCCTGG	CTGAGCACCT	GAGCCCCCTG	ATGCTGAGCA	1650
CCAGCTGGAC	CAGGATCACC	CTGTGGAACA	GGGACCTGGC	CCCCACCCCT	1700
GGGGCCAACC	TGTATGGCAG	CCACCCCTTC	TACCTGGCCC	TGGAGGATGG	1750
GGGCTCTGCC	CATGGGGTGT	TCCTGCTGAA	CAGCAATGCC	ATGGATGTGG	1800
TGCTGCAGCC	CAGCCCTGCC	CTGAGCTGGA	GGAGCACTGG	GGGCATCCTG	1850
GATGTGTACA	TCTTCCTGGG	CCCTGAGCCC	AAGTCTGTGG	TGCAGCAGTA	1900
CCTGGATGTG	GTGGGCTACC	CCTTCATGCC	CCCCTACTGG	GGCCTGGGCT	1950
TCCACCTGTG	CAGATGGGGC	TACAGCAGCA	CTGCCATCAC	CAGGCAGGTG	2000
GTGGAGAACA	TGACCAGGGC	CCACTTCCCC	CTGGATGTGC	AGTGGAATGA	2050
CCTGGACTAC	ATGGACAGCA	GGAGGGACTT	CACCTTCAAC	AAGGATGGCT	2100
TCAGGGACTT	CCCTGCCATG	GTGCAGGAGC	TGCACCAGGG	GGGCAGGAGA	2150
TACATGATGA	TTGTGGACCC	TGCCATCAGC	AGCTCTGGCC	CTGCTGGCAG	2200
CTACAGGCCC	TATGATGAGG	GCCTGAGGAG	GGGGGTGTTC	ATCACCAATG	2250
AGACTGGCCA	GCCCCTGATT	GGCAAGGTCT	GGCCTGGCAG	CACTGCCTTC	2300
CCTGACTTCA	CCAACCCCAC	TGCCCTGGCC	TGGTGGGAGG	ACATGGTGGC	2350
TGAGTTCCAT	GACCAGGTGC	CCTTTGATGG	CATGTGGATT	GACATGAATG	2400
AGCCCAGCAA	CTTCATCAGG	GGCTCTGAGG	ATGGCTGCCC	CAACAATGAG	2450
CTGGAGAACC	CCCCCTATGT	GCCTGGGGTG	GTGGGGGGCA	CCCTGCAGGC	2500
TGCCACCATC	TGTGCCAGCA	GCCACCAGTT	CCTGAGCACC	CACTACAACC	2550
TGCACAACCT	GTATGGCCTG	ACTGAGGCCA	TTGCCAGCCA	CAGGGCCCTG	2600
GTGAAGGCCA	GGGGCACCAG	GCCCTTTGTG	ATCAGCAGGA	GCACCTTTGC	2650
TGGCCATGGC	AGATATGCTG	GCCACTGGAC	TGGGGATGTG	TGGAGCAGCT	2700
GGGAGCAGCT	GGCCAGCTCT	GTGCCTGAGA	TCCTGCAGTT	CAACCTGCTG	2750
GGGGTGCCCC	TGGTGGGGGC	TGATGTGTGT	GGCTTCCTGG	GCAACACCTC	2800
TGAGGAGCTG	TGTGTGAGGT	GGACCCAGCT	GGGGGCCTTC	TACCCCTTCA	2850
TGAGGAACCA	CAACAGCCTG	CTGAGCCTGC	CCCAGGAGCC	CTACAGCTTC	2900
TCTGAGCCTG	CCCAGCAGGC	CATGAGGAAG	GCCCTGACCC	TGAGATATGC	2950
CCTGCTGCCC	CACCTGTACA	CCCTGTTCCA	CCAGGCCCAT	GTGGCTGGGG	3000
AGACTGTGGC	CAGGCCCCTG	TTCCTGGAGT	TCCCCAAGGA	CAGCAGCACC	3050
TGGACTGTGG	ACCACCAGCT	GCTGTGGGGG	GAGGCCCTGC	TGATCACCCC	3100
TGTGCTGCAA	GCTGGCAAGG	CTGAGGTGAC	TGGCTACTTC	CCCCTGGGCA	3150
CTTGGTATGA	CCTGCAGACT	GTGCCTGTGG	AGGCCCTGGG	CAGCCTGCCC	3200
CCCCCCCCTG	CTGCCCCACG	GGAGCCTGCC	ATCCACTCTG	AGGGCCAGTG	3250
GGTGACCCTG	CCTGCCCCCC	TGGACACCAT	CAATGTGCAC	CTGAGGGCTG	3300
GCTACATCAT	CCCCCTGCAA	GGCCCTGGCC	TGACCACCAC	TGAGAGCAGG	3350
CAGCAGCCCA	TGGCCCTGGC	TGTGGCCCTG	ACCAAGGGGG	GGGAGGCCAG	3400
GGGGGAGCTG	TTCTGGGATG	ATGGGGAGAG	CCTGGAGGTG	CTGGAGAGGG	3450
GGGCCTACAC	CCAGGTGATC	TTCCTGGCCA	GGAACAACAC	CATTGTGAAT	3500

GAGCTGGTGA	GGGTGACCTC	TGAGGGGGCT	GGCCTGCAGC	TGCAGAAGGT	3550
CACTGTGCTG	GGGGTGGCCA	CTGCCCCCCA	GCAGGTGCTG	AGCAATGGGG	3600
TGCCTGTGAG	CAACTTCACC	TACAGCCCTG	ACACCAAGGT	GCTGGACATC	3650
TGTGTGAGCC	TGCTGATGGG	GGAGCAGTTC	CTGGTCAGCT	GGTGCTGATA	3700
TCGCTTTATT	TGTGAAATTT	GTGATGCTAT	TGCTTTATTT	GTAACCATTA	3750
TAAGCTGCAA	TAAACAAGTT	AACAACAACA	ATTGCATTCA	TTTTATGTTT	3800
CAGGTTCAGG	GGGAGGTGTG	GGAGGTTTTT	TAAAGGCGGC	CGCTCTAGGA	3850
GGAACCCCTA	GTGATGGAGT	TGGCCACTCC	CTCTCTGCGC	GCTCGCTCGC	3900
TCACTGAGGC	CGCCCGGGCA	AAGCCCGGGC	GTCGGGCGAC	CTTTGGTCGC	3950
CCGGCCTCAG	TGAGCGAGCG	AGCGCGCAGA	GAGGGAGTGG	CCAA	3994

MOLECULAR FORMULA $C_{38693}H_{48708}N_{15145}O_{24002}P_{3995}$
(single-strand of full vector determined with mono-phosphate on both 5' and 3' ends)

MOLECULAR WEIGHT 1233.7 KDa (determined for full vector sequence (single-chain) as defined in above table

TRADEMARKS None

SPONSOR Astellas Gene Therapies, Inc.

DESIGNATION CODE AT845

CAS REGISTRY NUMBER 2601264-52-0

UNII FDB4GQ8A3Y

WHO NUMBER 12248

SCS