

FIG. 1**scAAV- MHCK7-CSR3 – 2,430 bp**

ttggccactccctctctgcgcgctcgctcgctcactgaggccgcccgggcaaagcccgggcgtcg
 ggcgacctttggtcgcccggcctcagtgagcgagcgagcgcgagagagggagtgccaactcc
 atcactaggggttcctACCCTTCAGATTAATAAATACTGAGGTAAGGGCCTGGG
 TAGGGGAGGTGGTGTGAGACGCTCCTGTCTCTCCTCTATCTGCCATCGG
 CCCTTTGGGAGGAGGAATGTGCCAAGGACTAAAAAAGGCCATGGAG
 CCAGAGGGGGCAGGGCAACAGACCTTTCATGGGCAAACCTTGGGGCCCT
 GCTGTCTAGCATGCCCCACTACGGGTCTAGGCTGCCCATGTAAGGAGGCA
 AGGCCTGGGGACACCCGAGATGCCTGGTTATAATTAACCCAGACATGTG
 GCTGCCCCCCCCCCCCAACACCTGCTGCCTCTAAAAATAACCTGTCCCT
 GGTGGATCCCCTGCATGCGAAGATCTTCGAACAAGGCTGTGGGGGACTG
 AGGGCAGGCTGTAACAGGCTTGGGGGCCAGGGCTTATACGTGCCTGGGA
 CTCCCAAAGTATTACTGTTCCATGTTCCCGGCGAAGGGCCAGCTGTCCCC
 GCCAGCTAGACTCAGCACTTAGTTTAGGAACCAAGTGAGCAAGTCAGCCCT
 TGGGGCAGCCCATAACAAGGCCATGGGGCTGGGCAAGCTGCACGCCTGG
 GTCCGGGGTGGGCACGGTGCCCGGGCAACGAGCTGAAAGCTCATCTGCT
 CTCAGGGGGCCCTCCCTGGGGACAGCCCTCCTGGCTAGTCACACCCTGT
 AGGCTCCTCTATATAACCCAGGGGCACAGGGGCTGCCCTCATTCTACCAC
 CACCTCCACAGCACAGACAGACACTCAGGAGCCAGCCAGGCCACCATGCC
 AACTGGGGCGGAGGCGCAAAATGTGGAGCCTGTGAAAAGACCGTCTAC
 CATGCAGAAGAAATCCAGTGCAATGGAAGGAGTTTCCACAAGACGTGTTT
 CCACTGCATGGCCTGCAGGAAGGCTCTTGACAGCACGACAGTCGCGGCT
 CATGAGTCGGAGATCTACTGCAAGGTGTGCTATGGGCGCAGATATGGCC
 CCAAAGGGATCGGGTATGGACAAGGCGCTGGCTGTCTCAGCACAGACAC
 GGGCGAGCATCTCGGCCTGCAGTTCCAACAGTCCCCAAAGCCGGCACGCT
 CAGTTACCACCAGCAACCCTTCCAAATCACTGCGAAGTTTGGAGAGTCC
 GAGAAGTGCCCTCGATGTGGCAAGTCAGTCTATGCTGCTGAGAAGGTTAT
 GGGAGGTGGCAAGCCTTGGCACAAGACCTGTTCCGCTGTGCCATCTGTG

GGAAGAGTCTGGAGTCCACAAATGTCACTGACAAAGATGGGGAACTTTA
TTGCAAAGTTTGCTATGCCAAAAATTTTGGCCCCACGGGTATTGGGTTTG
GAGGCCTTACACAACAAGTGGAAAAGAAAGAATGATCAACCTCTGGATT
ACAAAATTTGTGAAAGATTGACTGGTATTCTTAACTATGTTGCTCCTTTTA
CGCTATGTGGATACGCTGCTTTAATGCCTTTGTATCATGCTATTGCTTCCC
GTATGGCTTTCATTTTCTCCTCCTTGTATAAATCCTGGTTGCTGTCTCTTTAT
GAGGAGTTGTGGCCCGTTGTCAGGCAACGTGGCGTGGTGTGCACTGTGT
TTGCTGACGCAACCCCCACTGGTTGGGGCATTGCCACCACCTGTCAGCTC
CTTTCGGGACTTTCGCTTTCCTCCTTATTGCCACGGCGGAACTCATC
GCCGCCTGCCTTGCCGCTGCTGGACAGGGGCTCGGCTGTTGGGCACTG
ACAATTCCGTGGTGTGTCGGGGAAATCATCGTCCTTTCCTTGGCTGCTCG
CCTGTGTTGCCACCTGGATTCTGCGCGGGACGTCCTTCTGCTACGTCCCTT
CGGCCCTCAATCCAGCGGACCTTCCTTCCCGCGGCCTGCTGCCGGCTCTGC
GGCCTCTTCGCGTCTTCGCCTTCGCCCTCAGACGAGTCGGATCTCCCTTT
GGGCCGCCTCCCCGCACTGCCCCGGGTGGCATCCCTGTGACCCCTCCCCAG
TGCCTCTCCTGGCCCTGGAAGTTGCCACTCCAGTGCCCACCAGCCTTGTCC
TAATAAAATTAAGTTGCATCATTTTGTCTGACTAGGTGTCCTTCTATAATAT
TATGGGGTGGAGGGGGGTGGTATGGAGCAAGGGGCCCAAGTTGGGAA
GAAACCTGTAGGGCCTGCaggaaccctagtgatggagactccctctctgcgcgctcgt
cgctcactgaggccgggcgaccaaaggtcgcccgacgcccgggcttgcccgggcggcctcagt
gagcgagcgagcgcgagagaggagt

FIG. 1 (con't)

FIG. 2**scAAV-hTnT-CSR3 – 2,203 bp**

ttggccactccctctctgcgcgctcgctcgctcactgaggccgcccgggcaaagcccgggcgtcg
 ggcgacctttggtcgcccggcctcagtgagcgagcgagcgcgagagaggagtgccaaactcc
 atcactaggggttcctCTCAGTCCATTAGGAGCCAGTAGCCTGGAAGATGTCTT
 TACCCCCAGCATCAGTTCAAGTGGAGCAGCACATAACTCTTGCCCTCTGCC
 TTCCAAGATTCTGGTGCTGAGACTTATGGAGTGTCTTGGAGGTTGCCTTCT
 GCCCCCAACCCTGCTCCCAGCTGGCCCTCCCAGGCCTGGGTTGCTGGCC
 TCTGCTTTATCAGGATTCTCAAGAGGGACAGCTGGTTTATGTTGCATGACT
 GTTCCCTGCATATCTGCTCTGGTTTTAAATAGCTTATCTGAGCAGCTGGAG
 GACCACATGGGCTTATATGGCGTGGGGTACATGTTCTGTAGCCTTGTCC
 CTGGCACCTGCCAAAATAGCAGCCAACACCCCCACCCCCACCGCCATCCC
 CCTGCCCCACCCGTCCCCTGTCGCACATTCTCCCTCCGCAGGGCTGGCTC
 ACCAGGCCCCAGCCCACATGCCTGCTTAAAGCCCTCTCCATCCTCTGCCTC
 ACCAGTCCCCGCTGAGACTGAGCAGACGCCTCCAGGATCTGTCGGCAG
 GCCACCATGCCAAACTGGGGCGGAGGCGCAAAATGTGGAGCCTGTGAAA
 AGACCGTCTACCATGCAGAAGAAATCCAGTGCAATGGAAGGAGTTTCCAC
 AAGACGTGTTTCCACTGCATGGCCTGCAGGAAGGCTCTTGACAGCACGAC
 AGTCGCGGCTCATGAGTCGGAGATCTACTGCAAGGTGTGCTATGGGCGC
 AGATATGGCCCCAAAGGGATCGGGTATGGACAAGGCGCTGGCTGTCTCA
 GCACAGACACGGGCGAGCATCTCGGCCTGCAGTTCCAACAGTCCCCAAA
 GCCGGCACGCTCAGTTACCACCAGCAACCCTTCCAAATTCAGTGCGAAGTT
 TGGAGAGTCCGAGAAGTGCCCTCGATGTGGCAAGTCAGTCTATGCTGCT
 GAGAAGGTTATGGGAGGTGGCAAGCCTTGGCACAAGACCTGTTTCCGCT
 GTGCCATCTGTGGGAAGAGTCTGGAGTCCACAAATGTCACTGACAAAGAT
 GGGGAACCTTTATTGCAAAGTTTGCTATGCCAAAAATTTGGCCCCACGGG
 TATTGGGTTTGGAGGCCTTACACAACAAGTGGAAGAAAGAATGATCA
 ACCTCTGGATTACAAAATTTGTGAAAGATTGACTGGTATTCTTAACTATGT
 TGCTCCTTTTACGCTATGTGGATACGCTGCTTTAATGCCTTTGTATCATGCT

ATTGCTTCCCGTATGGCTTTCATTTTCTCCTCCTTGTATAAATCCTGGTTGC
TGTCTCTTTATGAGGAGTTGTGGCCCGTTGTCAGGCAACGTGGCGTGGTG
TGCACTGTGTTTGCTGACGCAACCCCCACTGGTTGGGGCATTGCCACCAC
CTGTCAGCTCCTTTCCGGGACTTTCGCTTTCCCCCTCCCTATTGCCACGGCG
GAACTCATCGCCGCCTGCCTTGCCCGCTGCTGGACAGGGGCTCGGCTGTT
GGGCACTGACAATTCCGTGGTGTGTCGGGGAAATCATCGTCCTTTCCTT
GGCTGCTCGCCTGTGTTGCCACCTGGATTCTGCGCGGGACGTCCTTCTGCT
ACGTCCCTTCGGCCCTCAATCCAGCGGACCTTCCTTCCCGCGGCCTGCTGC
CGGCTCTGCGGCCTCTTCCGCGTCTTCGCCTTCGCCCTCAGACGAGTCGGA
TCTCCCTTTGGGCCGCCTCCCCGCACTGCCCGGGTGGCATCCCTGTGACCC
CTCCCCAGTGCCTCTCCTGGCCCTGGAAGTTGCCACTCCAGTGCCCACCAG
CCTTGTCCTAATAAAATTAAGTTGCATCATTTTGTCTGACTAGGTGTCCTTC
TATAATATTATGGGGTGGAGGGGGGTGGTATGGAGCAAGGGGGCCCAAG
TTGGGAAGAAACCTGTAGGGCCTGCaggaaccctagtgatggagactccctctctg
cgcgctcgctcgctcactgaggccgggcgaccaaaggtcgcccgacgcccgggctttgcccggg
cggcctcagtgagcgagcgagcgcgagagaggagt

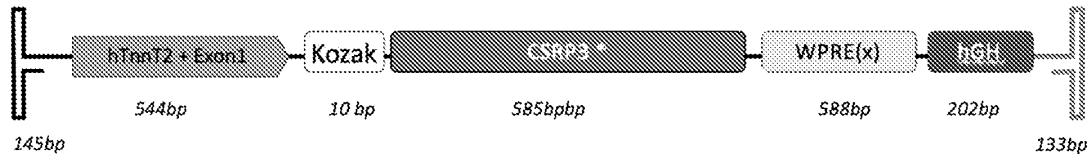
FIG. 2 (con't)

FIG. 3**scAAV- MHCK7-CSR3 – 2,430 bp – *codon optimized***

Ttggccactccctctctgcgcgctcgctcgctcactgaggccgcccgggcaaagcccgggctcg
ggcgaccttttggtcgcccggtcagtgagcgagcgagcgcgagagaggagtggtccaactcc
atcactaggggttcctACCCTTCAGATTAATAAATACTGAGGTAAGGGCCTGGG
TAGGGGAGGTGGTGTGAGACGCTCCTGTCTCTCCTCTATCTGCCATCGG
CCCTTTGGGAGGAGGAATGTGCCAAGGACTAAAAAAGGCCATGGAG
CCAGAGGGGGCAGGGCAACAGACCTTTCATGGGCAAACCTTGGGGCCCT
GCTGTCTAGCATGCCCCACTACGGGTCTAGGCTGCCCATGTAAGGAGGCA
AGGCCTGGGGACACCCGAGATGCCTGGTTATAATTAACCCAGACATGTG
GCTGCCCCCCCCCCCCAACACCTGCTGCCTCTAAAAATAACCCTGTCCCT
GGTGGATCCCCTGCATGCGAAGATCTTCGAACAAGGCTGTGGGGGACTG
AGGGCAGGCTGTAACAGGCTTGGGGGCCAGGGCTTATACGTGCCTGGGA
CTCCCAAAGTATTACTGTTCCATGTTCCCGGCGAAGGGCCAGCTGTCCCC
GCCAGCTAGACTCAGCACTTAGTTTAGGAACAGTGAGCAAGTCAGCCCT
TGGGGCAGCCCATAACAAGGCCATGGGGCTGGGCAAGCTGCACGCCTGG
GTCCGGGGTGGGCACGGTGCCCGGGCAACGAGCTGAAAGCTCATCTGCT
CTCAGGGGGCCCTCCCTGGGGACAGCCCTCCTGGCTAGTCACACCCTGT
AGGCTCCTCTATATAACCCAGGGGCACAGGGGCTGCCCTCATTCTACCAC
CACCTCCACAGCACAGACAGACACTCAGGAGCCAGCCAGGCCACCATGCC
CAATTGGGGTGGAGGAGCTAAATGTGGAGCTTGTGAAAAACAGTTTAT
CATGCTGAAGAAATTCAATGTAATGGAAGATCTTTTCATAAACATGTTTT
CATTGTATGGCTTGTAGAAAAGCACTTGATTCTACAAGTGTGCAGCACAT
GAAAGTGAAATCTATTGTAAAGTATGTTATGGAAGAAGATATGGACCA
AAGGAATTGGATATGGACAAGGAGCAGGATGTCTTTCTACAGATACTGG
AGAACATTTGGGATTGCAATTTCAACAAAGTCCTAAACCAGCTAGATCTG
TTACAACAAGTAATCCATCAAAATTTACTGCTAAATTTGGAGAATCCGAAA
AATGTCCTAGATGTGGAAAATCAGTATATGCTGCTGAAAAAGTTATGGGA
GGTGGAACCATGGCATAAGACATGTTTTAGATGTGCAATTTGTGGTAA

ATCTTTGGAATCTACAAATGTTACAGATAAAGATGGAGAATTGTATTGTA
AAGTTTGTATGCTAAAAATTTTGGACCTACAGGTATAGGATTTGGAGGT
TTGACACAACAAGTTGAAAAAAGAATGATCAACCTCTGGATTACAAAA
TTTGTGAAAGATTGACTGGTATTCTTAACCTATGTTGCTCCTTTTACGCTATG
TGGATACGCTGCTTTAATGCCTTTGTATCATGCTATTGCTTCCCGTATGGCT
TTCATTTTCTCCTCCTTGTATAAATCCTGGTTGCTGTCTCTTTATGAGGAGT
TGTGGCCCGTTGTGAGGCAACGTGGCGTGGTGTGCACTGTGTTTGCTGAC
GCAACCCCCACTGGTTGGGGCATTGCCACCACCTGTCAGCTCCTTTCCGG
GACTTTGCTTTCCCCCTCCCTATTGCCACGGCGGAACTCATCGCCGCCTG
CCTTGCCCGCTGCTGGACAGGGGCTCGGCTGTTGGGCACTGACAATTCCG
TGGTGTGTCGGGGAAATCATCGTCCTTTCTTGGCTGCTCGCCTGTGTTG
CCACCTGGATTCTGCGCGGGACGTCCTTCTGCTACGTCCCTTCGGCCCTCA
ATCCAGCGGACCTTCCTTCCCGCGGCCTGCTGCCGGCTCTGCGGCCTCTC
CGCGTCTTCGCTTCGCCCTCAGACGAGTCGGATCTCCCTTTGGGCCGCT
CCCCGCACTGCCCCGGGTGGCATCCCTGTGACCCCTCCCCAGTGCCTCTCCT
GGCCCTGGAAGTTGCCACTCCAGTGCCACCAAGCCTTGTCTAATAAAATT
AAGTTGCATCATTTTGTCTGACTAGGTGTCCTTCTATAATATTATGGGGTG
GAGGGGGGTGGTATGGAGCAAGGGGCCCAAGTTGGGAAGAAACCTGTA
GGGCCTGCaggaaccctagtgatggagactccctctctgcgcgctcgctcgctcactgaggc
cgggcgaccaaaggtcgcccgacgcccgggctttgccgggcggcctcagtgagcgagcgagc
gcgcagagagggagt

FIG. 3 (con't)

FIG. 4**scAAV-hTnT-CSR3 – 2,203 bp – Codon optimized**

ttggcactccctctctgcgcgctcgctcgctcactgaggccgcccgggcaaagcccgggctcg
 ggcgacctttggtcgcccgccctcagtgagcgagcgagcgcgagagaggagtgccaactcc
 atcactaggggttcctCTCAGTCCATTAGGAGCCAGTAGCCTGGAAGATGTCTT
 TACCCCCAGCATCAGTTCAAGTGGAGCAGCACATAACTCTTGCCCTCTGCC
 TTCCAAGATTCTGGTGCTGAGACTTATGGAGTGTCTTGGAGGTTGCCTTCT
 GCCCCCAACCCTGCTCCCAGCTGGCCCTCCCAGGCCTGGGTTGCTGGCC
 TCTGCTTTATCAGGATTCTCAAGAGGGACAGCTGGTTTATGTTGCATGACT
 GTTCCCTGCATATCTGCTCTGGTTTTAAATAGCTTATCTGAGCAGCTGGAG
 GACCACATGGGCTTATATGGCGTGGGGTACATGTTCTGTAGCCTTGTCC
 CTGGCACCTGCCAAAATAGCAGCCAACACCCCCACCCCCACCGCCATCCC
 CCTGCCCCACCCGTCCCCTGTCGCACATTCTCCCTCCGCAGGGCTGGCTC
 ACCAGGCCCCAGCCCACATGCCTGCTTAAAGCCCTCTCCATCCTCTGCCTC
 ACCAGTCCCCGCTGAGACTGAGCAGACGCCTCCAGGATCTGTCGGCAG
 GCCACCATGCCCAATTGGGGTGGAGGAGCTAAATGTGGAGCTTGTGAAA
 AACAGTTTATCATGCTGAAGAAATTCAATGTAATGGAAGATCTTTTCATA
 AACATGTTTTATTGTATGGCTTGTAGAAAAGCACTTGATTCTACAACCTG
 TTGCAGCACATGAAAGTGAAATCTATTGTAAAGTATGTTATGGAAGAAGA
 TATGGACCAAAAGGAATTGGATATGGACAAGGAGCAGGATGTCTTTCTA
 CAGATACTGGAGAACATTTGGGATTGCAATTTCAACAAAGTCCTAAACCA
 GCTAGATCTGTTACAACAAGTAATCCATCAAAATTTACTGCTAAATTTGGA
 GAATCCGAAAAATGTCCTAGATGTGGAAAATCAGTATATGCTGCTGAAAA
 AGTTATGGGAGGTGGAAAACCATGGCATAAGACATGTTTTAGATGTGCA
 ATTTGTGGTAAATCTTTGGAATCTACAAATGTTACAGATAAAGATGGAGA
 ATTGTATTGTAAAGTTTGTATGCTAAAAATTTTGACCTACAGGTATAGG
 ATTTGGAGGTTTGACACAACAAGTTGAAAAAAAAGAATGATCAACCTCTG
 GATTACAAAATTTGTGAAAGATTGACTGGTATTCTTAACTATGTTGCTCCT
 TTTACGCTATGTGGATACGCTGCTTTAATGCCTTTGTATCATGCTATTGCTT

CCCGTATGGCTTTTCATTTTCTCCTCCTTGTATAAATCCTGGTTGCTGTCTCT
TTATGAGGAGTTGTGGCCCGTTGTCAGGCAACGTGGCGTGGTGTGCACT
GTGTTTGCTGACGCAACCCCCACTGGTTGGGGCATTGCCACCACCTGTCA
GCTCCTTTCCGGGACTTTCGCTTTCCCCCTCCCTATTGCCACGGCGGAACT
CATCGCCGCCTGCCTTGCCCGCTGCTGGACAGGGGCTCGGCTGTTGGGCA
CTGACAATTCCGTGGTGTGTCGGGGAAATCATCGTCCTTTCTTGGCTGC
TCGCCTGTGTTGCCACCTGGATTCTGCGCGGGACGTCCTTCTGCTACGTCC
CTTCGGCCCTCAATCCAGCGGACCTTCCTTCCCGCGGCCTGCTGCCGGCTC
TGCGGCCTCTTCCGCGTCTTCGCCTTCGCCCTCAGACGAGTCGGATCTCCC
TTTGGGCCCGCCTCCCCGCACTGCCCCGGGTGGCATCCCTGTGACCCCTCCCC
AGTGCCTCTCCTGGCCCTGGAAGTTGCCACTCCAGTGCCCACCAGCCTTGT
CCTAATAAAATTAAGTTGCATCATTTTGTCTGACTAGGTGTCCTTCTATAAT
ATTATGGGGTGGAGGGGGGTGGTATGGAGCAAGGGGGCCCAAGTTGGGA
AGAAACCTGTAGGGCCTGCaggaaccctagtgatggagactccctctctgcgcgctcg
ctcgctcactgaggccgggcgaccaaaggtcgcccgacgcccgggctttgcccgggcggcctca
gtgagcgagcgagcgcgagagaggagt

FIG. 4 (con't)

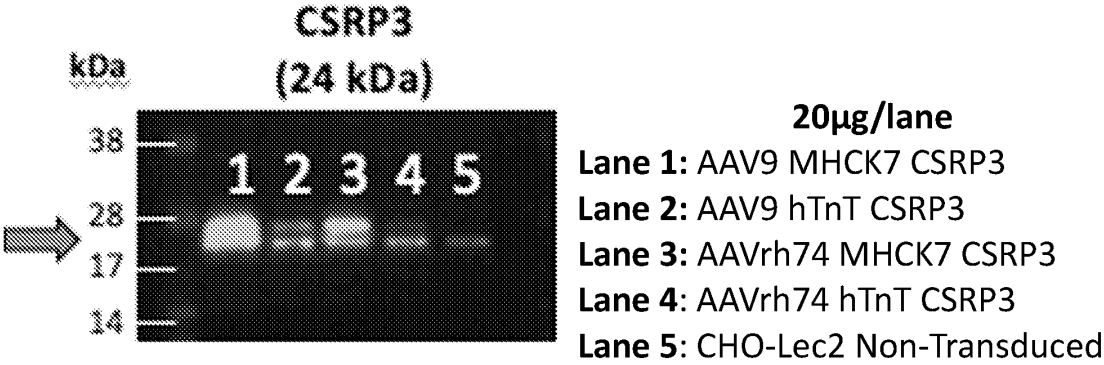


FIG. 5A

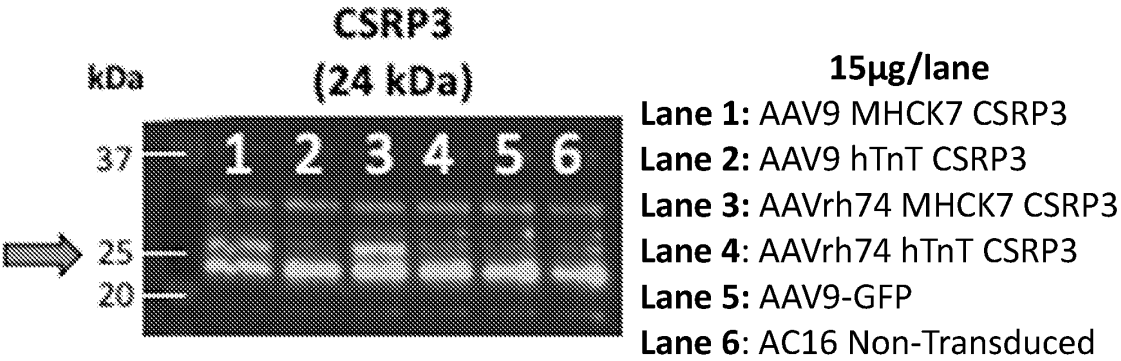


FIG. 5B