STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (NO-78)	AVIGBAGENE PARVEC
PRONOUNCIATION	a vig' bah gene par' vek
THERAPEUTIC CLAIM	Treatment of Gaucher disease

DESCRIPTION

FLT201 comprises two components: the S3 (AAVS3) viral capsid into which is packaged the single stranded 3046 nucleotide FRE76-GBAco variant 85-BgpA genome.

AAV2/S3-HCR-hAAT-GBAco variant 85. AAV2/S3 describes the capsid and HCR-hAAT-GBAco variant 85 describes the DNA genome.

Capsomere, recombinant adeno-associated virus 8/adeno-associated virus 3B viral capsid VP1, complex with adeno-associated virus 3B viral capsid protein VP2, complex with adeno-associated virus 3B viral capsid protein VP3 decamer, hexacontamer, complex with DNA (recombinant adeno-associated virus 2 derived vector human apolipoprotein E gene hepatic control locus region plus human trypsin-inhibiting protein serpin A1 gene promoter plus simian virus 40 intron plus human glucosylceramidase [351-cysteine, 380-cysteine] codon-optimized gene plus bovine somatotropin gene polyadenylation signal plus adeno-associated virus 2 inverted terminal repeat flanks) (1:1). The drug substance is an adeno-associated virus composed of 2 elements: a single-stranded DNA vector genome which is packaged in an adeno-associated virus-derived capsid.

SCHEMATIC and SEQUENCE

Schematic of FLT201 vector



ITR=AAV2 inverse terminal repeat; FRE76=HCR-hAAT liver-specific promoter; SV40i=SV40 intron; GBAco-85=codon-optimized beta-glucocerebrosidase engineered for increased stability (variant 85); bGHpA=bovine growth hormone polyadenylation signal

Nucleotide position:	feature
1 to 154:	AAV2 inverse terminal repeat
178 to 921:	HCR-hAAT promoter (FRE76)
935 to 1000:	SV40 intron
1038 to 2648:	GBA-85 coding sequence
2662 to 2869:	Bovine growth hormone polyA
2890 to 3046:	AAV2 inverse terminal repeat

For Amino Acid Sequence of GBAco Variant 85 and Sequence Alignment of GBA Wildtype and GBAco Variant 85, please see section “Sequence or structural information from firm” in this review below.

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TTGGCCACTC CCTCTCTGCG CGCTCGCTCG CTCACTGAGG CCGGGCGACC      50
AAAGGTCGCC CGACGCCCGG GCTTTGCCCG GGCGGCCTCA GTGAGCGAGC     100
GAGCGCGCAG AGAGGGAGTG GCCAACTCCA TCACTAGGGG TTCCTGGAGG     150
GGTGTTAATT AAAAGCTTGA TATCAGGCTC AGAGGCACAC AGGAGTTTCT     200
GGGCTCACCC TGCCCCCTTC CAACCCCTCA GTTCCCATCC TCCAGCAGCT     250
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GTTTGTGTGC TGCCTCTGAA GTCCACACTG AACAAACTTC AGCCTACTCA 300
TGTCCCTAAA ATGGGCAAAC ATTGCAAGCA GCAAACAGCA AACACACAGC 350
CCTCCCTGCC TGCTGACCTT GGAGCTGGGG CAGAGGTCAG AGACCTCTCT 400
GGGCCCATGC CACCTCCAAC ATCCACTCGA CCCCTTGGAA TTTCGGTGGA 450
GAGGAGCAGA GGTTGTCCTG GCGTGGTTTA GGTAGTGTGA GAGGGGTACC 500
CGGGGATCTT GCTACCAGTG GAACAGCCAC TAAGGATTCT GCAGTGAGAG 550
CAGAGGGCCA GCTAAGTGGT ACTCTCCCAG AGACTGTCTG ACTCACGCCA 600
CCCCCTCCAC CTTGGACACA GGACGCTGTG GTTTCTGAGC CAGGTACAAT 650
GACTCCTTTC GGTAAGTGCA GTGGAAGCTG TACACTGCCC AGGCAAAGCG 700
TCCGGGCAGC GTAGGCGGGC GACTCAGATC CCAGCCAGTG GACTTAGCCC 750
CTGTTTGCTC CTCCGATAAC TGGGGTGACC TTGGTTAATA TTCACCAGCA 800
GCCTCCCCCG TTGCCCCTCT GGATCCACTG CTTAAATACG GACGAGGACA 850
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TGATCCCCCT GATCTGCGGC CAATTCGCTA GCAGGTAAAT ATAAAATTTT 950
TAAGTGTATA ATGTGTTAAA CTACTGATTC TAATTGTTTC TCTCTTTTAG 1000
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GCTGGCAGCC TCACAGGATT GCTTCTACTT CAGGCAGTGT CGTGGGCATC 1150
AGGTGCCAGG CCCTGCATCC CTAAGAGCTT TGGCTACAGC TCTGTGGTGT 1200
GTGTGTGCAA TGCCACCTAC TGTGACAGCT TTGACCCCCC CACCTTTCCT 1250
GCCCTGGGCA CCTTCAGCAG ATATGAGAGC ACCAGGTCTG GGAGGAGGAT 1300
GGAGCTGAGC ATGGGGCCCA TCCAGGCTAA TCACACTGGC ACTGGCCTGC 1350
TGCTGACCCT GCAGCCTGAG CAGAAGTTCC AGAAAGTAAA GGGCTTTGGA 1400
GGGGCCATGA CTGATGCTGC TGCTCTGAAC ATCCTGGCCC TGAGCCCCCC 1450
TGCCCAGAAT CTGCTGCTGA AGAGCTACTT CTCTGAGGAG GGCATTGGCT 1500
ATAACATCAT CAGGGTGCCC ATGGCCAGCT GTGACTTCAG CATCAGGACC 1550
TACACCTATG CTGACACCCC TGATGATTTC CAGCTGCACA ACTTCAGCCT 1600
GCCTGAGGAG GATACCAAGC TGAAGATCCC ACTGATCCAC AGGGCTCTGC 1650
AGCTGGCCCA GAGGCCTGTG AGCCTGCTGG CCAGCCCCTG GACCAGCCCC 1700
ACTTGGCTGA AGACCAATGG GGCTGTGAAT GGGAAGGGGA GCCTGAAGGG 1750
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ATAGCAGGCA TGCTGGGGAG TTTAAACACT AGTGTCGACT CATTAACTAC 2900
AAGGAACCCC TAGTGATGGA GTTGGCCACT CCCTCTCTGC GCGCTCGCTC 2950
GCTCACTGAG GCCGGGCGAC CAAAGGTCGC CCGACGCCCG GGCTTTGCCC 3000
GGGCGGCCTC AGTGAGCGAG CGAGCGCGCA GAGAGGGAGT GGCCAA 3046

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