SEQUENCE LISTING

<110> Benitec Biopharma Limited

<120> Reagents for treatment of Oculopharyngeal Muscular Dystrophy (OPMD) and use thereof

<130> 177475

<150> US 62/322,745
<151> 2016-04-14

<160> 29

<170> PatentIn version 3.5

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<400> 2
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<210> 18
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<400> 18
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aacaccgagg agaagatgga ggctgatcaa gagaatcagc ctccatcttc tcctcttttt 360

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agcactttct caactccagt caataactac gttagttgca ttacacattg ggctaatata 540

aatagaggtt aaatctctag gtcatttaag agaagtcggc ctatgtgtac agacatttgt 600

tccaggggct ttaaatagct ggtggtggaa ctcaatattc ggaagaagct gagaagctaa 660

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<213> artificial sequence

<220>
<223> OPMD Triple construct long

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attagaatta atttgactgt aaacacaaag atattagtac aaaatacgtg acgtagaaag 180

taataatttc ttgggtagtt tgcagtttta aaattatgtt ttaaaatgga ctatcatata 240

agatgggctt accgtaactt gaaagtattt cgatttcttg gctttatata tcttgtggaa 300

aggacgaaac accgaggaga agatggaggc tgatcaagag aatcagcctc catcttctcc 360

tctttttac atcaggttgt ttttctgttt ttacatcagg ttgtttttct gtttggtttt 420

ttttttacac cacgtttata cgccggtgca cggtttacca ctgaaaacac ctttcatcta 480

caggtgatat cttttaacac aaataaaatg tagtagtcca tgcgcacgtt taaacaaggg 540

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cgggcggatc acaaggtcag gaggtcgaga ccatccaggc taacacggtg aaacccccc 1440

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tggggtacca cgcgtgagct cctcgag 1527

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<211> 950
<212> DNA
<213> artificial sequence

<220>
<223> Human codon-optimized PABpN1 cDNA sequence (with kozak sequence but without myc-tag)

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gcagacggcg gcatctggtc cctggcgccg gaggggaggc cggcgaaggc gcccctggcg 120

gagccggcga ctacggcaac ggcctggaaa gcgaggaact ggaacccgag gaactgctgc 180

tggaacctga gcccgagcca gagcccgagg aagagccccc taggccaaga gccccccctg 240

gcgccccagg accaggacca ggctctgggg caccaggctc tcaggaagag gaagaagagc 300

ccggcctcgt cgagggagac ccaggcgatg gcgctatcga agatcccgag ctggaagcca 360

tcaaggccag agtgcgggag atggaagagg aggccgaaaa attgaaagag ctgcagaacg 420

aagtcgaaaa acaaatgaac atgtcccccc ctcctggaaa tgctggccct gtgatcatga 480

gcatcgagga aaagatggaa gccgacgccc ggtctatcta cgtgggcaac gtggactacg 540

gcgccaccgc cgaagaactg gaagcccact ttcacggctg tggcagcgtg aaccgggtga 600

ccatcctgtg cgacaagttc agcggccacc ccaagggctt cgcctacatc gagttcagcg 660

acaaagaaag cgtgcggacc tctctggctc tcgacgagtc tctgttcagg ggaaggcaga 720

tcaaggtcat ccccaagcgg accaacaggc ccggcatcag caccaccgac agaggcttcc 780

ctagggctag gtacagagcc cggaccacca actacaacag cagcagaagc cggttctaca 840

gcggcttcaa ttctcggcct agaggcagag tgtaccgggg cagggccagg gccacctcct 900

ggtacagccc ctactgaacc tcctggtaca gcccctactg atgagatatc 950

<210> 25
<211> 306
<212> PRT
<213> artificial sequence

<220>
<223> Human codon-optimized PABpN1 amino acid sequence (without Myc-tag)

<400> 25

Met Ala Ala Ala Ala Ala Ala Ala Ala Ala Ala Gly Ala Ala Gly Gly
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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

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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
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260 265 270

Tyr Asn Ser Ser Arg Ser Arg Phe Tyr Ser Gly Phe Asn Ser Arg Pro
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Arg Gly Arg Val Tyr Arg Gly Arg Ala Arg Ala Thr Ser Trp Tyr Ser
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Pro Tyr
305

<210> 26
<211> 947
<212> DNA
<213> artificial sequence

<220>
<223> Human codon-optimized PABpN1 cDNA sequence (with kozak sequence and Myc-tag)

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gagccggcga ctacggcaac ggcctggaaa gcgaggaact ggaacccgag gaactgctgc 180

tggaacctga gcccgagcca gagcccgagg aagagccccc taggccaaga gccccccctg 240

gcgccccagg accaggacca ggctctgggg caccaggctc tcaggaagag gaagaagagc 300

ccggcctcgt cgagggagac ccaggcgatg gcgctatcga agatcccgag ctggaagcca 360

tcaaggccag agtgcgggag atggaagagg aggccgaaaa attgaaagag ctgcagaacg 420

aagtcgaaaa acaaatgaac atgtcccccc ctcctggaaa tgctggccct gtgatcatga 480

gcatcgagga aaagatggaa gccgacgccc ggtctatcta cgtgggcaac gtggactacg 540

gcgccaccgc cgaagaactg gaagcccact ttcacggctg tggcagcgtg aaccgggtga 600

ccatcctgtg cgacaagttc agcggccacc ccaagggctt cgcctacatc gagttcagcg 660

acaaagaaag cgtgcggacc tctctggctc tcgacgagtc tctgttcagg ggaaggcaga 720

tcaaggtcat ccccaagcgg accaacaggc ccggcatcag caccaccgac agaggcttcc 780

ctagggctag gtacagagcc cggaccacca actacaacag cagcagaagc cggttctaca 840

gcggcttcaa ttctcggcct agaggcagag tgtaccgggg cagggccagg gccacctcct 900

ggtacagccc ctacgaacag aagctgatca gcgaggaaga tctgtga 947

<210> 27
<211> 316
<212> PRT
<213> artificial sequence

<220>
<223> Human codon-optimized PABpN1 amino acid sequence (with Myc-tag)

<400> 27

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

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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
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Pro Tyr Glu Gln Lys Leu Ile Ser Glu Glu Asp Leu
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gggggcgcag gggactacgg gaacggcctg gagtctgagg aactggagcc tgaggagctg      180
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ccgggagctc cgggccctgg gcctggttcg ggagcccccg gcagccaaga ggaggaggag 300

gagccgggac tggtcgaggg tgacccgggg gacggcgcca ttgaggaccc ggagctggaa 360

gctatcaaag ctcgagtcag ggagatggag gaagaagctg agaagctaaa ggagctacag 420

aacgaggtag agaagcagat gaatatgagt ccacctccag gcaatgctgg cccggtgatc 480

atgtccattg aggagaagat ggaggctgat gcccgttcca tctatgttgg caatgtggac 540

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<211> 314
<212> PRT
<213> artificial sequence

<220>
<223> Mutant human PABpN1 amino acid sequence (with FLAG tag)

<400> 29

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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

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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
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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
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