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<110> Benitec Biopharma Limited

<120> Compositions and Methods for Treating Oculopharyngeal Muscular Dystrophy (OPMD)

<130> 186752PCT

<150> US 62/812,187
<151> 2019-02-28

<160> 98

<170> PatentIn version 3.5

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ccaacacctg ctgcctctaa aaataaccct gcatgccatg ttcccggcga agggccagct 180

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agccatcaag gccagagtgc gggagatgga agaggaggcc gaaaaattga aagagctgca 1740

gaacgaagtc gaaaaacaaa tgaacatgtc ccccccctcct ggaaatgctg gccctgtgat 1800

catgagcatc gaggaaaaga tggaagccga cgcccggtct atctacgtgg gcaacgtgga 1860

ctacggcgcc accgccgaag aactggaagc ccactttcac ggctgtggca gcgtgaaccg 1920

ggtgaccatc ctgtgcgaca agttcagcgg ccaccccaag ggcttcgcct acatcgagtt 1980

cagcgacaaa gaaagcgtgc ggacctctct ggctctcgac gagtctctgt tcaggggaag 2040

gcagatcaag gtcatcccca agcggaccaa caggcccggc atcagcacca ccgacagagg 2100

cttccctagg gctaggtaca gagcccggac caccaactac aacagcagca gaagccggtt 2160

ctacagcggc ttcaattctc ggcctagagg cagagtgtac cggggcaggg ccagggccac 2220

ctcctggtac agcccctacg aacagaagct gatcagcgag gaagatctgt gatgagatat 2280

ctgatgacat atgacgcgtt taattaactg tgccttctag ttgccagcca tctgttgttt 2340

gcccctcccc cgtgccttcc ttgaccctgg aaggtgccac tcccactgtc ctttcctaat 2400

aaaatgagga aattgcatcg cattgtctga gtaggtgtca ttctattctg gggggtgggg 2460

tggggcagga cagcaagggg gaggattggg aagacaatag caggcatgct ggggatgcgg 2520

tgggctctat gg 2532

<210> 71
<211> 1943
<212> DNA
<213> Artificial sequence

<220>
<223> Double expression construct version 2 coding for shmiR3, shmiR14 and codon optimized PABPN1

<400> 71
cgagctccac cgcggtggcg gccgtccgcc ctcggcacca tcctcacgac acccaaatat 60

ggcgacgggt gaggaatggt ggggagttat ttttagagcg gtgaggaagg tgggcaggca 120

gcaggtgttg gcgctctaaa aataactccc gggagttatt tttagagcgg aggaatggtg 180

gacacccaaa tatggcgacg gttcctcacc cgtcgccata tttgggtgtc cgccctcggc 240

cggggccgca ttcctggggg ccgggcggtg ctcccgcccg cctcgataaa aggctccggg 300

gccggcggcg gcccacgagc tacccggagg agcgggaggc gccaagctct agaactagtg 360

gatcccccgg gctgcaggaa ttcgatgcca ccatggccgc tgccgccgct gctgctgccg 420

cagccggcgc tgccggcgga agaggcagcg gccctggcag acggcggcat ctggtccctg 480

gcgccggagg ggaggccggc gaaggcgccc ctggcggagc cggcgactac ggcaacggcc 540

tggaaagcga ggaactggaa cccgaggaac tgctgctgga acctgagccc gagccagagc 600

ccgaggaaga gccccctagg ccaagagccc cccctggcgc cccaggacca ggaccaggct 660

ctggggcacc aggctctcag gaagaggaag aagagcccgg cctcgtcgag ggagacccag 720

gcgatggcgc tatcgaagat cccgagctgg aagccatcaa ggccagagtg cgggagatgg 780

aagaggaggc cgaaaaattg aaagagctgc agaacgaagt cgaaaaacaa atgaacatgt 840

ccccccctcc tggaaatgct ggccctgtga tcatgagcat cgaggaaaag atggaagccg 900

acgcccggtc tatctacgtg ggcaacgtgg actacggcgc caccgccgaa gaactggaag 960

cccactttca cggctgtggc agcgtgaacc gggtgaccat cctgtgcgac aagttcagcg 1020

gccaccccaa gggcttcgcc tacatcgagt tcagcgacaa agaaagcgtg cggacctctc 1080

tggctctcga cgagtctctg ttcaggggaa ggcagatcaa ggtcatcccc aagcggacca 1140

acaggcccgg catcagcacc accgacagag gcttccctag ggctaggtac agagcccgga 1200

ccaccaacta caacagcagc agaagccggt tctacagcgg cttcaattct cggcctagag 1260

gcagagtgta ccggggcagg gccagggcca cctcctggta cagcccctac tgatgacata 1320

tgacgcgtgg tatattgctg ttgacagtga gcgagaggta gagaagcaga tgaaactgtg 1380

aagcagatgg gtttcatctg cttctctacc tcgcgcctac tgcctcggac ttcaaatcat 1440

ctactccatg gccctctgcg tttgctgaag acagaaccgc aaagcaggac ccgacaggat 1500

tctccccgcc tcttcagaga ctatgtttac aagatatcgg tatattgctg ttgacagtga 1560

gcgtgagaag cagatgaata tgagactgtg aagcagatgg gtctcatatt catctgcttc 1620

tctcgcctac tgcctcggac ttcaagtcga cgctagcaat aaaggatcct ttattttcat 1680

tggatccgtg tgttggtttt ttgtgtgcgg ttaattaact gtgccttcta gttgccagcc 1740

atctgttgtt tgcccctccc ccgtgccttc cttgaccctg gaaggtgcca ctcccactgt 1800

cctttcctaa taaaatgagg aaattgcatc gcattgtctg agtaggtgtc attctattct 1860

ggggggtggg gtggggcagg acagcaaggg ggaggattgg gaagacaata gcaggcatgc 1920

tggggatgcg gtgggctcta tgg 1943

<210> 72
<211> 1943
<212> DNA
<213> Artificial sequence

<220>
<223> Double expression construct version 2 coding for shmiR17, shmiR13 and codon optimized PABPN1

<400> 72
cgagctccac cgcggtggcg gccgtccgcc ctcggcacca tcctcacgac acccaaatat 60

ggcgacgggt gaggaatggt ggggagttat ttttagagcg gtgaggaagg tgggcaggca 120

gcaggtgttg gcgctctaaa aataactccc gggagttatt tttagagcgg aggaatggtg 180

gacacccaaa tatggcgacg gttcctcacc cgtcgccata tttgggtgtc cgccctcggc 240

cggggccgca ttcctggggg ccgggcggtg ctcccgcccg cctcgataaa aggctccggg 300

gccggcggcg gcccacgagc tacccggagg agcgggaggc gccaagctct agaactagtg 360

gatcccccgg gctgcaggaa ttcgatgcca ccatggccgc tgccgccgct gctgctgccg 420

cagccggcgc tgccggcgga agaggcagcg gccctggcag acggcggcat ctggtccctg 480

gcgccggagg ggaggccggc gaaggcgccc ctggcggagc cggcgactac ggcaacggcc 540

tggaaagcga ggaactggaa cccgaggaac tgctgctgga acctgagccc gagccagagc 600

ccgaggaaga gccccctagg ccaagagccc cccctggcgc cccaggacca ggaccaggct 660

ctggggcacc aggctctcag gaagaggaag aagagcccgg cctcgtcgag ggagacccag 720

gcgatggcgc tatcgaagat cccgagctgg aagccatcaa ggccagagtg cgggagatgg 780

aagaggaggc cgaaaaattg aaagagctgc agaacgaagt cgaaaaacaa atgaacatgt 840

ccccccctcc tggaaatgct ggccctgtga tcatgagcat cgaggaaaag atggaagccg 900

acgcccggtc tatctacgtg ggcaacgtgg actacggcgc caccgccgaa gaactggaag 960

cccactttca cggctgtggc agcgtgaacc gggtgaccat cctgtgcgac aagttcagcg 1020

gccaccccaa gggcttcgcc tacatcgagt tcagcgacaa agaaagcgtg cggacctctc 1080

tggctctcga cgagtctctg ttcaggggaa ggcagatcaa ggtcatcccc aagcggacca 1140

acaggcccgg catcagcacc accgacagag gcttccctag ggctaggtac agagcccgga 1200

ccaccaacta caacagcagc agaagccggt tctacagcgg cttcaattct cggcctagag 1260

gcagagtgta ccggggcagg gccagggcca cctcctggta cagcccctac tgatgacata 1320

tgacgcgtgg tatattgctg ttgacagtga gcgaaggtag agaagcagat gaatactgtg 1380

aagcagatgg gtattcatct gcttctctac ctccgcctac tgcctcggac ttcaaatcat 1440

ctactccatg gccctctgcg tttgctgaag acagaaccgc aaagcaggac ccgacaggat 1500

tctccccgcc tcttcagaga ctatgtttac aagatatcgg tatattgctg ttgacagtga 1560

gcgacgacat catggtattc ccctactgtg aagcagatgg gtaggggaat accatgatgt 1620

cgccgcctac tgcctcggac ttcaagtcga cgctagcaat aaaggatcct ttattttcat 1680

tggatccgtg tgttggtttt ttgtgtgcgg ttaattaact gtgccttcta gttgccagcc 1740

atctgttgtt tgcccctccc ccgtgccttc cttgaccctg gaaggtgcca ctcccactgt 1800

cctttcctaa taaaatgagg aaattgcatc gcattgtctg agtaggtgtc attctattct 1860

gggggggtggg gtggggcagg acagcaaggg ggaggattgg gaagacaata gcaggcatgc 1920

tggggatgcg gtgggctcta tgg 1943

<210> 73

<211> 921
<212> DNA
<213> Artificial sequence

<220>
<223> Human codon-optimized PABPN1 cDNA sequence

<400> 73
atggccgctg ccgccgctgc tgctgccgca gccggcgctg ccggcggaag aggcagcggc 60

cctggcagac ggcggcatct ggtccctggc gccggagggg aggccggcga aggcgcccct 120

ggcggagccg gcgactacgg caacggcctg gaaagcgagg aactggaacc cgaggaactg 180

ctgctggaac ctgagcccga gccagagccc gaggaagagc cccctaggcc aagagccccc 240

cctggcgccc caggaccagg accaggctct ggggcaccag gctctcagga agaggaagaa 300

gagcccggcc tcgtcgaggg agacccaggc gatggcgcta tcgaagatcc cgagctggaa 360

gccatcaagg ccagagtgcg ggagatggaa gaggaggccg aaaaattgaa agagctgcag 420

aacgaagtcg aaaaacaaat gaacatgtcc ccccctcctg gaaatgctgg ccctgtgatc 480

atgagcatcg aggaaaagat ggaagccgac gcccggtcta tctacgtggg caacgtggac 540

tacggcgcca ccgccgaaga actggaagcc cactttcacg gctgtggcag cgtgaaccgg 600

gtgaccatcc tgtgcgacaa gttcagcggc caccccaagg gcttcgccta catcgagttc 660

agcgacaaag aaagcgtgcg gacctctctg gctctcgacg agtctctgtt caggggaagg 720

cagatcaagg tcatccccaa gcggaccaac aggcccggca tcagcaccac cgacagaggc 780

ttccctaggg ctaggtacag agcccggacc accaactaca acagcagcag aagccggttc 840

tacagcggct tcaattctcg gcctagaggc agagtgtacc ggggcagggc cagggccacc 900

tcctggtaca gcccctactg a 921

<210> 74
<211> 306
<212> PRT
<213> Artificial sequence

<220>
<223> Human wildtype PABPN1 amino acid sequence

<400> 74

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Met Ala Ala Ala Ala Ala Ala Ala Ala Ala Ala Gly Ala Ala Gly Gly
1               5                  10                  15

Arg Gly Ser Gly Pro Gly Arg Arg Arg His Leu Val Pro Gly Ala Gly
            20                  25                  30

Gly Glu Ala Gly Glu Gly Ala Pro Gly Gly Ala Gly Asp Tyr Gly Asn
        35                  40                  45

Gly Leu Glu Ser Glu Glu Leu Glu Pro Glu Glu Leu Leu Leu Glu Pro
    50                  55                  60

Glu Pro Glu Pro Glu Pro Glu Glu Glu Pro Pro Arg Pro Arg Ala Pro
65                  70                  75                  80

Pro Gly Ala Pro Gly Pro Gly Pro Gly Ser Gly Ala Pro Gly Ser Gln
                85                  90                  95

Glu Glu Glu Glu Glu Pro Gly Leu Val Glu Gly Asp Pro Gly Asp Gly
            100                 105                 110

Ala Ile Glu Asp Pro Glu Leu Glu Ala Ile Lys Ala Arg Val Arg Glu
        115                 120                 125

Met Glu Glu Glu Ala Glu Lys Leu Lys Glu Leu Gln Asn Glu Val Glu
    130                 135                 140

Lys Gln Met Asn Met Ser Pro Pro Pro Gly Asn Ala Gly Pro Val Ile
145                 150                 155                 160

Met Ser Ile Glu Glu Lys Met Glu Ala Asp Ala Arg Ser Ile Tyr Val
                165                 170                 175

Gly Asn Val Asp Tyr Gly Ala Thr Ala Glu Glu Leu Glu Ala His Phe
            180                 185                 190

His Gly Cys Gly Ser Val Asn Arg Val Thr Ile Leu Cys Asp Lys Phe
```

```
        195                 200                 205

Ser Gly His Pro Lys Gly Phe Ala Tyr Ile Glu Phe Ser Asp Lys Glu
    210                 215                 220

Ser Val Arg Thr Ser Leu Ala Leu Asp Glu Ser Leu Phe Arg Gly Arg
225                 230                 235                 240

Gln Ile Lys Val Ile Pro Lys Arg Thr Asn Arg Pro Gly Ile Ser Thr
                245                 250                 255

Thr Asp Arg Gly Phe Pro Arg Ala Arg Tyr Arg Ala Arg Thr Thr Asn
            260                 265                 270

Tyr Asn Ser Ser Arg Ser Arg Phe Tyr Ser Gly Phe Asn Ser Arg Pro
        275                 280                 285

Arg Gly Arg Val Tyr Arg Gly Arg Ala Arg Ala Thr Ser Trp Tyr Ser
    290                 295                 300

Pro Tyr
305


<210>  75
<211>  314
<212>  PRT
<213>  Artificial sequence

<220>
<223>  Human wildtype PABPN1 amino acid sequence (with FLAG tag)

<400>  75

Met Ala Ala Ala Ala Ala Ala Ala Ala Ala Ala Gly Ala Ala Gly Gly
1               5                   10                  15

Arg Gly Ser Gly Pro Gly Arg Arg Arg His Leu Val Pro Gly Ala Gly
            20                  25                  30

Gly Glu Ala Gly Glu Gly Ala Pro Gly Gly Ala Gly Asp Tyr Gly Asn
```

```
        35                  40                  45

Gly Leu Glu Ser Glu Glu Leu Glu Pro Glu Glu Leu Leu Leu Glu Pro
    50                  55                  60

Glu Pro Glu Pro Glu Pro Glu Glu Glu Pro Pro Arg Pro Arg Ala Pro
65                  70                  75                  80

Pro Gly Ala Pro Gly Pro Gly Pro Gly Ser Gly Ala Pro Gly Ser Gln
                85                  90                  95

Glu Glu Glu Glu Glu Pro Gly Leu Val Glu Gly Asp Pro Gly Asp Gly
            100                 105                 110

Ala Ile Glu Asp Pro Glu Leu Glu Ala Ile Lys Ala Arg Val Arg Glu
        115                 120                 125

Met Glu Glu Glu Ala Glu Lys Leu Lys Glu Leu Gln Asn Glu Val Glu
    130                 135                 140

Lys Gln Met Asn Met Ser Pro Pro Pro Gly Asn Ala Gly Pro Val Ile
145                 150                 155                 160

Met Ser Ile Glu Glu Lys Met Glu Ala Asp Ala Arg Ser Ile Tyr Val
                165                 170                 175

Gly Asn Val Asp Tyr Gly Ala Thr Ala Glu Glu Leu Glu Ala His Phe
            180                 185                 190

His Gly Cys Gly Ser Val Asn Arg Val Thr Ile Leu Cys Asp Lys Phe
        195                 200                 205

Ser Gly His Pro Lys Gly Phe Ala Tyr Ile Glu Phe Ser Asp Lys Glu
    210                 215                 220

Ser Val Arg Thr Ser Leu Ala Leu Asp Glu Ser Leu Phe Arg Gly Arg
225                 230                 235                 240
```

```
Gln Ile Lys Val Ile Pro Lys Arg Thr Asn Arg Pro Gly Ile Ser Thr
                245                 250                 255


Thr Asp Arg Gly Phe Pro Arg Ala Arg Tyr Arg Ala Arg Thr Thr Asn
            260                 265                 270


Tyr Asn Ser Ser Arg Ser Arg Phe Tyr Ser Gly Phe Asn Ser Arg Pro
        275                 280                 285


Arg Gly Arg Val Tyr Arg Gly Arg Ala Arg Ala Thr Ser Trp Tyr Ser
    290                 295                 300


Pro Tyr Asp Tyr Lys Asp Asp Asp Asp Lys
305                 310
```

<210> 76
<211> 314
<212> PRT
<213> Artificial sequence

<220>
<223> Human codon-optimized PABPN1 amino acid sequence (with FLAG-tag)

<400> 76

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Met Ala Ala Ala Ala Ala Ala Ala Ala Ala Ala Gly Ala Ala Gly Gly
1               5                   10                  15


Arg Gly Ser Gly Pro Gly Arg Arg Arg His Leu Val Pro Gly Ala Gly
            20                  25                  30


Gly Glu Ala Gly Glu Gly Ala Pro Gly Gly Ala Gly Asp Tyr Gly Asn
        35                  40                  45


Gly Leu Glu Ser Glu Glu Leu Glu Pro Glu Glu Leu Leu Leu Glu Pro
    50                  55                  60


Glu Pro Glu Pro Glu Pro Glu Glu Glu Pro Pro Arg Pro Arg Ala Pro
65                  70                  75                  80
```

```
Pro Gly Ala Pro Gly Pro Gly Pro Gly Ser Gly Ala Pro Gly Ser Gln
                85                  90                  95

Glu Glu Glu Glu Glu Pro Gly Leu Val Glu Gly Asp Pro Gly Asp Gly
            100                 105                 110

Ala Ile Glu Asp Pro Glu Leu Glu Ala Ile Lys Ala Arg Val Arg Glu
        115                 120                 125

Met Glu Glu Glu Ala Glu Lys Leu Lys Glu Leu Gln Asn Glu Val Glu
    130                 135                 140

Lys Gln Met Asn Met Ser Pro Pro Pro Gly Asn Ala Gly Pro Val Ile
145                 150                 155                 160

Met Ser Ile Glu Glu Lys Met Glu Ala Asp Ala Arg Ser Ile Tyr Val
                165                 170                 175

Gly Asn Val Asp Tyr Gly Ala Thr Ala Glu Glu Leu Glu Ala His Phe
            180                 185                 190

His Gly Cys Gly Ser Val Asn Arg Val Thr Ile Leu Cys Asp Lys Phe
        195                 200                 205

Ser Gly His Pro Lys Gly Phe Ala Tyr Ile Glu Phe Ser Asp Lys Glu
    210                 215                 220

Ser Val Arg Thr Ser Leu Ala Leu Asp Glu Ser Leu Phe Arg Gly Arg
225                 230                 235                 240

Gln Ile Lys Val Ile Pro Lys Arg Thr Asn Arg Pro Gly Ile Ser Thr
                245                 250                 255

Thr Asp Arg Gly Phe Pro Arg Ala Arg Tyr Arg Ala Arg Thr Thr Asn
            260                 265                 270

Tyr Asn Ser Ser Arg Ser Arg Phe Tyr Ser Gly Phe Asn Ser Arg Pro
```

275 280 285

Arg Gly Arg Val Tyr Arg Gly Arg Ala Arg Ala Thr Ser Trp Tyr Ser
290 295 300

Pro Tyr Asp Tyr Lys Asp Asp Asp Asp Lys
305 310

<210> 77
<211> 20
<212> DNA
<213> Artificial sequence

<220>
<223> wtPABPN1-Fwd primer

<400> 77
atggtgcaac agcagaagag 20

<210> 78
<211> 20
<212> DNA
<213> Artificial sequence

<220>
<223> wtPABPN1-Rev primer

<400> 78
ctttgggatg gccactaaat 20

<210> 79
<211> 23
<212> DNA
<213> Artificial sequence

<220>
<223> wtPABPN1-Probe

<400> 79
cggttgactg aaccacagcc atg 23

<210> 80
<211> 19
<212> DNA

<213> Artificial sequence

<220>
<223> optPABPN1-For primer

<400> 80
accgacagag gcttcccta 19

<210> 81
<211> 21
<212> DNA
<213> Artificial sequence

<220>
<223> optPABPN1-Rev primer

<400> 81
ttctgctgct gttgtagttg g 21

<210> 82
<211> 23
<212> DNA
<213> Artificial sequence

<220>
<223> optPABPN1-Probe

<400> 82
tggtccgggc tctgtaccta gcc 23

<210> 83
<211> 21
<212> DNA
<213> Artificial sequence

<220>
<223> shmiR3-Fwd primer

<400> 83
ttcatctgct tctctacctc g 21

<210> 84
<211> 21
<212> DNA
<213> Artificial sequence

<220>
<223> shmiR13-Fwd primer

<400> 84
aggggaatac catgatgtcg c 21

<210> 85
<211> 21
<212> DNA
<213> Artificial sequence

<220>
<223> shmiR14-Fwd primer

<400> 85
ctcatattca tctgcttctc t 21

<210> 86
<211> 21
<212> DNA
<213> Artificial sequence

<220>
<223> shmiR17-Fwd primer

<400> 86
attcatctgc ttctctacct c 21

<210> 87
<211> 64
<212> PRT
<213> Adenoassociated virus serotype 9

<400> 87

Ala Arg Gly Leu Val Leu Pro Gly Tyr Lys Tyr Leu Gly Pro Gly Asn
1 5 10 15

Gly Leu Asp Lys Gly Glu Pro Val Asn Ala Ala Asp Ala Ala Ala Leu
20 25 30

Glu His Asp Lys Ala Tyr Asp Gln Gln Leu Lys Ala Gly Asp Asn Pro
35 40 45

Tyr Leu Lys Tyr Asn His Ala Asp Ala Glu Phe Gln Glu Arg Leu Lys
    50   55   60

<210> 88
<211> 64
<212> PRT
<213> Artificial Sequence

<220>
<223> Modified VP1 PL subsequence for AAV9

<400> 88

Ser Arg Gly Leu Val Leu Pro Gly Tyr Lys Tyr Leu Gly Pro Gly Asn
1   5   10   15

Gly Leu Asp Lys Gly Glu Pro Val Asn Glu Ala Asp Ala Ala Ala Leu
            20   25   30

Glu His Asp Lys Ala Tyr Asp Arg Gln Leu Asp Ser Gly Asp Asn Pro
        35   40   45

Tyr Leu Lys Tyr Asn His Ala Asp Ala Glu Phe Gln Glu Arg Leu Lys
    50   55   60

<210> 89
<211> 736
<212> PRT
<213> Adenoassociated virus serotype 9

<400> 89

Met Ala Ala Asp Gly Tyr Leu Pro Asp Trp Leu Glu Asp Asn Leu Ser
1   5   10   15

Glu Gly Ile Arg Glu Trp Trp Ala Leu Lys Pro Gly Ala Pro Gln Pro
            20   25   30

Lys Ala Asn Gln Gln His Gln Asp Asn Ala Arg Gly Leu Val Leu Pro
        35   40   45

Gly Tyr Lys Tyr Leu Gly Pro Gly Asn Gly Leu Asp Lys Gly Glu Pro

```
    50                  55                  60

Val Asn Ala Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
65                  70                  75                  80

Gln Gln Leu Lys Ala Gly Asp Asn Pro Tyr Leu Lys Tyr Asn His Ala
                85                  90                  95

Asp Ala Glu Phe Gln Glu Arg Leu Lys Glu Asp Thr Ser Phe Gly Gly
            100                 105                 110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Leu Leu Glu Pro
        115                 120                 125

Leu Gly Leu Val Glu Glu Ala Ala Lys Thr Ala Pro Gly Lys Lys Arg
    130                 135                 140

Pro Val Glu Gln Ser Pro Gln Glu Pro Asp Ser Ser Ala Gly Ile Gly
145                 150                 155                 160

Lys Ser Gly Ala Gln Pro Ala Lys Lys Arg Leu Asn Phe Gly Gln Thr
                165                 170                 175

Gly Asp Thr Glu Ser Val Pro Asp Pro Gln Pro Ile Gly Glu Pro Pro
            180                 185                 190

Ala Ala Pro Ser Gly Val Gly Ser Leu Thr Met Ala Ser Gly Gly Gly
        195                 200                 205

Ala Pro Val Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Ser Ser
    210                 215                 220

Ser Gly Asn Trp His Cys Asp Ser Gln Trp Leu Gly Asp Arg Val Ile
225                 230                 235                 240

Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His Leu
                245                 250                 255
```

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Tyr Lys Gln Ile Ser Asn Ser Thr Ser Gly Gly Ser Ser Asn Asp Asn
            260                 265                 270

Ala Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg
        275                 280                 285

Phe His Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn
    290                 295                 300

Asn Trp Gly Phe Arg Pro Lys Arg Leu Asn Phe Lys Leu Phe Asn Ile
305                 310                 315                 320

Gln Val Lys Glu Val Thr Asp Asn Asn Gly Val Lys Thr Ile Ala Asn
                325                 330                 335

Asn Leu Thr Ser Thr Val Gln Val Phe Thr Asp Ser Asp Tyr Gln Leu
            340                 345                 350

Pro Tyr Val Leu Gly Ser Ala His Glu Gly Cys Leu Pro Pro Phe Pro
        355                 360                 365

Ala Asp Val Phe Met Ile Pro Gln Tyr Gly Tyr Leu Thr Leu Asn Asp
    370                 375                 380

Gly Ser Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr Phe
385                 390                 395                 400

Pro Ser Gln Met Leu Arg Thr Gly Asn Asn Phe Gln Phe Ser Tyr Glu
                405                 410                 415

Phe Glu Asn Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser Leu
            420                 425                 430

Asp Arg Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu Ser
        435                 440                 445

Lys Thr Ile Asn Gly Ser Gly Gln Asn Gln Gln Thr Leu Lys Phe Ser
```

```
    450                 455                 460

Val Ala Gly Pro Ser Asn Met Ala Val Gln Gly Arg Asn Tyr Ile Pro
465                 470                 475                 480

Gly Pro Ser Tyr Arg Gln Gln Arg Val Ser Thr Thr Val Thr Gln Asn
                485                 490                 495

Asn Asn Ser Glu Phe Ala Trp Pro Gly Ala Ser Ser Trp Ala Leu Asn
            500                 505                 510

Gly Arg Asn Ser Leu Met Asn Pro Gly Pro Ala Met Ala Ser His Lys
        515                 520                 525

Glu Gly Glu Asp Arg Phe Phe Pro Leu Ser Gly Ser Leu Ile Phe Gly
    530                 535                 540

Lys Gln Gly Thr Gly Arg Asp Asn Val Asp Ala Asp Lys Val Met Ile
545                 550                 555                 560

Thr Asn Glu Glu Glu Ile Lys Thr Thr Asn Pro Val Ala Thr Glu Ser
                565                 570                 575

Tyr Gly Gln Val Ala Thr Asn His Gln Ser Ala Gln Ala Gln Ala Gln
            580                 585                 590

Thr Gly Trp Val Gln Asn Gln Gly Ile Leu Pro Gly Met Val Trp Gln
        595                 600                 605

Asp Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro His
    610                 615                 620

Thr Asp Gly Asn Phe His Pro Ser Pro Leu Met Gly Gly Phe Gly Met
625                 630                 635                 640

Lys His Pro Pro Pro Gln Ile Leu Ile Lys Asn Thr Pro Val Pro Ala
                645                 650                 655
```

Asp Pro Pro Thr Ala Phe Asn Lys Asp Lys Leu Asn Ser Phe Ile Thr
            660   665   670

Gln Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu Leu Gln
        675   680   685

Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Ser Asn
    690   695   700

Tyr Tyr Lys Ser Asn Asn Val Glu Phe Ala Val Asn Thr Glu Gly Val
705   710   715   720

Tyr Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Asn Leu
                725   730   735

<210> 90
<211> 736
<212> PRT
<213> Artificial Sequence

<220>
<223> Modified AAV9 capsid VP1 (full length)

<400> 90

Met Ala Ala Asp Gly Tyr Leu Pro Asp Trp Leu Glu Asp Asn Leu Ser
1   5   10   15

Glu Gly Ile Arg Glu Trp Trp Ala Leu Lys Pro Gly Ala Pro Gln Pro
            20   25   30

Lys Ala Asn Gln Gln His Gln Asp Asn Ser Arg Gly Leu Val Leu Pro
        35   40   45

Gly Tyr Lys Tyr Leu Gly Pro Gly Asn Gly Leu Asp Lys Gly Glu Pro
    50   55   60

Val Asn Glu Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
65   70   75   80

```
Arg Gln Leu Asp Ser Gly Asp Asn Pro Tyr Leu Lys Tyr Asn His Ala
                85                  90                  95

Asp Ala Glu Phe Gln Glu Arg Leu Lys Glu Asp Thr Ser Phe Gly Gly
            100                 105                 110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Leu Leu Glu Pro
        115                 120                 125

Leu Gly Leu Val Glu Glu Ala Ala Lys Thr Ala Pro Gly Lys Lys Arg
    130                 135                 140

Pro Val Glu Gln Ser Pro Gln Glu Pro Asp Ser Ser Ala Gly Ile Gly
145                 150                 155                 160

Lys Ser Gly Ala Gln Pro Ala Lys Lys Arg Leu Asn Phe Gly Gln Thr
                165                 170                 175

Gly Asp Thr Glu Ser Val Pro Asp Pro Gln Pro Ile Gly Glu Pro Pro
            180                 185                 190

Ala Ala Pro Ser Gly Val Gly Ser Leu Thr Met Ala Ser Gly Gly Gly
        195                 200                 205

Ala Pro Val Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Ser Ser
    210                 215                 220

Ser Gly Asn Trp His Cys Asp Ser Gln Trp Leu Gly Asp Arg Val Ile
225                 230                 235                 240

Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His Leu
                245                 250                 255

Tyr Lys Gln Ile Ser Asn Ser Thr Ser Gly Gly Ser Ser Asn Asp Asn
            260                 265                 270

Ala Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg
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        275                 280                 285

Phe His Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn
    290                 295                 300

Asn Trp Gly Phe Arg Pro Lys Arg Leu Asn Phe Lys Leu Phe Asn Ile
305                 310                 315                 320

Gln Val Lys Glu Val Thr Asp Asn Asn Gly Val Lys Thr Ile Ala Asn
                325                 330                 335

Asn Leu Thr Ser Thr Val Gln Val Phe Thr Asp Ser Asp Tyr Gln Leu
            340                 345                 350

Pro Tyr Val Leu Gly Ser Ala His Glu Gly Cys Leu Pro Pro Phe Pro
        355                 360                 365

Ala Asp Val Phe Met Ile Pro Gln Tyr Gly Tyr Leu Thr Leu Asn Asp
    370                 375                 380

Gly Ser Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr Phe
385                 390                 395                 400

Pro Ser Gln Met Leu Arg Thr Gly Asn Asn Phe Gln Phe Ser Tyr Glu
                405                 410                 415

Phe Glu Asn Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser Leu
            420                 425                 430

Asp Arg Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu Ser
        435                 440                 445

Lys Thr Ile Asn Gly Ser Gly Gln Asn Gln Gln Thr Leu Lys Phe Ser
    450                 455                 460

Val Ala Gly Pro Ser Asn Met Ala Val Gln Gly Arg Asn Tyr Ile Pro
465                 470                 475                 480
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```
Gly Pro Ser Tyr Arg Gln Gln Arg Val Ser Thr Thr Val Thr Gln Asn
                485                 490                 495

Asn Asn Ser Glu Phe Ala Trp Pro Gly Ala Ser Ser Trp Ala Leu Asn
            500                 505                 510

Gly Arg Asn Ser Leu Met Asn Pro Gly Pro Ala Met Ala Ser His Lys
        515                 520                 525

Glu Gly Glu Asp Arg Phe Phe Pro Leu Ser Gly Ser Leu Ile Phe Gly
    530                 535                 540

Lys Gln Gly Thr Gly Arg Asp Asn Val Asp Ala Asp Lys Val Met Ile
545                 550                 555                 560

Thr Asn Glu Glu Glu Ile Lys Thr Thr Asn Pro Val Ala Thr Glu Ser
                565                 570                 575

Tyr Gly Gln Val Ala Thr Asn His Gln Ser Ala Gln Ala Gln Ala Gln
            580                 585                 590

Thr Gly Trp Val Gln Asn Gln Gly Ile Leu Pro Gly Met Val Trp Gln
        595                 600                 605

Asp Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro His
    610                 615                 620

Thr Asp Gly Asn Phe His Pro Ser Pro Leu Met Gly Gly Phe Gly Met
625                 630                 635                 640

Lys His Pro Pro Pro Gln Ile Leu Ile Lys Asn Thr Pro Val Pro Ala
                645                 650                 655

Asp Pro Pro Thr Ala Phe Asn Lys Asp Lys Leu Asn Ser Phe Ile Thr
            660                 665                 670

Gln Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu Leu Gln
```

675 680 685

Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Ser Asn
690 695 700

Tyr Tyr Lys Ser Asn Asn Val Glu Phe Ala Val Asn Thr Glu Gly Val
705 710 715 720

Tyr Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Asn Leu
725 730 735

<210> 91
<211> 64
<212> PRT
<213> Adenoassociated virus serotype 8

<400> 91

Gly Arg Gly Leu Val Leu Pro Gly Tyr Lys Tyr Leu Gly Pro Phe Asn
1 5 10 15

Gly Leu Asp Lys Gly Glu Pro Val Asn Ala Ala Asp Ala Ala Ala Leu
20 25 30

Glu His Asp Lys Ala Tyr Asp Gln Gln Leu Lys Ala Gly Asp Asn Pro
35 40 45

Tyr Leu Arg Tyr Asn His Ala Asp Ala Glu Phe Gln Glu Arg Leu Gln
50 55 60

<210> 92
<211> 64
<212> PRT
<213> Artificial Sequence

<220>
<223> Modified VP1 PL subsequence for AAV8

<400> 92

Ser Arg Gly Leu Val Leu Pro Gly Tyr Lys Tyr Leu Gly Pro Phe Asn
1 5 10 15

Gly Leu Asp Lys Gly Glu Pro Val Asn Glu Ala Asp Ala Ala Ala Leu
            20   25   30

Glu His Asp Lys Ala Tyr Asp Arg Gln Leu Asp Ser Gly Asp Asn Pro
        35   40   45

Tyr Leu Arg Tyr Asn His Ala Asp Ala Glu Phe Gln Glu Arg Leu Lys
    50   55   60

<210> 93
<211> 738
<212> PRT
<213> Adenoassociated virus serotype 8

<400> 93

Met Ala Ala Asp Gly Tyr Leu Pro Asp Trp Leu Glu Asp Asn Leu Ser
1   5   10   15

Glu Gly Ile Arg Glu Trp Trp Ala Leu Lys Pro Gly Ala Pro Lys Pro
            20   25   30

Lys Ala Asn Gln Gln Lys Gln Asp Asp Gly Arg Gly Leu Val Leu Pro
        35   40   45

Gly Tyr Lys Tyr Leu Gly Pro Phe Asn Gly Leu Asp Lys Gly Glu Pro
    50   55   60

Val Asn Ala Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
65   70   75   80

Gln Gln Leu Gln Ala Gly Asp Asn Pro Tyr Leu Arg Tyr Asn His Ala
                85   90   95

Asp Ala Glu Phe Gln Glu Arg Leu Gln Glu Asp Thr Ser Phe Gly Gly
            100   105   110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Val Leu Glu Pro

```
        115                 120                 125

Leu Gly Leu Val Glu Glu Gly Ala Lys Thr Ala Pro Gly Lys Lys Arg
    130                 135                 140

Pro Val Glu Pro Ser Pro Gln Arg Ser Pro Asp Ser Ser Thr Gly Ile
145                 150                 155                 160

Gly Lys Lys Gly Gln Gln Pro Ala Arg Lys Arg Leu Asn Phe Gly Gln
                165                 170                 175

Thr Gly Asp Ser Glu Ser Val Pro Asp Pro Gln Pro Leu Gly Glu Pro
            180                 185                 190

Pro Ala Ala Pro Ser Gly Val Gly Pro Asn Thr Met Ala Ala Gly Gly
        195                 200                 205

Gly Ala Pro Met Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Ser
    210                 215                 220

Ser Ser Gly Asn Trp His Cys Asp Ser Thr Trp Leu Gly Asp Arg Val
225                 230                 235                 240

Ile Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His
                245                 250                 255

Leu Tyr Lys Gln Ile Ser Asn Gly Thr Ser Gly Gly Ala Thr Asn Asp
            260                 265                 270

Asn Thr Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn
        275                 280                 285

Arg Phe His Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn
    290                 295                 300

Asn Asn Trp Gly Phe Arg Pro Lys Arg Leu Ser Phe Lys Leu Phe Asn
305                 310                 315                 320
```

```
Ile Gln Val Lys Glu Val Thr Gln Asn Glu Gly Thr Lys Thr Ile Ala
                325                 330                 335

Asn Asn Leu Thr Ser Thr Ile Gln Val Phe Thr Asp Ser Glu Tyr Gln
            340                 345                 350

Leu Pro Tyr Val Leu Gly Ser Ala His Gln Gly Cys Leu Pro Pro Phe
        355                 360                 365

Pro Ala Asp Val Phe Met Ile Pro Gln Tyr Gly Tyr Leu Thr Leu Asn
    370                 375                 380

Asn Gly Ser Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr
385                 390                 395                 400

Phe Pro Ser Gln Met Leu Arg Thr Gly Asn Asn Phe Gln Phe Thr Tyr
                405                 410                 415

Thr Phe Glu Asp Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser
            420                 425                 430

Leu Asp Arg Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu
        435                 440                 445

Ser Arg Thr Gln Thr Thr Gly Gly Thr Ala Asn Thr Gln Thr Leu Gly
    450                 455                 460

Phe Ser Gln Gly Gly Pro Asn Thr Met Ala Asn Gln Ala Lys Asn Trp
465                 470                 475                 480

Leu Pro Gly Pro Cys Tyr Arg Gln Gln Arg Val Ser Thr Thr Thr Gly
                485                 490                 495

Gln Asn Asn Asn Ser Asn Phe Ala Trp Thr Ala Gly Thr Lys Tyr His
            500                 505                 510

Leu Asn Gly Arg Asn Ser Leu Ala Asn Pro Gly Ile Ala Met Ala Thr
```

```
        515                 520                 525

His Lys Asp Asp Glu Glu Arg Phe Phe Pro Ser Asn Gly Ile Leu Ile
    530                 535                 540

Phe Gly Lys Gln Asn Ala Ala Arg Asp Asn Ala Asp Tyr Ser Asp Val
545                 550                 555                 560

Met Leu Thr Ser Glu Glu Glu Ile Lys Thr Thr Asn Pro Val Ala Thr
                565                 570                 575

Glu Glu Tyr Gly Ile Val Ala Asp Asn Leu Gln Gln Gln Asn Thr Ala
            580                 585                 590

Pro Gln Ile Gly Thr Val Asn Ser Gln Gly Ala Leu Pro Gly Met Val
        595                 600                 605

Trp Gln Asn Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile
    610                 615                 620

Pro His Thr Asp Gly Asn Phe His Pro Ser Pro Leu Met Gly Gly Phe
625                 630                 635                 640

Gly Leu Lys His Pro Pro Pro Gln Ile Leu Ile Lys Asn Thr Pro Val
                645                 650                 655

Pro Ala Asp Pro Pro Thr Thr Phe Asn Gln Ser Lys Leu Asn Ser Phe
            660                 665                 670

Ile Thr Gln Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu
        675                 680                 685

Leu Gln Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr
    690                 695                 700

Ser Asn Tyr Tyr Lys Ser Thr Ser Val Asp Phe Ala Val Asn Thr Glu
705                 710                 715                 720
```

Gly Val Tyr Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg
725 730 735

Asn Leu

<210> 94
<211> 738
<212> PRT
<213> Artificial Sequence

<220>
<223> Modified AAV8 capsid VP1 (full length)

<400> 94

Met Ala Ala Asp Gly Tyr Leu Pro Asp Trp Leu Glu Asp Asn Leu Ser
1 5 10 15

Glu Gly Ile Arg Glu Trp Trp Ala Leu Lys Pro Gly Ala Pro Lys Pro
20 25 30

Lys Ala Asn Gln Gln Lys Gln Asp Asp Ser Arg Gly Leu Val Leu Pro
35 40 45

Gly Tyr Lys Tyr Leu Gly Pro Phe Asn Gly Leu Asp Lys Gly Glu Pro
50 55 60

Val Asn Glu Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
65 70 75 80

Arg Gln Leu Asp Ser Gly Asp Asn Pro Tyr Leu Arg Tyr Asn His Ala
85 90 95

Asp Ala Glu Phe Gln Glu Arg Leu Lys Glu Asp Thr Ser Phe Gly Gly
100 105 110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Val Leu Glu Pro
115 120 125

```
Leu Gly Leu Val Glu Glu Gly Ala Lys Thr Ala Pro Gly Lys Lys Arg
    130                 135                 140

Pro Val Glu Pro Ser Pro Gln Arg Ser Pro Asp Ser Ser Thr Gly Ile
145                 150                 155                 160

Gly Lys Lys Gly Gln Gln Pro Ala Arg Lys Arg Leu Asn Phe Gly Gln
                165                 170                 175

Thr Gly Asp Ser Glu Ser Val Pro Asp Pro Gln Pro Leu Gly Glu Pro
            180                 185                 190

Pro Ala Ala Pro Ser Gly Val Gly Pro Asn Thr Met Ala Ala Gly Gly
        195                 200                 205

Gly Ala Pro Met Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Ser
    210                 215                 220

Ser Ser Gly Asn Trp His Cys Asp Ser Thr Trp Leu Gly Asp Arg Val
225                 230                 235                 240

Ile Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His
                245                 250                 255

Leu Tyr Lys Gln Ile Ser Asn Gly Thr Ser Gly Gly Ala Thr Asn Asp
            260                 265                 270

Asn Thr Tyr Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn
        275                 280                 285

Arg Phe His Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn
    290                 295                 300

Asn Asn Trp Gly Phe Arg Pro Lys Arg Leu Ser Phe Lys Leu Phe Asn
305                 310                 315                 320

Ile Gln Val Lys Glu Val Thr Gln Asn Glu Gly Thr Lys Thr Ile Ala
```

```
                325                 330                 335

Asn Asn Leu Thr Ser Thr Ile Gln Val Phe Thr Asp Ser Glu Tyr Gln
            340                 345                 350

Leu Pro Tyr Val Leu Gly Ser Ala His Gln Gly Cys Leu Pro Pro Phe
        355                 360                 365

Pro Ala Asp Val Phe Met Ile Pro Gln Tyr Gly Tyr Leu Thr Leu Asn
    370                 375                 380

Asn Gly Ser Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr
385                 390                 395                 400

Phe Pro Ser Gln Met Leu Arg Thr Gly Asn Asn Phe Gln Phe Thr Tyr
                405                 410                 415

Thr Phe Glu Asp Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser
            420                 425                 430

Leu Asp Arg Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu
        435                 440                 445

Ser Arg Thr Gln Thr Thr Gly Gly Thr Ala Asn Thr Gln Thr Leu Gly
    450                 455                 460

Phe Ser Gln Gly Gly Pro Asn Thr Met Ala Asn Gln Ala Lys Asn Trp
465                 470                 475                 480

Leu Pro Gly Pro Cys Tyr Arg Gln Gln Arg Val Ser Thr Thr Thr Gly
                485                 490                 495

Gln Asn Asn Asn Ser Asn Phe Ala Trp Thr Ala Gly Thr Lys Tyr His
            500                 505                 510

Leu Asn Gly Arg Asn Ser Leu Ala Asn Pro Gly Ile Ala Met Ala Thr
        515                 520                 525
```

```
His Lys Asp Asp Glu Glu Arg Phe Phe Pro Ser Asn Gly Ile Leu Ile
    530                 535                 540

Phe Gly Lys Gln Asn Ala Ala Arg Asp Asn Ala Asp Tyr Ser Asp Val
545                 550                 555                 560

Met Leu Thr Ser Glu Glu Glu Ile Lys Thr Thr Asn Pro Val Ala Thr
                565                 570                 575

Glu Glu Tyr Gly Ile Val Ala Asp Asn Leu Gln Gln Gln Asn Thr Ala
            580                 585                 590

Pro Gln Ile Gly Thr Val Asn Ser Gln Gly Ala Leu Pro Gly Met Val
        595                 600                 605

Trp Gln Asn Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile
    610                 615                 620

Pro His Thr Asp Gly Asn Phe His Pro Ser Pro Leu Met Gly Gly Phe
625                 630                 635                 640

Gly Leu Lys His Pro Pro Pro Gln Ile Leu Ile Lys Asn Thr Pro Val
                645                 650                 655

Pro Ala Asp Pro Pro Thr Thr Phe Asn Gln Ser Lys Leu Asn Ser Phe
            660                 665                 670

Ile Thr Gln Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu
        675                 680                 685

Leu Gln Lys Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr
    690                 695                 700

Ser Asn Tyr Tyr Lys Ser Thr Ser Val Asp Phe Ala Val Asn Thr Glu
705                 710                 715                 720

Gly Val Tyr Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg
```

725 730 735

Asn Leu

<210> 95
<211> 145
<212> DNA
<213> adeno-associated virus 2

<400> 95
ttggccactc cctctctgcg cgctcgctcg ctcactgagg ccgggcgacc aaaggtcgcc 60

cgacgcccgg gctttgcccg ggcggcctca gtgagcgagc gagcgcgcag agagggagtg 120

gccaactcca tcactagggg ttcct 145

<210> 96
<211> 145
<212> DNA
<213> adeno-associated virus 2

<400> 96
aggaacccct agtgatggag ttggccactc cctctctgcg cgctcgctcg ctcactgagg 60

ccgggcgacc aaaggtcgcc cgacgcccgg gctttgcccg ggcggcctca gtgagcgagc 120

gagcgcgcag agagggagtg gccaa 145

<210> 97
<211> 735
<212> PRT
<213> adeno-associated virus 2

<400> 97

Met Ala Ala Asp Gly Tyr Leu Pro Asp Trp Leu Glu Asp Thr Leu Ser
1 5 10 15

Glu Gly Ile Arg Gln Trp Trp Lys Leu Lys Pro Gly Pro Pro Pro Pro
20 25 30

Lys Pro Ala Glu Arg His Lys Asp Asp Ser Arg Gly Leu Val Leu Pro
35 40 45

```
Gly Tyr Lys Tyr Leu Gly Pro Phe Asn Gly Leu Asp Lys Gly Glu Pro
    50                  55                  60

Val Asn Glu Ala Asp Ala Ala Ala Leu Glu His Asp Lys Ala Tyr Asp
65                  70                  75                  80

Arg Gln Leu Asp Ser Gly Asp Asn Pro Tyr Leu Lys Tyr Asn His Ala
                85                  90                  95

Asp Ala Glu Phe Gln Glu Arg Leu Lys Glu Asp Thr Ser Phe Gly Gly
            100                 105                 110

Asn Leu Gly Arg Ala Val Phe Gln Ala Lys Lys Arg Val Leu Glu Pro
        115                 120                 125

Leu Gly Leu Val Glu Glu Pro Val Lys Thr Ala Pro Gly Lys Lys Arg
    130                 135                 140

Pro Val Glu His Ser Pro Val Glu Pro Asp Ser Ser Ser Gly Thr Gly
145                 150                 155                 160

Lys Ala Gly Gln Gln Pro Ala Arg Lys Arg Leu Asn Phe Gly Gln Thr
                165                 170                 175

Gly Asp Ala Asp Ser Val Pro Asp Pro Gln Pro Leu Gly Gln Pro Pro
            180                 185                 190

Ala Ala Pro Ser Gly Leu Gly Thr Asn Thr Met Ala Thr Gly Ser Gly
        195                 200                 205

Ala Pro Met Ala Asp Asn Asn Glu Gly Ala Asp Gly Val Gly Asn Ser
    210                 215                 220

Ser Gly Asn Trp His Cys Asp Ser Thr Trp Met Gly Asp Arg Val Ile
225                 230                 235                 240
```

```
Thr Thr Ser Thr Arg Thr Trp Ala Leu Pro Thr Tyr Asn Asn His Leu
                245                 250                 255

Tyr Lys Gln Ile Ser Ser Gln Ser Gly Ala Ser Asn Asp Asn His Tyr
            260                 265                 270

Phe Gly Tyr Ser Thr Pro Trp Gly Tyr Phe Asp Phe Asn Arg Phe His
        275                 280                 285

Cys His Phe Ser Pro Arg Asp Trp Gln Arg Leu Ile Asn Asn Asn Trp
    290                 295                 300

Gly Phe Arg Pro Lys Arg Leu Asn Phe Lys Leu Phe Asn Ile Gln Val
305                 310                 315                 320

Lys Glu Val Thr Gln Asn Asp Gly Thr Thr Thr Ile Ala Asn Asn Leu
                325                 330                 335

Thr Ser Thr Val Gln Val Phe Thr Asp Ser Glu Tyr Gln Leu Pro Tyr
            340                 345                 350

Val Leu Gly Ser Ala His Gln Gly Cys Leu Pro Pro Phe Pro Ala Asp
        355                 360                 365

Val Phe Met Val Pro Gln Tyr Gly Tyr Leu Thr Leu Asn Asn Gly Ser
    370                 375                 380

Gln Ala Val Gly Arg Ser Ser Phe Tyr Cys Leu Glu Tyr Phe Pro Ser
385                 390                 395                 400

Gln Met Leu Arg Thr Gly Asn Asn Phe Thr Phe Ser Tyr Thr Phe Glu
                405                 410                 415

Asp Val Pro Phe His Ser Ser Tyr Ala His Ser Gln Ser Leu Asp Arg
            420                 425                 430

Leu Met Asn Pro Leu Ile Asp Gln Tyr Leu Tyr Tyr Leu Ser Arg Thr
        435                 440                 445
```

```
Asn Thr Pro Ser Gly Thr Thr Thr Gln Ser Arg Leu Gln Phe Ser Gln
    450                 455                 460

Ala Gly Ala Ser Asp Ile Arg Asp Gln Ser Arg Asn Trp Leu Pro Gly
465                 470                 475                 480

Pro Cys Tyr Arg Gln Gln Arg Val Ser Lys Thr Ser Ala Asp Asn Asn
                485                 490                 495

Asn Ser Glu Tyr Ser Trp Thr Gly Ala Thr Lys Tyr His Leu Asn Gly
            500                 505                 510

Arg Asp Ser Leu Val Asn Pro Gly Pro Ala Met Ala Ser His Lys Asp
        515                 520                 525

Asp Glu Glu Lys Phe Phe Pro Gln Ser Gly Val Leu Ile Phe Gly Lys
    530                 535                 540

Gln Gly Ser Glu Lys Thr Asn Val Asp Ile Glu Lys Val Met Ile Thr
545                 550                 555                 560

Asp Glu Glu Glu Ile Arg Thr Thr Asn Pro Val Ala Thr Glu Gln Tyr
                565                 570                 575

Gly Ser Val Ser Thr Asn Leu Gln Arg Gly Asn Arg Gln Ala Ala Thr
            580                 585                 590

Ala Asp Val Asn Thr Gln Gly Val Leu Pro Gly Met Val Trp Gln Asp
        595                 600                 605

Arg Asp Val Tyr Leu Gln Gly Pro Ile Trp Ala Lys Ile Pro His Thr
    610                 615                 620

Asp Gly His Phe His Pro Ser Pro Leu Met Gly Gly Phe Gly Leu Lys
625                 630                 635                 640
```

His Pro Pro Pro Gln Ile Leu Ile Lys Asn Thr Pro Val Pro Ala Asn
645 650 655

Pro Ser Thr Thr Phe Ser Ala Ala Lys Phe Ala Ser Phe Ile Thr Gln
660 665 670

Tyr Ser Thr Gly Gln Val Ser Val Glu Ile Glu Trp Glu Leu Gln Lys
675 680 685

Glu Asn Ser Lys Arg Trp Asn Pro Glu Ile Gln Tyr Thr Ser Asn Tyr
690 695 700

Asn Lys Ser Val Asn Val Asp Phe Thr Val Asp Thr Asn Gly Val Tyr
705 710 715 720

Ser Glu Pro Arg Pro Ile Gly Thr Arg Tyr Leu Thr Arg Asn Leu
725 730 735

<210> 98
<211> 921
<212> RNA
<213> homo sapien

<400> 98
auggcggcgg cggcggcggc ggcagcagca gcgggggcug cgggcggucg gggcuccggg 60

ccggggcggc ggcgccaucu ugugcccggg gccgguggggg aggccgggga ggggggcccg 120

gggggcgcag gggacuacgg gaacggccug gagucugagg aacuggagcc ugaggagcug 180

cugcuggagc ccgagccgga gcccgagccc gaagaggagc cgccccggcc ccgcgccccc 240

ccgggagcuc cgggcccugg gccugguucg ggagcccccg gcagccaaga ggaggaggag 300

gagccgggac uggucgaggg ugacccgggg gacggcgcca uugaggaccc ggagcuggaa 360

gcuaucaaag cucgagucag ggagauggag gaagaagcug agaagcuaaa ggagcuacag 420

aacgagguag agaagcagau gaauaugagu ccaccuccag gcaaugcugg cccggugauc 480

auguccauug aggagaagau ggaggcugau gcccguucca ucuauguugg caauguggac 540

uauggugcaa cagcagaaga gcuggaagcu cacuuucaug gcugugguuc agucaaccgu 600

guuaccauac ugugugacaa auuuaguggc caucccaaag gguuugcgua uauagaguuc 660

ucagacaaag agucagugag gacuuccuug gccuuagaug agucccuauu uagaggaagg 720

caaaucaagg ugaucccaaa acgaaccaac agaccaggca ucagcacaac agaccggggu 780

uuuccacgag cccgcuaccg cgcccggacc accaacuaca acagcucccg cucucgauuc 840

uacagugguu uuaacagcag gccccggggu cgcgucuaca ggggccgggc uagagcgaca 900

ucaugguauu ccccuuacua a 921