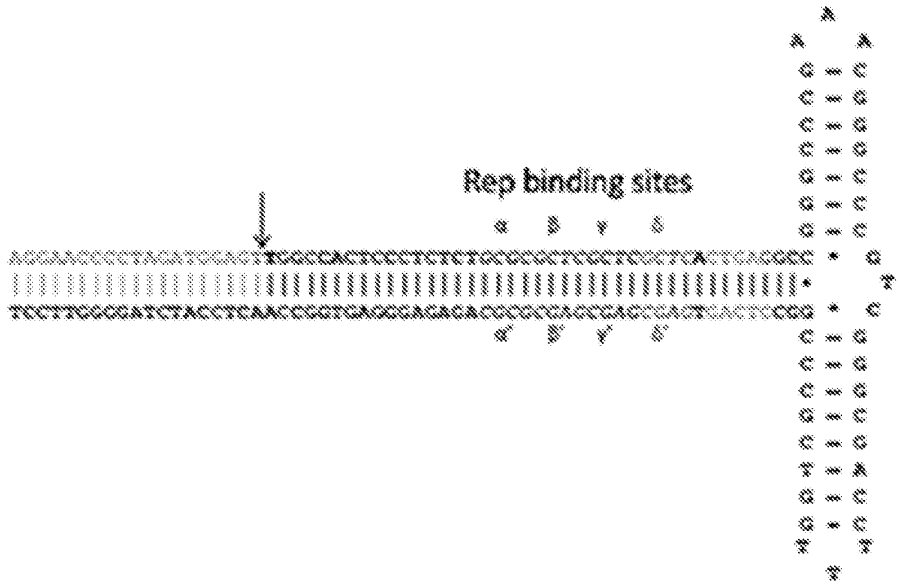
FIG. 1A



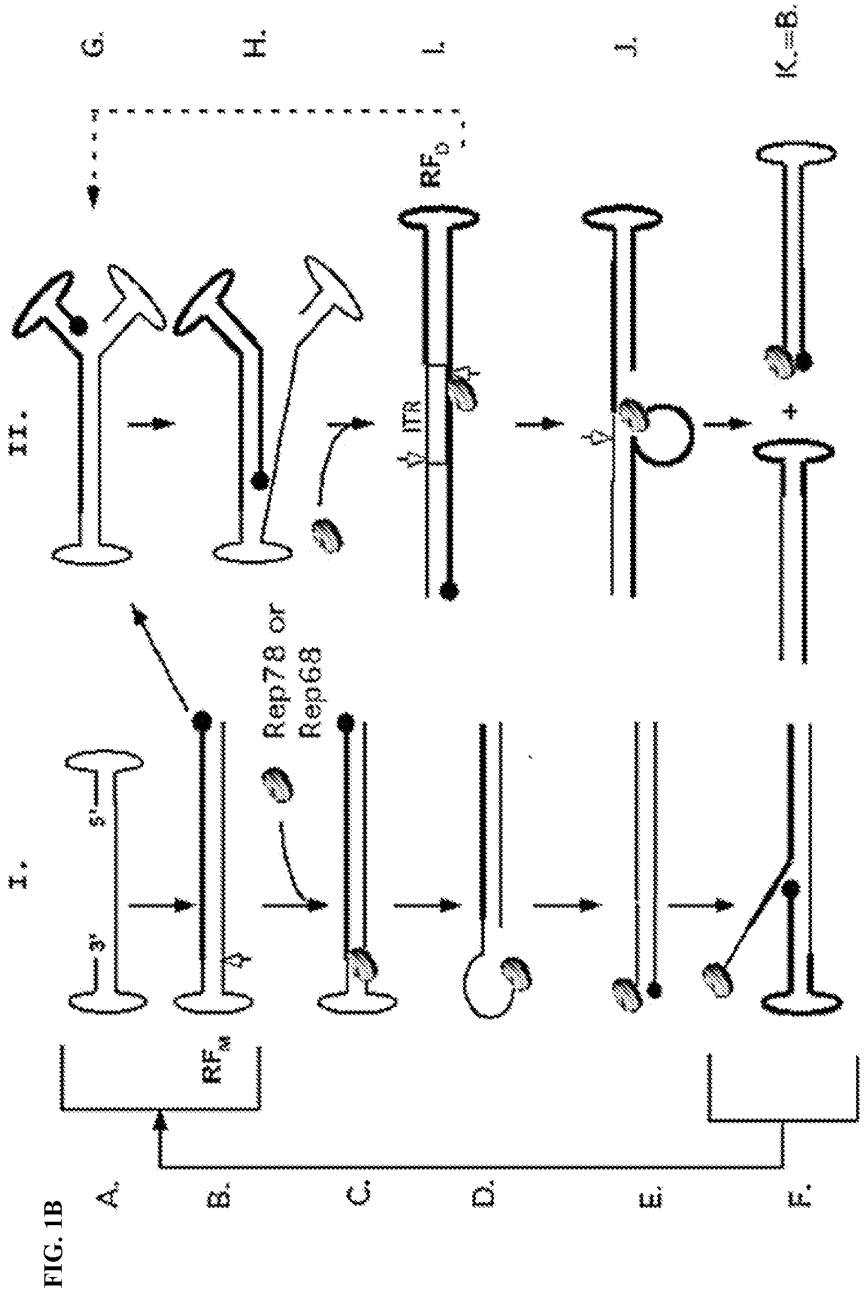


FIG. 1B

FIG. 1C

```
AAV3    GCCATACCTCTAGTGATGGAGTTGGCCACTCCCTCTATGCGCACTCGCTCGCTCGGTGGG   60
AAV4    GGGCAAACCTAGATGATGGAGTTGGCCACTCCCTCTATGCGCTCGCTCGCTCACTCACTCGG   60
AAV2    --AGGAACCCCTAGTGATGGAGTTGGCCACTCCCTCTCTGCGCGCTCGCTCACTGAG       59
AAV7    --CGGTACCCCTAGTGAGGGAGTTGGGCCACTCCCTCTATGCGCGCTCGCTCGCTCGGTGGG   59
AAV1    ----------------------------TTGCCCACTCCCTCTCTGCGCGCTCGCTCGCTGGTGGG   39
AAV6    ----------------------------TTGCCCACTCCCTCTATGCGCGCTCGCTCGCTGGTGGG   39
                                     *** ********** ****** ******* ***  *  *

AAV3    GCCGGACGTGCAAACGACGTCCGTCTGGCGACCTTTGGTCGCCAGGCCCCACCGAGCGAG   120
AAV4    CCCTGGAGACCAAAGGTCTCCAGACTGCCGGCTCTGGCCGGCAGGGCCGAGTGAGTGAG   120
AAV2    GCCGGGCGACCAAAGGTCGCCCGACGCCCGGGCTTTGCCCGGGCGGCCTCAGTgagcgag   119
AAV7    GCCTGCGGACCAAAGGTCCGCAGACGGCAGAGCTCTGCTCTGCCGGCCCCACCGAGCGAG   119
AAV1    GCCTGCGGACCAAAGGTCCTGCAGACGGCAGAGCTCTGCTCTGCCGGCCCCACCGAGCGAG   99
AAV6    GCCTGCGGACCAAAGGTCCGGCAGACGGCAGAGCTCTGCTCTGCCGGCCCCACCGAGCGAG   99
          ** *  *  ***** *  * * *  *  ** **  *   ** *  *  * *** ***

AAV3    CGAGTGCGCATAGAGGGAGTGGCCAA----------------------------------   146
AAV4    CGAGCGCGCATAGAGGGAGTGGCCAA----------------------------------   146
AAV2    cgagcgcgcAG------------------------------------------------   130
AAV7    CGAGCGCGCATAGAGGGAGTGGCCAACTCCATCACTAGGGGTACCG               165
AAV1    CGAGCGCGCAGAGAGGGAGTGGGCAACTCCATCACTAGGGGTAA----             143
AAV6    CGAGCGCGCATAGAGGGAGTGGGCAACTCCATCACTAGGGGTAA----             143
```

FIG. 2

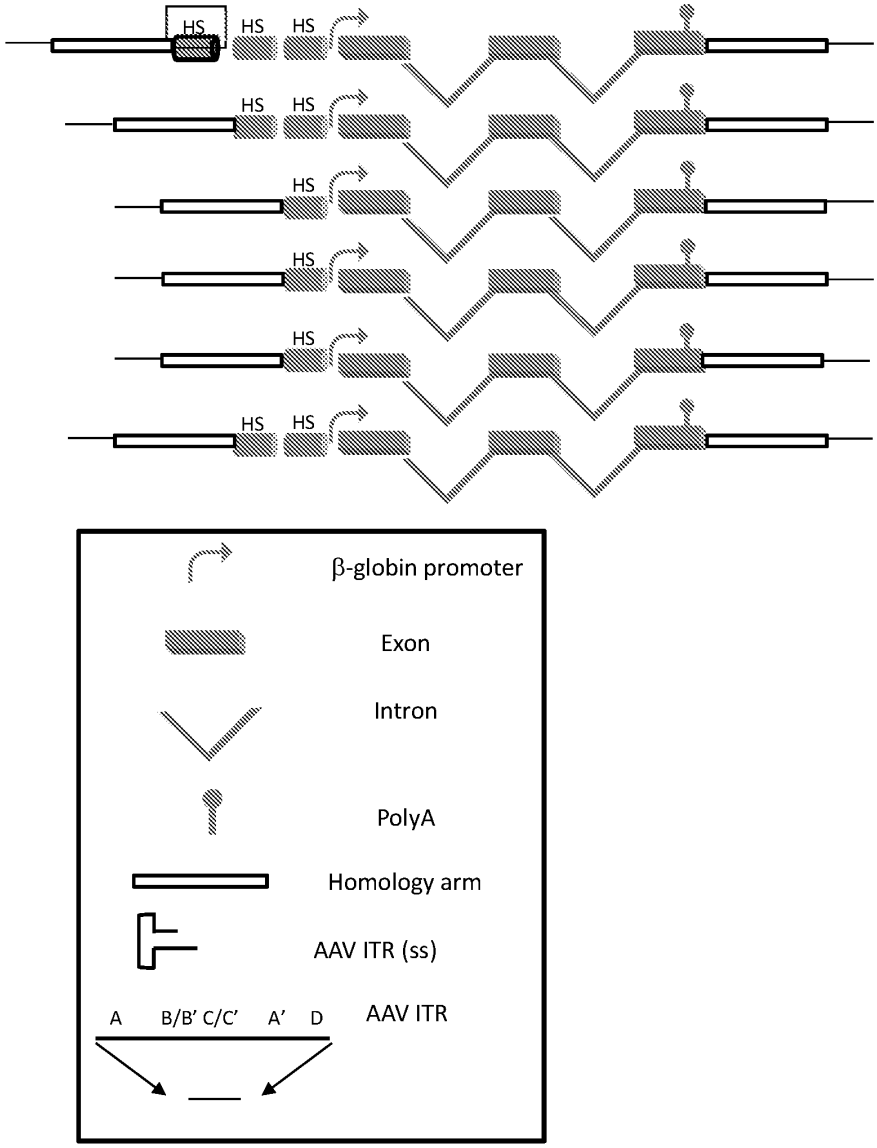

HS
HS
HS
HS
HS
HS
HS
HS
HS
HS
HS
HS
β-globin promoter
Exon
Intron
PolyA
Homology arm
AAV ITR (ss)
AAV ITR
A
B/B' C/C'
A'
D

FIG. 3

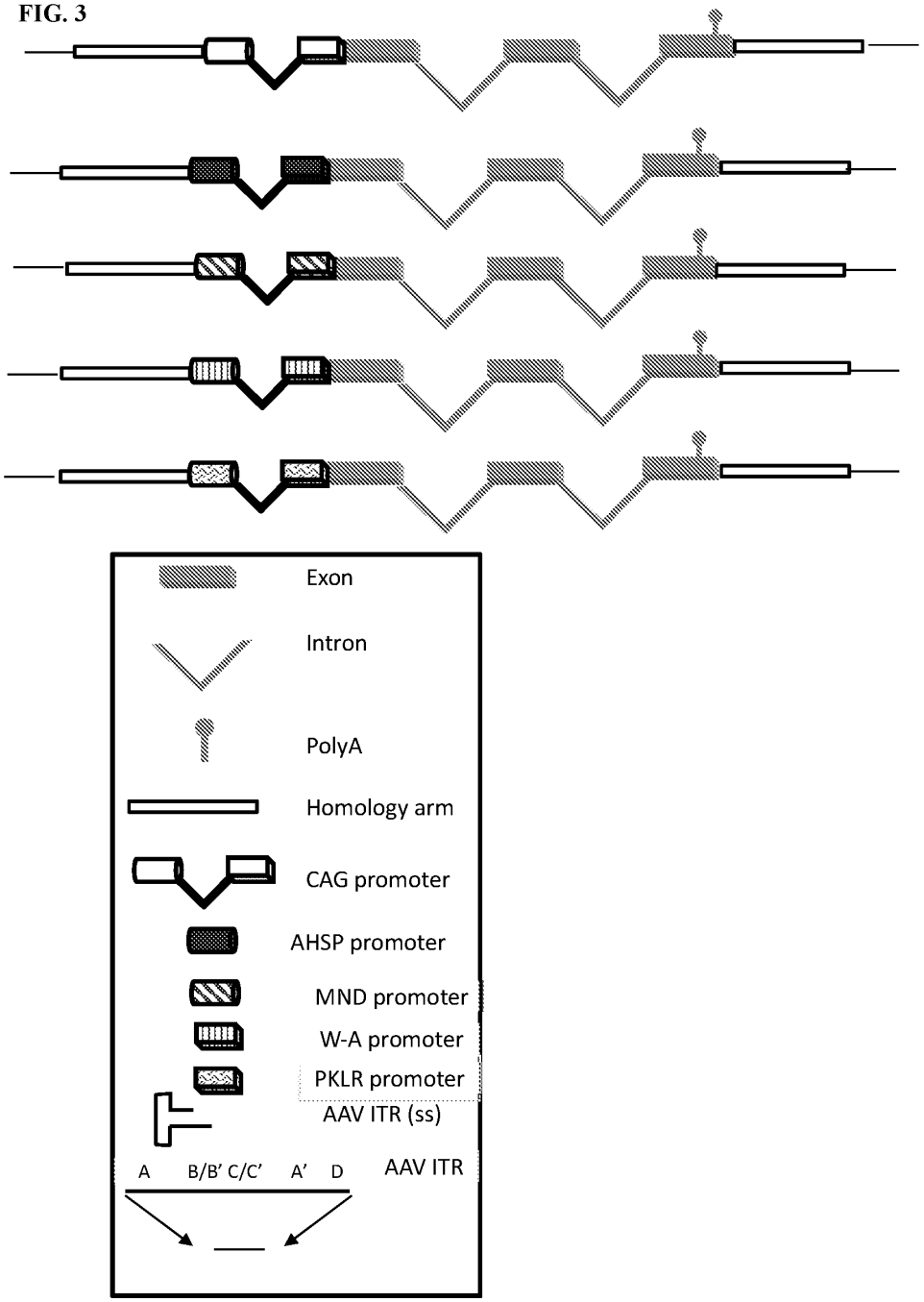

Exon
Intron
PolyA
Homology arm
CAG promoter
AHSP promoter
MND promoter
W-A promoter
PKLR promoter
AAV ITR (ss)
A
B/B' C/C'
A'
D
AAV ITR

FIG. 4

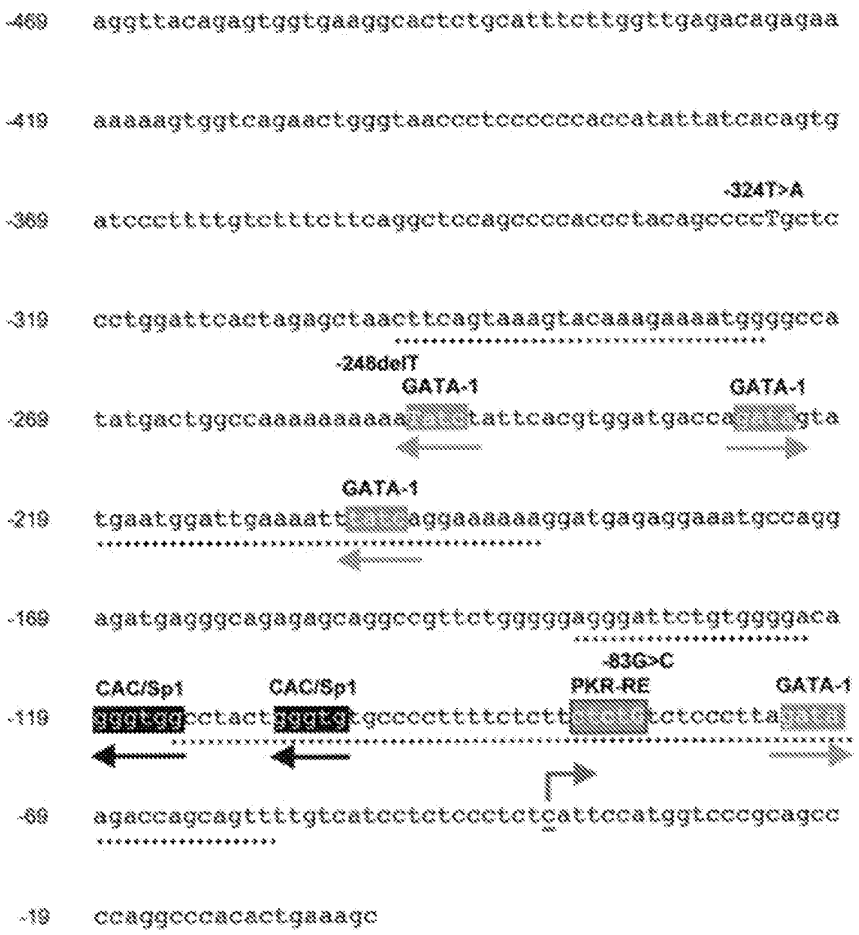

-469
aggttacagagtggtgaaggcactctgcatttcttggttgagacagagaa
-419
aaaaagtggtcagaactgggtaaccctcccccaccatattatcacagtg
-324T>A
-369
atccctttgtctttcttcaggctccagccccaccctacagccccTgctc
-319
cctggattcactagagctaacttcagtaaagtacaaagaaaatggggcca
-248delT
GATA-1
GATA-1
-269
tatgactggccaaaaaaaaaaa tattcacgtggatgacca gta
GATA-1
-219
tgaatggattgaaaatt aggaaaaaaggatgagaggaaatgccagg
-169
agatgagggcagagagcaggccgttctgggggagggattctgtggggaca
-83G>C
CAC/Sp1
CAC/Sp1
PKR-RE
GATA-1
-119
cctact gcccctttctctt ctccctta
-69
agaccagcagttttgtcatcctctccctctcattccatggtcccgcagcc
-19
ccaggcccacactgaaagc

FIG. 5A

Category	Exemplary nucleic acid of interest
miRNA genes, and their homologues as nucleic acid of interest	hsa-let-7a, hsa-let-7a', hsa-let-7b, hsa-let-7b', hsa-let-7c, hsa-let-7c', hsa-let-7d, hsa-let-7d', hsa-let-7e, hsa-let-7e', hsa-let-7f-1, hsa-let-7f-1', hsa-let-7f-2, hsa-let-7g, hsa-let-7g', hsa-let-7i, hsa-let-7i', hsa-miR-1, hsa-miR-100, hsa-miR-100', hsa-miR-101, hsa-miR-101', hsa-miR-103, hsa-miR-105, hsa-miR-105', hsa-miR-106a, hsa-miR-106b, hsa-miR-106b', hsa-miR-107, hsa-miR-10a, hsa-miR-10a', hsa-miR-10b, hsa-miR-10b', hsa-miR-1178, hsa-miR-1179, hsa-miR-1180, hsa-miR-1181, hsa-miR-1182, hsa-miR-1183, hsa-miR-1184, hsa-miR-1185, hsa-miR-1197, hsa-miR-1200, hsa-miR-1201, hsa-miR-1202, hsa-miR-1203, hsa-miR-1204, hsa-miR-1205, hsa-miR-1206, hsa-miR-1207-5p, hsa-miR-1208, hsa-miR-122, hsa-miR-122', hsa-miR-1224-3p, hsa-miR-1224-5p, hsa-miR-1225-3p, hsa-miR-1226, hsa-miR-1226', hsa-miR-1227, hsa-miR-1228, hsa-miR-1228', hsa-miR-1229, hsa-miR-1231, hsa-miR-1234, hsa-miR-1236, hsa-miR-1237, hsa-miR-1238, hsa-miR-124, hsa-miR-124', hsa-miR-1243, hsa-miR-1244, hsa-miR-1245, hsa-miR-1246, hsa-miR-1247, hsa-miR-1248, hsa-miR-1249, hsa-miR-1250, hsa-miR-1251, hsa-miR-1252, hsa-miR-1253, hsa-miR-1254, hsa-miR-1255a, hsa-miR-1255b, hsa-miR-1257, hsa-miR-1258, hsa-miR-1259, hsa-miR-125a-3p, hsa-miR-125a-5p, hsa-miR-125b, hsa-miR-125b-1', hsa-miR-125b-2', hsa-miR-126, hsa-miR-126', hsa-miR-1260, hsa-miR-1261, hsa-miR-1262, hsa-miR-1263, hsa-miR-1266, hsa-miR-1267, hsa-miR-1268, hsa-miR-1269, hsa-miR-1270, hsa-miR-1271, hsa-miR-1272, hsa-miR-1273, hsa-miR-1537-3p, hsa-miR-1274a, hsa-miR-1274b, hsa-miR-1275, hsa-miR-1277-5p, hsa-miR-1276, hsa-miR-1277, hsa-miR-1278, hsa-miR-1279, hsa-miR-128, hsa-miR-1280, hsa-miR-1281, hsa-miR-1282, hsa-miR-1283, hsa-miR-1284, hsa-miR-1285, hsa-miR-1287, hsa-miR-1288, hsa-miR-129', hsa-miR-1290, hsa-miR-1291, hsa-miR-1292, hsa-miR-1293, hsa-miR-129-3p, hsa-miR-1294, hsa-miR-1295, hsa-miR-1296-5p, hsa-miR-1296, hsa-miR-1297, hsa-miR-1298, hsa-miR-1299, hsa-miR-1300, hsa-miR-1301, hsa-miR-1302, hsa-miR-1303, hsa-miR-1304, hsa-miR-1305, hsa-miR-1306, hsa-miR-1307, hsa-miR-1308, hsa-miR-130a, hsa-miR-130b', hsa-miR-130b, hsa-miR-130b', hsa-miR-132', hsa-miR-1321, hsa-miR-1322, hsa-miR-1323, hsa-miR-1324, hsa-miR-133a, hsa-miR-133b, hsa-miR-134, hsa-miR-1343, hsa-miR-135a, hsa-miR-135b, hsa-miR-1362', hsa-miR-136, hsa-miR-136', hsa-miR-137, hsa-miR-138, hsa-miR-138-1', hsa-miR-138-2', hsa-miR-139-3p, hsa-miR-140-3p, hsa-miR-140-5p, hsa-miR-141, hsa-miR-141', hsa-miR-4423-3p, hsa-miR-142-5p, hsa-miR-143, hsa-miR-143', hsa-miR-144, hsa-miR-144', hsa-miR-145, hsa-miR-145', hsa-miR-146a, hsa-miR-146a', hsa-miR-146b-3p, hsa-miR-146b-5p, hsa-miR-147, hsa-miR-147b, hsa-miR-148a, hsa-miR-148a', hsa-miR-148b, hsa-miR-148b', hsa-miR-149, hsa-miR-149', hsa-miR-150, hsa-miR-150', hsa-

FIG. 5A, Cont.

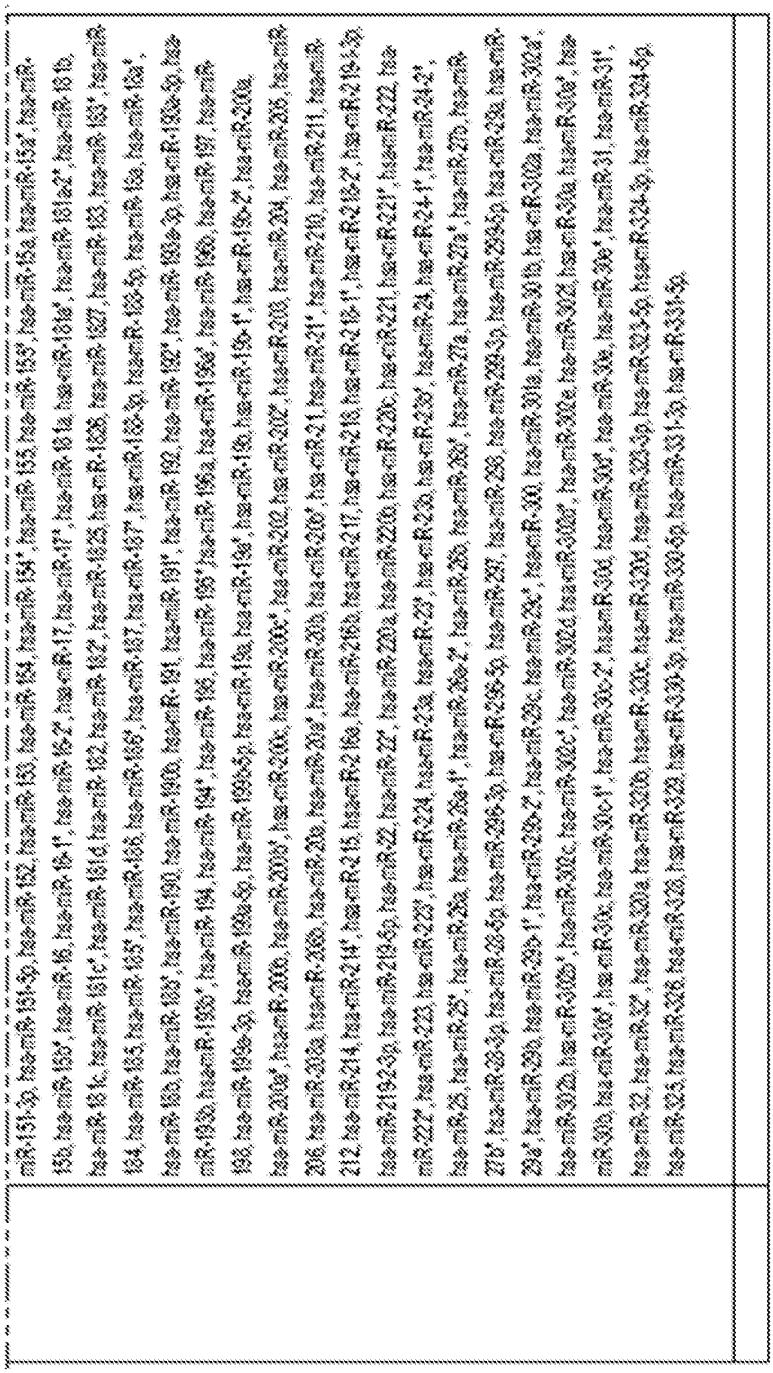
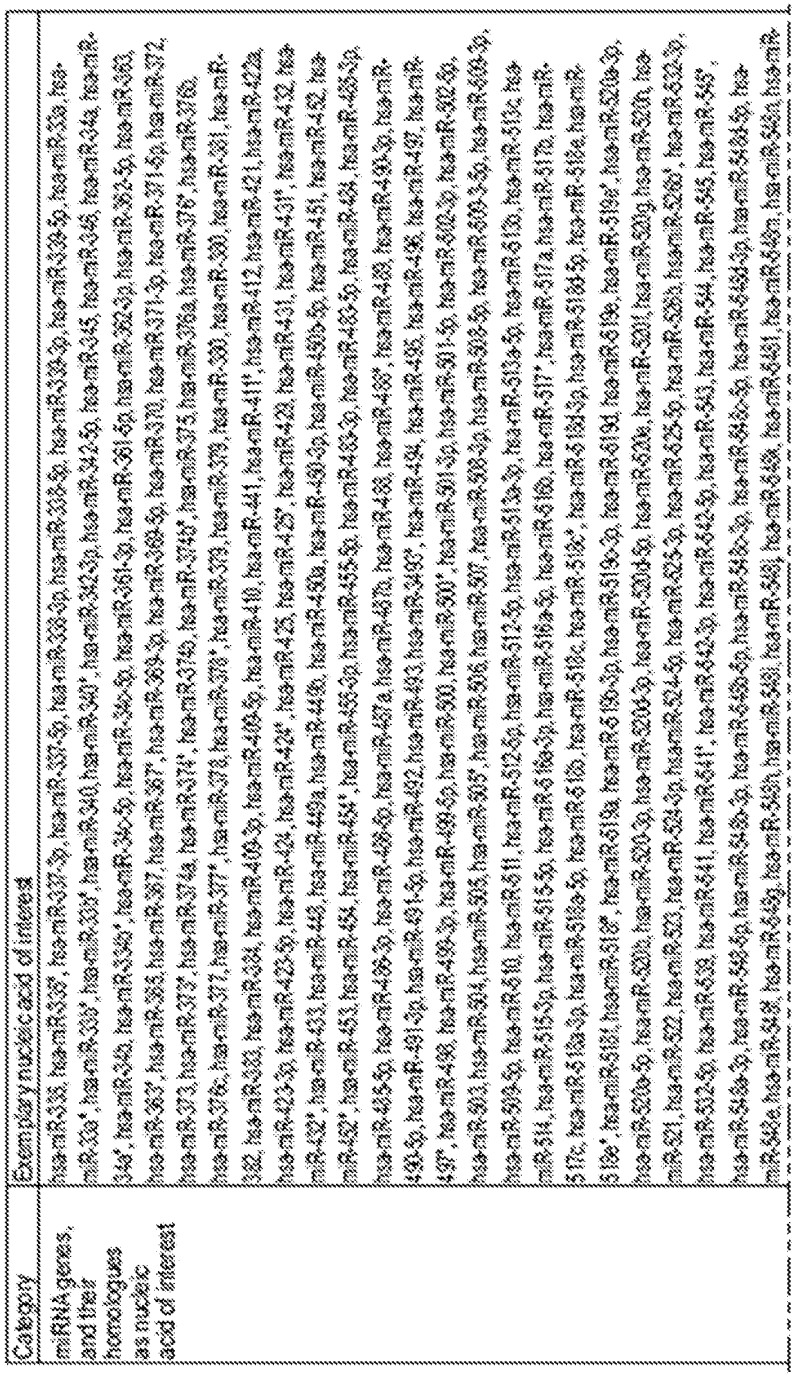

miR-151-3p, hsa-miR-181-3p, hsa-miR-182, hsa-miR-183, hsa-miR-184, hsa-miR-194', hsa-miR-155, hsa-miR-153', hsa-miR-15a, hsa-miR-15a', hsa-miR-15b, hsa-miR-16', hsa-miR-16, hsa-miR-16-1', hsa-miR-16-2', hsa-miR-17, hsa-miR-17', hsa-miR-181a, hsa-miR-181a', hsa-miR-181a-2', hsa-miR-181b, hsa-miR-181b-1, hsa-miR-181c', hsa-miR-181d, hsa-miR-182, hsa-miR-182', hsa-miR-1825, hsa-miR-1826, hsa-miR-1827, hsa-miR-183, hsa-miR-183', hsa-miR-184, hsa-miR-185, hsa-miR-185', hsa-miR-186, hsa-miR-187, hsa-miR-187', hsa-miR-188-3p, hsa-miR-188-5p, hsa-miR-18a, hsa-miR-18a', hsa-miR-18b, hsa-miR-1908', hsa-miR-190, hsa-miR-190b, hsa-miR-191, hsa-miR-191', hsa-miR-192, hsa-miR-192', hsa-miR-193a-3p, hsa-miR-193a-5p, hsa-miR-193b', hsa-miR-194, hsa-miR-194', hsa-miR-195, hsa-miR-195', hsa-miR-196a, hsa-miR-1908', hsa-miR-197, hsa-miR-198, hsa-miR-199a-3p, hsa-miR-199a-5p, hsa-miR-199b-3p, hsa-miR-19a, hsa-miR-19b, hsa-miR-19b-1', hsa-miR-19b-2', hsa-miR-200a, hsa-miR-200b', hsa-miR-200b, hsa-miR-200c, hsa-miR-200c', hsa-miR-202, hsa-miR-202', hsa-miR-203, hsa-miR-204, hsa-miR-205, hsa-miR-206, hsa-miR-208a, hsa-miR-20b, hsa-miR-20b', hsa-miR-20b-3p, hsa-miR-210', hsa-miR-21, hsa-miR-21', hsa-miR-210, hsa-miR-211, hsa-miR-212, hsa-miR-214, hsa-miR-214', hsa-miR-215, hsa-miR-216a, hsa-miR-216a-3p, hsa-miR-217, hsa-miR-218, hsa-miR-218-1', hsa-miR-218-2', hsa-miR-219-1-3p, hsa-miR-2192-3p, hsa-miR-219-5p, hsa-miR-22, hsa-miR-22', hsa-miR-220a, hsa-miR-220b, hsa-miR-220c, hsa-miR-221, hsa-miR-221', hsa-miR-222, hsa-miR-222', hsa-miR-223, hsa-miR-223', hsa-miR-224, hsa-miR-23a, hsa-miR-23b, hsa-miR-23b', hsa-miR-24, hsa-miR-24-1', hsa-miR-24-2', hsa-miR-25, hsa-miR-25', hsa-miR-26a, hsa-miR-26a-1', hsa-miR-26a-2', hsa-miR-26b, hsa-miR-26b', hsa-miR-27a, hsa-miR-27a', hsa-miR-27b, hsa-miR-27b', hsa-miR-28-3p, hsa-miR-28-5p, hsa-miR-296-3p, hsa-miR-296-5p, hsa-miR-297, hsa-miR-298, hsa-miR-299-3p, hsa-miR-299-5p, hsa-miR-29a, hsa-miR-29b, hsa-miR-29b-1', hsa-miR-29b-2', hsa-miR-29c', hsa-miR-300, hsa-miR-301a, hsa-miR-301b, hsa-miR-302a, hsa-miR-302a', hsa-miR-302b, hsa-miR-302b', hsa-miR-302c, hsa-miR-302c', hsa-miR-302d, hsa-miR-302d', hsa-miR-302e, hsa-miR-302f, hsa-miR-30a, hsa-miR-30a', hsa-miR-30b, hsa-miR-30b', hsa-miR-30c, hsa-miR-30c-1', hsa-miR-30c-2', hsa-miR-30d, hsa-miR-30d', hsa-miR-30e, hsa-miR-30e', hsa-miR-31, hsa-miR-31', hsa-miR-32, hsa-miR-32', hsa-miR-320a, hsa-miR-320b, hsa-miR-320c, hsa-miR-320d, hsa-miR-323-3p, hsa-miR-323-5p, hsa-miR-324-3p, hsa-miR-324-5p, hsa-miR-326, hsa-miR-328, hsa-miR-329, hsa-miR-330-3p, hsa-miR-330-5p, hsa-miR-331-3p, hsa-miR-331-5p,

FIG. 5B



Category	Exemplary nucleic acid of interest
miRNA genes, and their homologues as nucleic acid of interest	hsa-miR-338, hsa-miR-338', hsa-miR-337-3p, hsa-miR-337-5p, hsa-miR-338-3p, hsa-miR-338-5p, hsa-miR-339-3p, hsa-miR-339-5p, hsa-miR-33a, hsa-miR-33b', hsa-miR-33b', hsa-miR-340, hsa-miR-340', hsa-miR-342-3p, hsa-miR-342-5p, hsa-miR-345, hsa-miR-346, hsa-miR-34a, hsa-miR-34b', hsa-miR-34b, hsa-miR-34b', hsa-miR-34c-5p, hsa-miR-361-5p, hsa-miR-361-3p, hsa-miR-362-3p, hsa-miR-362-5p, hsa-miR-363, hsa-miR-367, hsa-miR-368, hsa-miR-367, hsa-miR-369-3p, hsa-miR-370, hsa-miR-371-3p, hsa-miR-371-5p, hsa-miR-372, hsa-miR-373, hsa-miR-373', hsa-miR-374a, hsa-miR-374', hsa-miR-374b, hsa-miR-375, hsa-miR-376a, hsa-miR-376', hsa-miR-376b, hsa-miR-376c, hsa-miR-377, hsa-miR-377', hsa-miR-378, hsa-miR-378', hsa-miR-379, hsa-miR-380, hsa-miR-381, hsa-miR-382, hsa-miR-383, hsa-miR-409-3p, hsa-miR-409-5p, hsa-miR-410, hsa-miR-411, hsa-miR-411', hsa-miR-412, hsa-miR-421, hsa-miR-422a, hsa-miR-423-3p, hsa-miR-423-5p, hsa-miR-424, hsa-miR-424', 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hsa-miR-623, hsa-miR-624-3p, hsa-miR-624-5p, hsa-miR-625-3p, hsa-miR-625-5p, hsa-miR-626b', hsa-miR-632-3p, hsa-miR-632-5p, hsa-miR-639, hsa-miR-641', hsa-miR-642-3p, hsa-miR-642-5p, hsa-miR-643, hsa-miR-644, hsa-miR-645, hsa-miR-645

FIG. 5B, Cont.

	542a, hsa-miR-548p, hsa-miR-548, hsa-miR-331-5p, hsa-miR-551b', hsa-miR-551a, hsa-miR-551b, hsa-miR-552, hsa-miR-553, hsa-miR-554, hsa-miR-555, hsa-miR-556-3p, hsa-miR-556-5p, hsa-miR-557, hsa-miR-558, hsa-miR-559, hsa-miR-561, hsa-miR-562, hsa-miR-563, hsa-miR-564, hsa-miR-566, hsa-miR-567, hsa-miR-568, hsa-miR-569, hsa-miR-570, hsa-miR-571, hsa-miR-572, hsa-miR-573, hsa-miR-574-3p, hsa-miR-574-5p, hsa-miR-576-3p, hsa-miR-576-5p, hsa-miR-577, hsa-miR-578, hsa-miR-579, hsa-miR-580, hsa-miR-581, hsa-miR-582-3p, hsa-miR-582-5p, hsa-miR-583, hsa-miR-584, hsa-miR-585, hsa-miR-587, hsa-miR-588, hsa-miR-589, hsa-miR-589-3p, hsa-miR-591-5p, hsa-miR-591, hsa-miR-592, hsa-miR-593, hsa-miR-597, hsa-miR-596, hsa-miR-598, hsa-miR-599, hsa-miR-600, hsa-miR-601, hsa-miR-602, hsa-miR-603, hsa-miR-604, hsa-miR-605, hsa-miR-606, hsa-miR-607, hsa-miR-608, hsa-miR-609, hsa-miR-610, hsa-miR-611, hsa-miR-612, hsa-miR-613, hsa-miR-614, hsa-miR-615-3p, hsa-miR-615-5p, hsa-miR-616, hsa-miR-616', hsa-miR-617, hsa-miR-618, hsa-miR-619, hsa-miR-620, hsa-miR-621, hsa-miR-622, hsa-miR-623, hsa-miR-624, hsa-miR-624', hsa-miR-625, hsa-miR-625', hsa-miR-626, hsa-miR-627, hsa-miR-628-3p, hsa-miR-628-5p, hsa-miR-629, hsa-miR-629', hsa-miR-630, hsa-miR-631, hsa-miR-632, hsa-miR-633, hsa-miR-634, hsa-miR-635, hsa-miR-636, hsa-miR-637, hsa-miR-638, hsa-miR-639, hsa-miR-640, hsa-miR-641, hsa-miR-642, hsa-miR-643, hsa-miR-644, hsa-miR-645, hsa-miR-646, hsa-miR-647, hsa-miR-648, hsa-miR-649, hsa-miR-650, hsa-miR-651, hsa-miR-652, hsa-miR-653, hsa-miR-654-3p, hsa-miR-654-5p, hsa-miR-655, hsa-miR-656, hsa-miR-657, hsa-miR-658, hsa-miR-659, hsa-miR-660, hsa-miR-661, hsa-miR-662, hsa-miR-663, hsa-miR-663b, hsa-miR-664, hsa-miR-664', hsa-miR-665, hsa-miR-671-3p, hsa-miR-671-5p, hsa-miR-675, hsa-miR-7-1, hsa-miR-708, hsa-miR-708', hsa-miR-7-1', hsa-miR-7-2', hsa-miR-720, hsa-miR-744, hsa-miR-744', hsa-miR-758, hsa-miR-760, hsa-miR-765, hsa-miR-766, hsa-miR-767-3p, hsa-miR-767-5p, hsa-miR-769-3p, hsa-miR-769-5p, hsa-miR-770-5p, hsa-miR-802, hsa-miR-873, hsa-miR-874, hsa-miR-875-3p, hsa-miR-875-5p, hsa-miR-876-3p, hsa-miR-876-5p, hsa-miR-877, hsa-miR-877', hsa-miR-885-3p, hsa-miR-885-5p, hsa-miR-886-3p, hsa-miR-886-5p, hsa-miR-887, hsa-miR-888, hsa-miR-888', hsa-miR-889, hsa-miR-890, hsa-miR-891a, hsa-miR-891b, hsa-miR-892a, hsa-miR-892b, hsa-miR-9, hsa-miR-9', hsa-miR-920, hsa-miR-921, hsa-miR-922, hsa-miR-923, hsa-miR-924, hsa-miR-92a, hsa-miR-92a-1', hsa-miR-92a-2*, hsa-miR-92b, hsa-miR-92b', hsa-miR-93, hsa-miR-93', hsa-miR-933, hsa-miR-934, hsa-miR-935, hsa-miR-936, hsa-miR-937, hsa-miR-938, hsa-miR-939, hsa-miR-940, hsa-miR-941, hsa-miR-942, hsa-miR-943, hsa-miR-944, hsa-miR-95, hsa-miR-96, hsa-miR-96', hsa-miR-98, hsa-miR-99a, hsa-miR-99a', hsa-miR-99b, hsa-miR-99b'.

FIG. 6

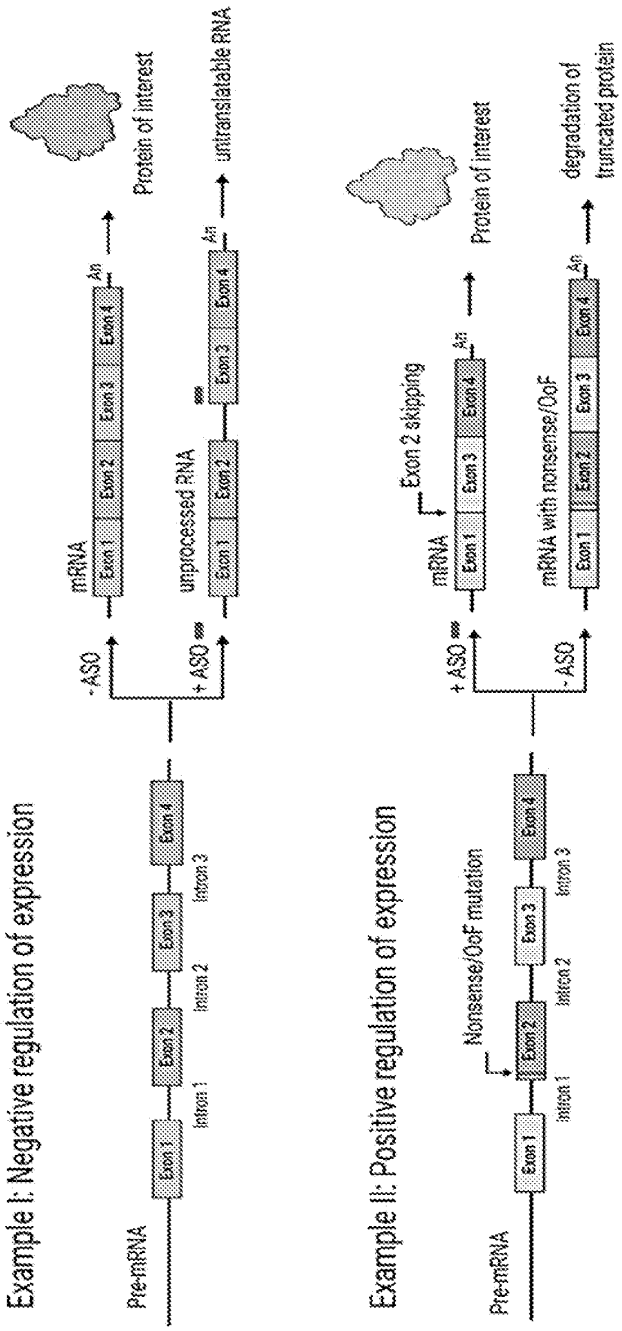
Example I: Negative regulation of expression
Pre-mRNA
Exon 1
Exon 2
Exon 3
Exon 4
Intron 1
Intron 2
Intron 3
- ASO
+ ASO
mRNA
unprocessed RNA
Protein of interest
untranslatable RNA
Example II: Positive regulation of expression
Nonsense/OoF mutation
Exon 2 skipping
mRNA with nonsense/OoF
degradation of truncated protein

FIG. 7

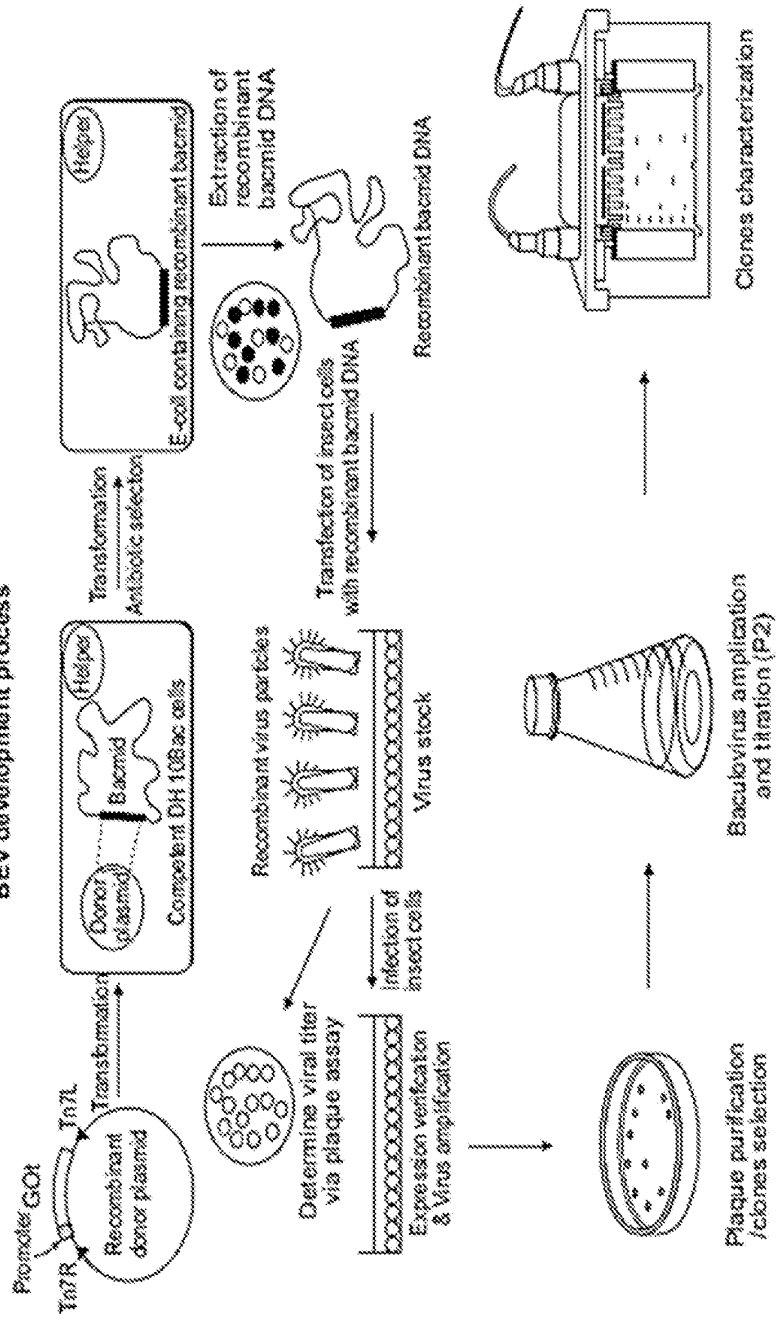
BEV development process
Promoter
GOI
Tn7R
Tn7L
Transformation
Recombinant donor plasmid
Transformation
Antibiotic selection
Donor plasmid
Bacmid
Helper
Competent DH 10Bac cells
E.coli containing recombinant bacmid
Extraction of recombinant bacmid DNA
Recombinant bacmid DNA
Transfection of insect cells with recombinant bacmid DNA
Recombinant virus particles
Virus stock
Determine viral titer via plaque assay
Infection of insect cells
Expression verification & Virus amplification
Plaque purification /clones selection
Baculovirus amplication and titration (P2)
Clones characterization

FIG. 8A

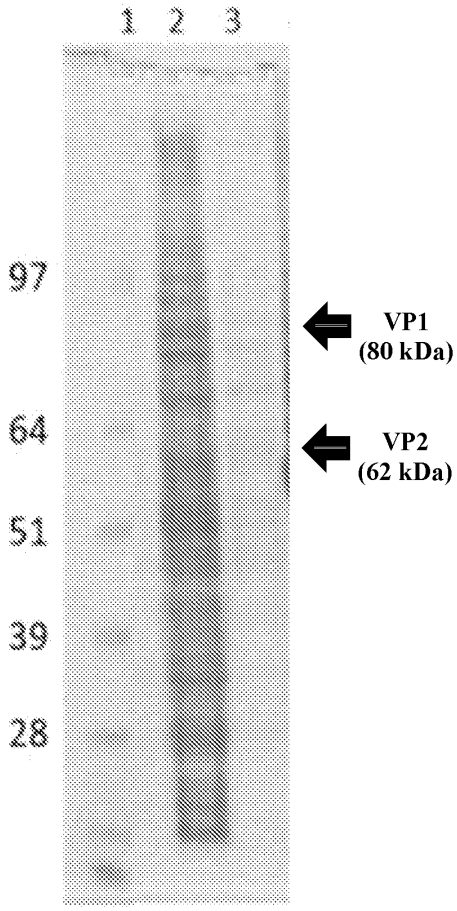
1 2 3
97
64
51
39
28
VP1
(80 kDa)
VP2
(62 kDa)

FIG. 8B

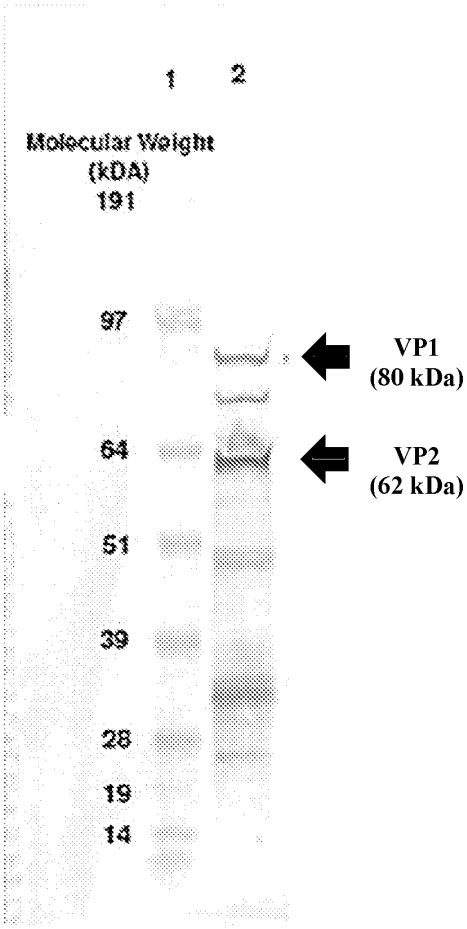
1 2
Molecular Weight
(kDA)
191
97
64
51
39
28
19
14
VP1
(80 kDa)
VP2
(62 kDa)

FIG. 9A

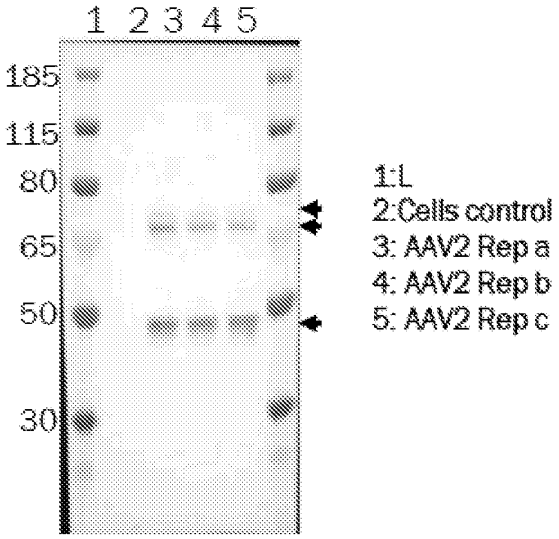

FIG. 9B
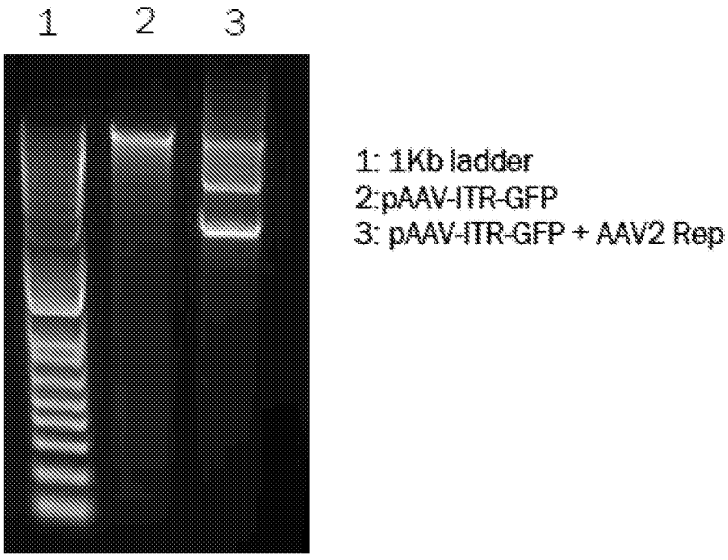
FIG. 9C