STATEMENT ON A NONPROPRIETARY NAME ADOPTED BY THE USAN COUNCIL

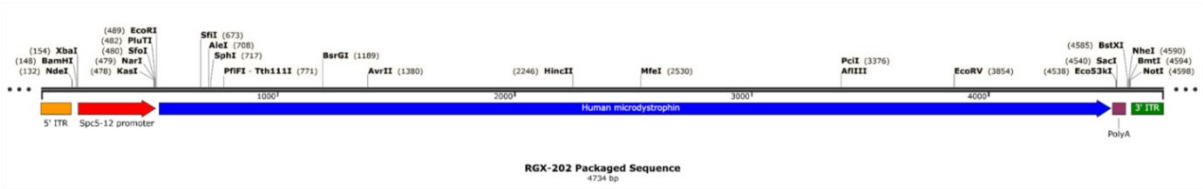
| | |
|---|---|
| USAN (OP-110) | PIVODISGENE PARVEC |
| PRONOUNCIATION | piv" oh dis' jeen par' vek |
| THERAPEUTIC CLAIM | Treatment of Duchenne muscular dystrophy (DMD) |

DESCRIPTION

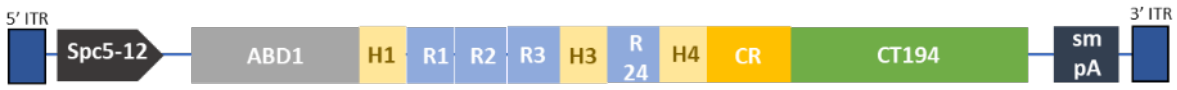
DNA (recombinant replication-defective adeno-associated virus 8 vector RGX-202 synthetic promoter Spc5-12 plus human codon-optimized microdystrophin variant ABD1-H1-R1-R2-R3-H3-R24-H4-CR-CT194 plus synthetic polyadenylation signal plus adeno-associated virus 2 inverted terminal repeat flanks-specifying)

STRUCTURAL FORMULA

**Genetic Map of the entire product:**




**Schematic representation of the vector genome:**




**Linear DNA sequence (bp):**

Table with position of features:

| Feature | Location (bp) |
|---|---|
| 5' Inverted Terminal Repeats (ITR) | 1 – 130 |
| Spc5-12 promoter | 160 - 487 |
| Human microdystrophin: | 501 – 4517: |
| ABD1 | 501 – 1220 |
| H1 | 1257 - 1481 |
| R1 | 1509 - 1841 |
| R2 | 1842 - 2168 |
| R3 | 2175 - 2501 |
| H3 | 2502 - 2642 |
| R24 | 2652 - 2969 |
| H4 | 2970 - 3185 |

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|---|---|
| CR | 3186 - 3929 |
| CT194 | 3930 - 4511 |
| Polyadenylation signal | 4524 - 4577 |
| 3’ ITR | 4605 - 4734 |

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CTGCGCGCTC GCTCGCTCAC TGAGGCCGCC CGGGCAAAGC CCGGGCGTCG        50
GGCGACCTTT GGTCGCCCGG CCTCAGTGAG CGAGCGAGCG CGCAGAGAGG       100
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TCCTCTAGAG GCCGTCCGCC CTCGGCACCA TCCTCACGAC ACCCAAATAT       200
GGCGACGGGT GAGGAATGGT GGGGAGTTAT TTTTAGAGCG GTGAGGAAGG       250
TGGGCAGGCA GCAGGTGTTG GCGCTCTAAA AATAACTCCC GGGAGTTATT       300
TTTAGAGCGG AGGAATGGTG GACACCCAAA TATGGCGACG GTTCCTCACC       350
CGTCGCCATA TTTGGGTGTC CGCCCTCGGC CGGGGCCGCA TTCCTGGGGG       400
CCGGGCGGTG CTCCCGCCCG CCTCGATAAA AGGCTCCGGG GCCGGCGGCG       450
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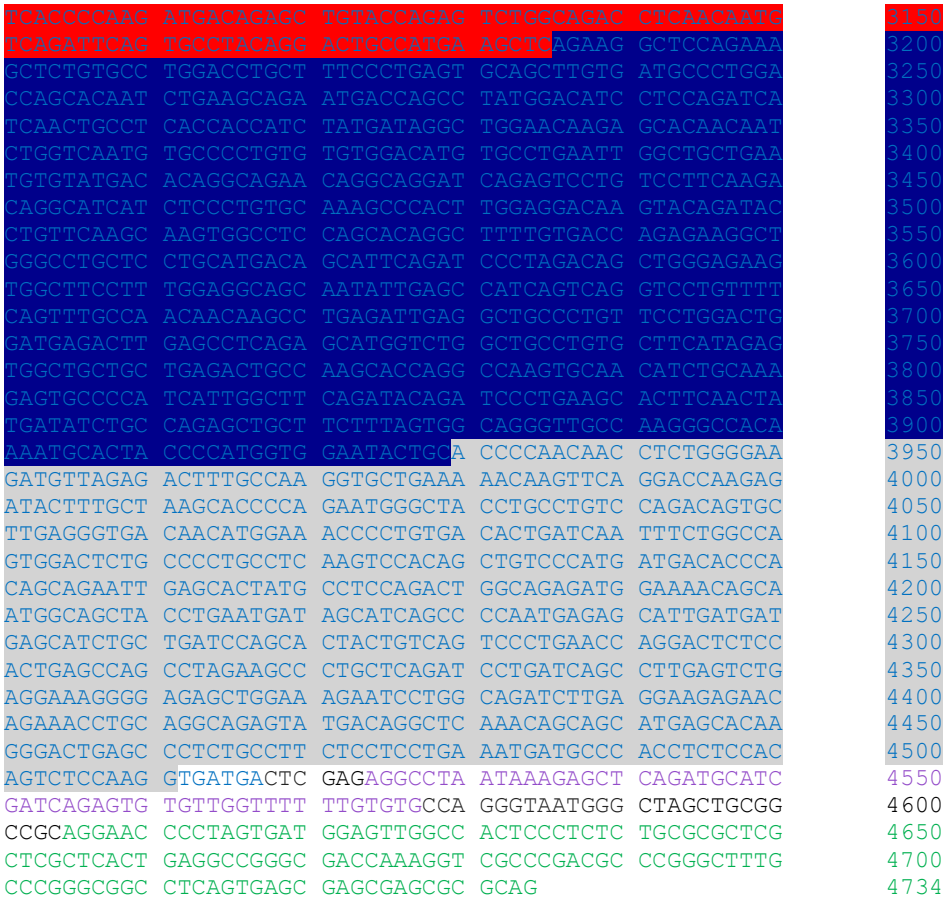


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|---|---|
| TRADEMARKS | None |
| SPONSOR | REGENXBIO Inc. |
| DESIGNATION CODE | RGX-202 |
| CAS REGISTRY NUMBER | 2648631-52-9 |
| UNII | NVQ25YNH8P |
| WHO NUMBER | 13471 |

scs