STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

USAN (OP-05)	PLENCIFGENE PARVEC
PRONOUNCIATION	plen sif‘ jeen par‘ vek
THERAPEUTIC CLAIM	Treatment of Cystic Fibrosis lung disease (CF)

DESCRIPTION

DNA (recombinant replication-defective adeno-associated virus vector 4D-710 cytomegalovirus promoter CMV173 plus human (1-707)-(760-1480)-cystic fibrosis transmembrane conductance regulator codon-optimized gene CFTRΔR plus synthetic polyadenylation signal plus adeno-associated virus 2 inverted terminal repeat flanks-specifying

SCHEMATIC

Genetic schematic map of transgene:

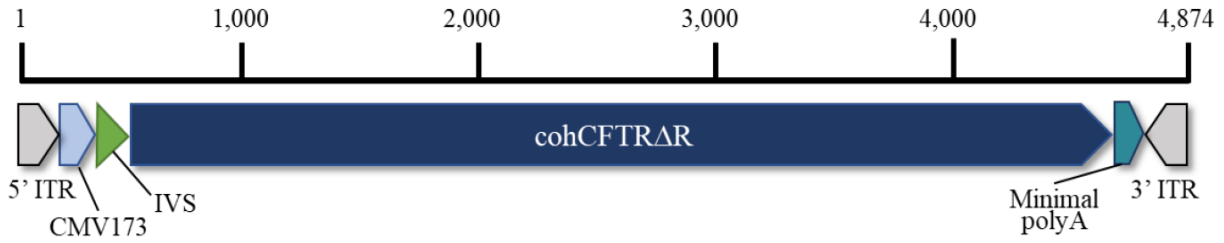

Table of features:

Annotation	**Description**	**Position**
5’ ITR	5’ Inverted Terminal Repeat from the AAV2 genome function as the origin of viral DNA replication and the packaging signal of the viral genome	1-145
CMV173 promoter	173-bp minimal cytomegalovirus (CMV173) promoter initiates the expression of the CFTRΔR transgene	154-326
Intervening Sequence (IVS)	51-bp intervening sequence between transcription start site and translational start site of the CMV173 promoter to aid promoter function	327-377
cohCFTRΔR	codon optimized human cystic fibrosis transmembrane conductance regulator (CFTR) transgene with the regulatory domain deleted; coding for the human CFTR protein upon expression into mRNA and translation into protein	378-4664
synthetic polyA	short synthetic consensus poly adenylation signal sequence is used to stabilize mRNA transcript and ensure proper processing and translation	4665-4713
3’ ITR	3’ Inverted Terminal Repeat from the AAV2 genome function as the origin of viral DNA replication and the packaging signal of the viral genome	4730-

		4874

SEQUENCE

Nucleotide sequence

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TTGGCCACTC CCTCTCTGCG CGCTCGCTCG CTCACTGAGG CCGGGCGACC 50
AAAGGTCGCC CGACGCCCGG GCTTTGCCCG GGCGGCCTCA GTGAGCGAGC 100
GAGCGCGCAG AGAGGGAGTG GCCAACTCCA TCACTAGGGG TTCCTGCGGC 150
CGCACTCACG GGGATTTCCA AGTCTCCACC CCATTGACGT CAATGGGAGT 200
TTGTTTTGGC ACCAAAATCA ACGGGACTTT CCAAAATGTC GTAATAACCC 250
CGCCCCGTTG ACGCAAATGG GCGGTAGGCG TGTACGGTGG GAGGTCTATA 300
TAAGCAGAGC TCGTTTAGTG AACCGTCAGA ATTCTCGAGT GATCGAAAGA 350
GCCTGCTAAA GCAAAAAAGA AGTCACCATG CAGCGCAGCC CACTGGAGAA 400
GGCAAGCGTG GTGTCCAAGC TGTTCTTTTC CTGGACCAGG CCTATCCTGA 450
GGAAGGGATA CAGGCAGCGG CTGGAGCTGA GCGACATCTA TCAGATCCCT 500
TCTGTGGACA GCGCCGATAA TCTGTCCGAG AAGCTGGAGA GAGAGTGGGA 550
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TRADEMARKS None

SPONSOR 4D Molecular Therapeutics, Inc.

DESIGNATION CODES	4D-710; 4D-A101.CMV173-CFTRΔR
CAS REGISTRY NUMBER	2737363-32-3
UNII	9SG6QLJ7VJ
WHO NUMBER	13321

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