

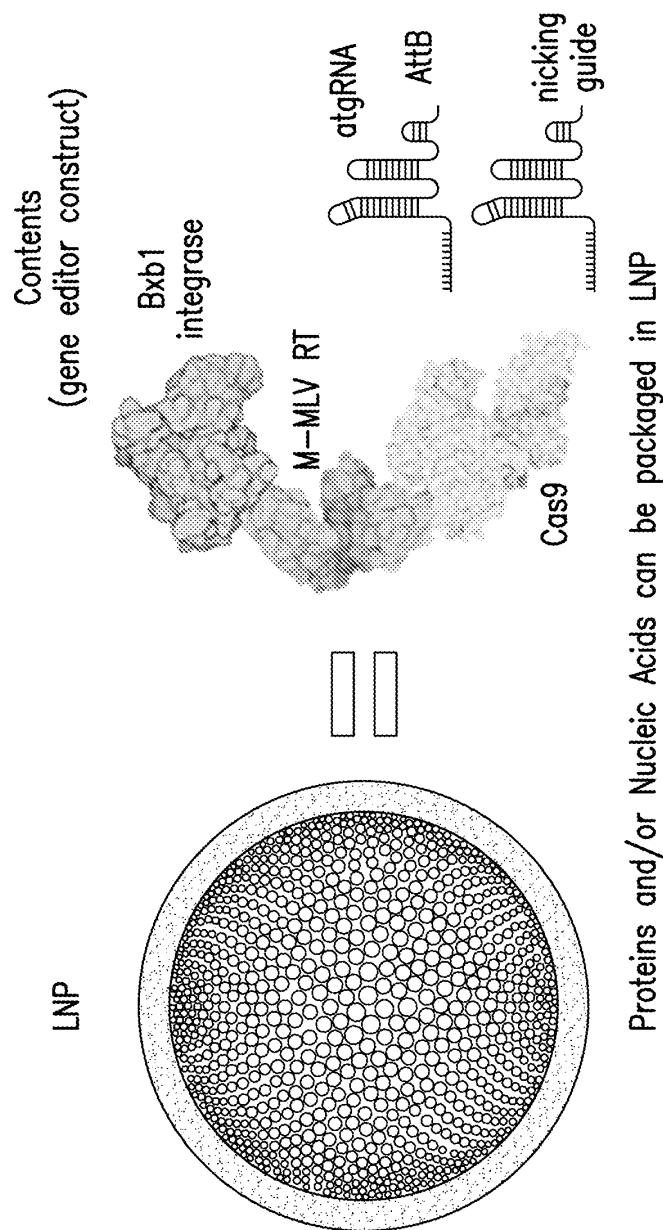


FIG.1

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Template Vector

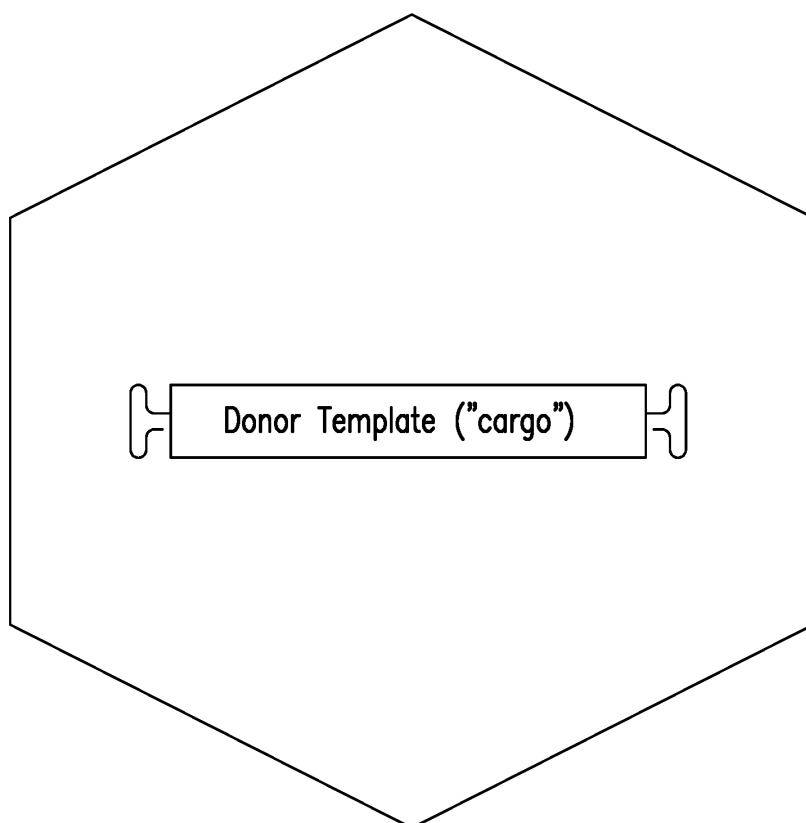


FIG.2

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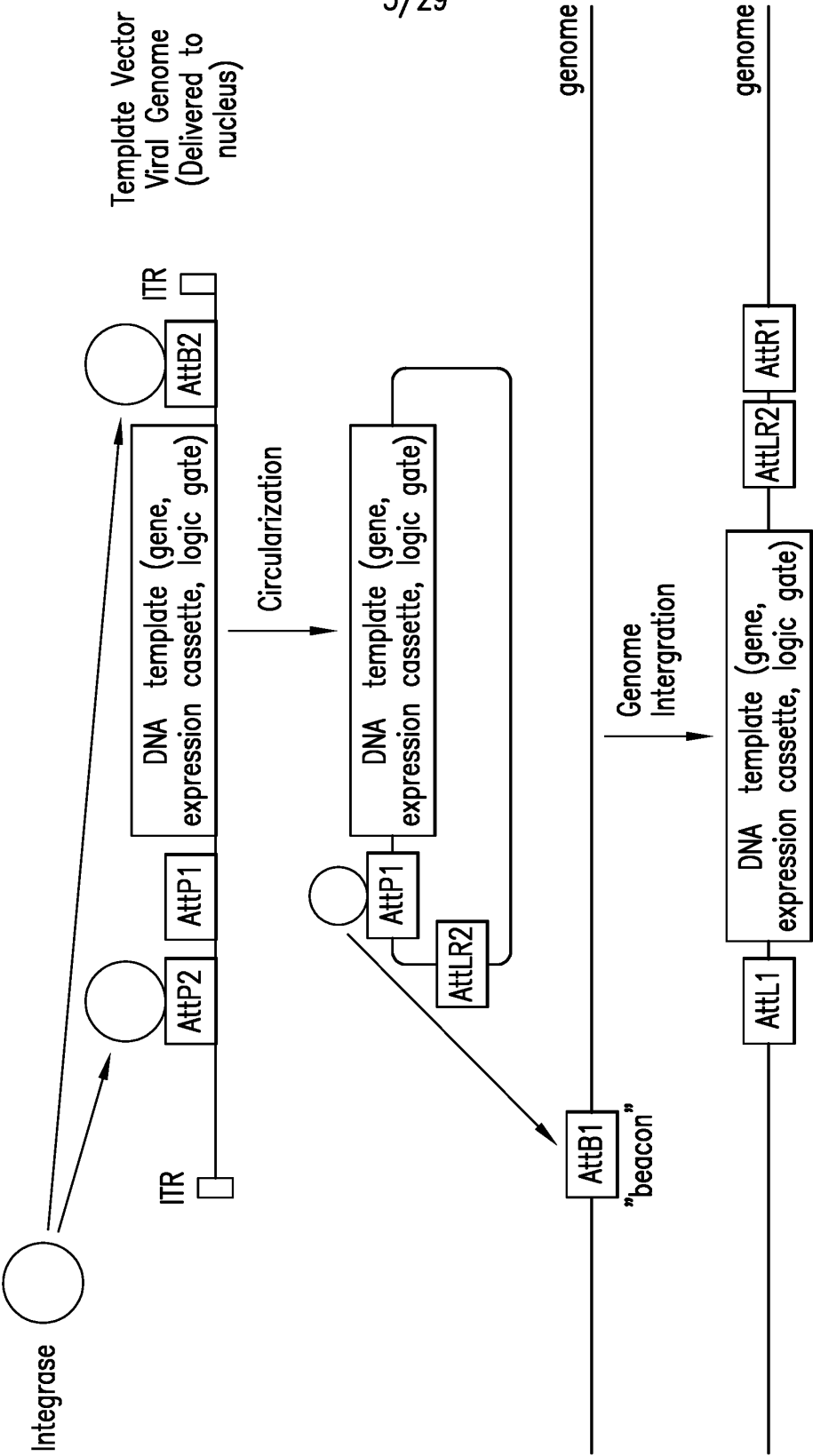


FIG.3

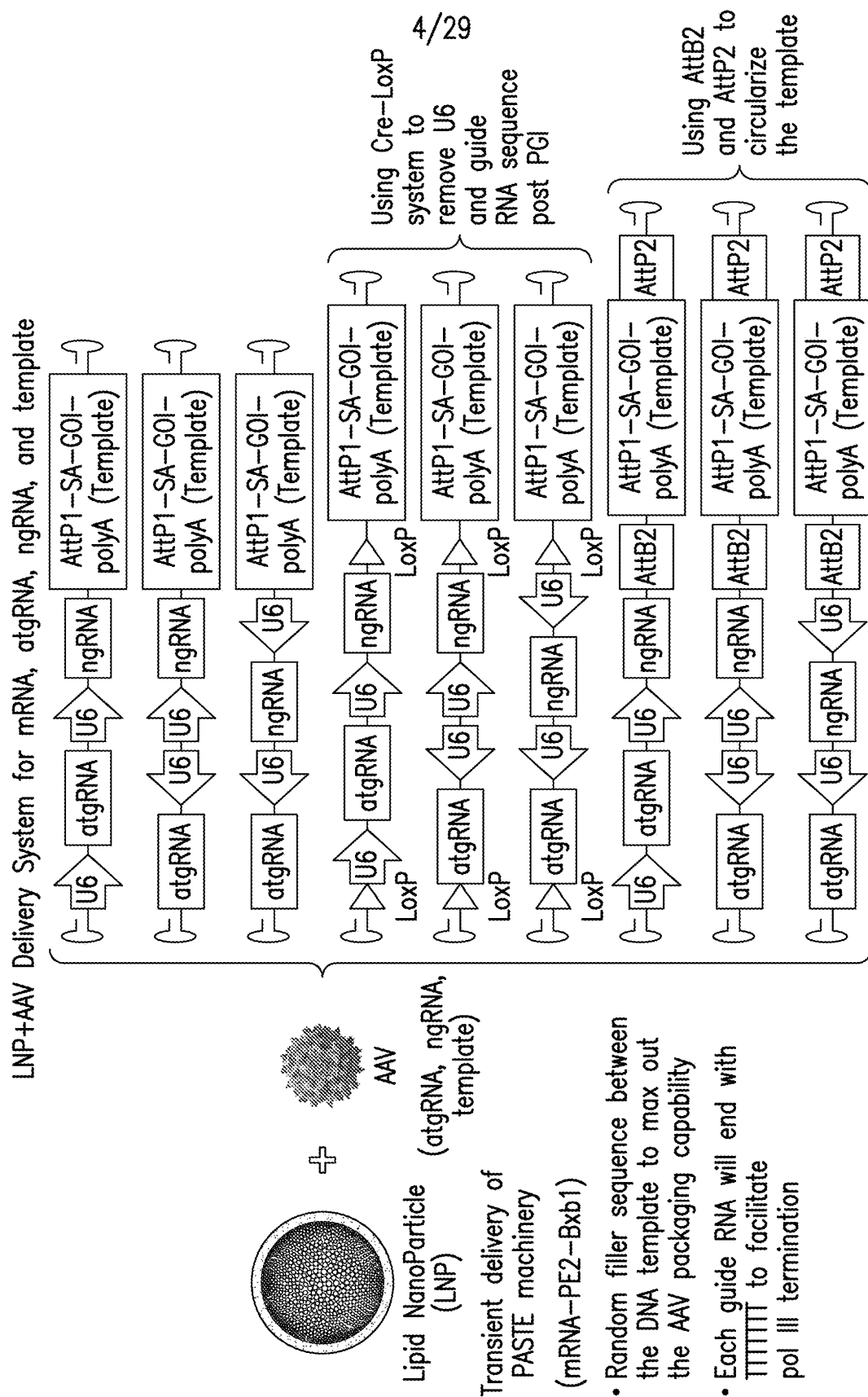
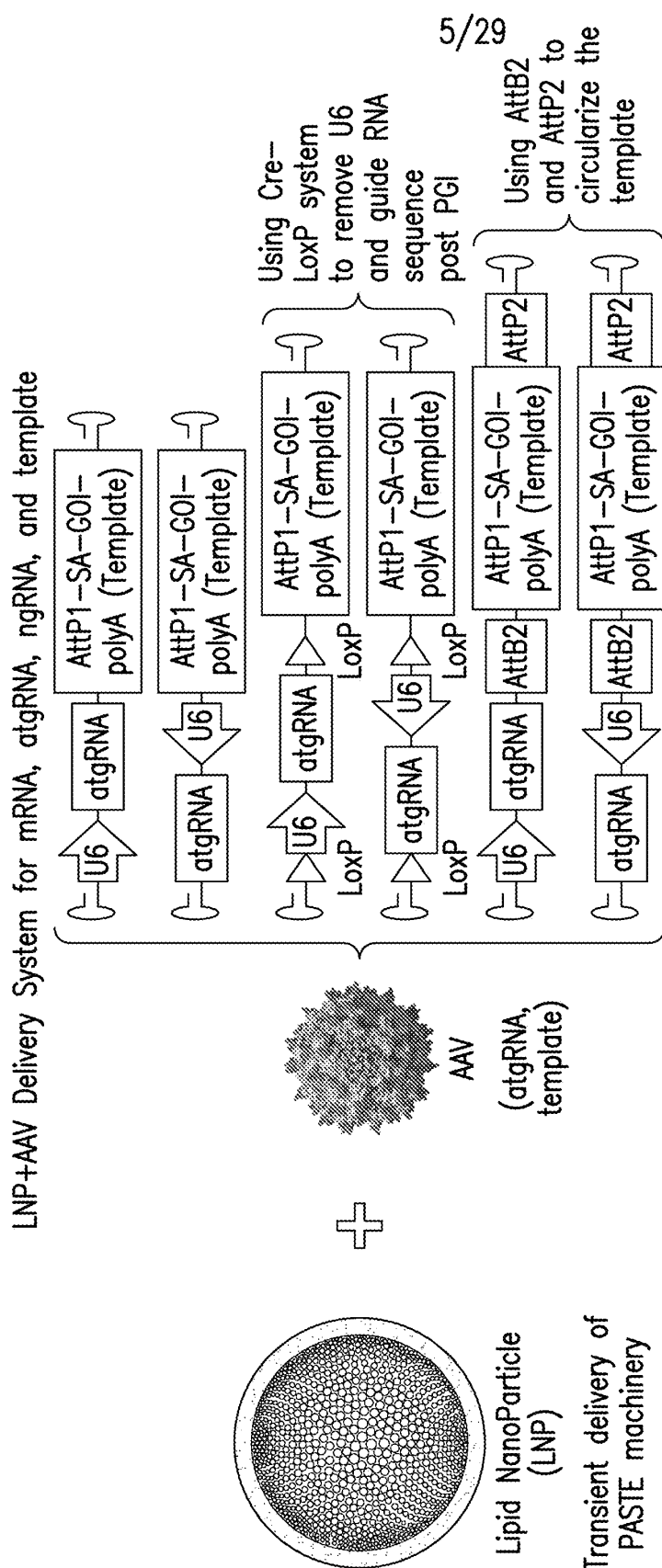


FIG. 4



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- Random filler sequence between each guide RNA and the DNA template to max out the AAV packaging capability
- Each guide RNA will end with TTTTTT to facilitate pol III termination

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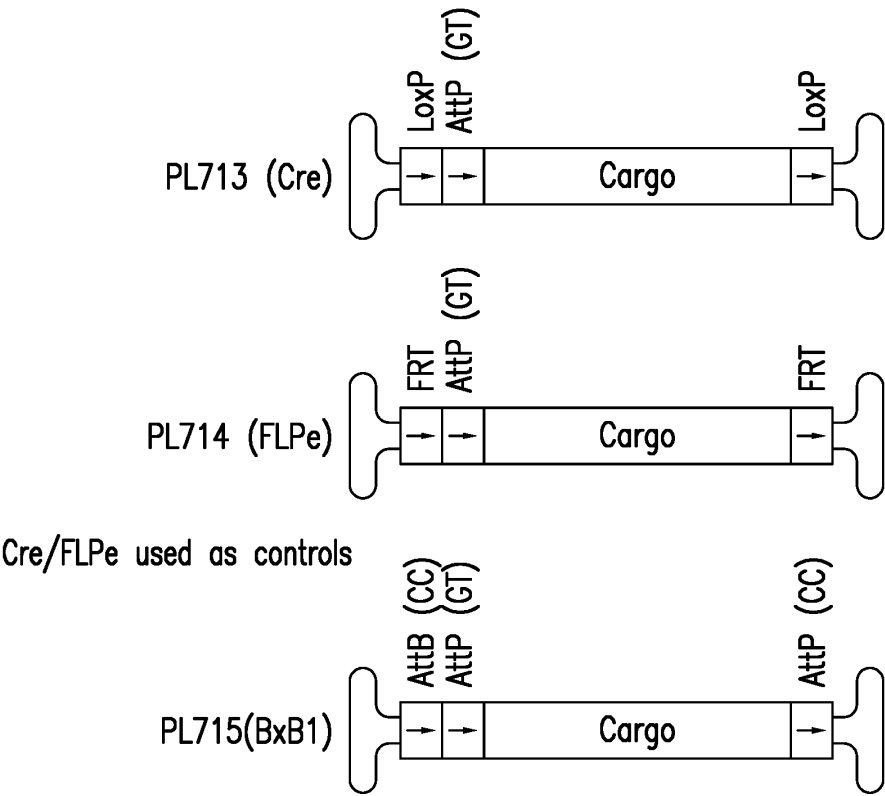


FIG.6A

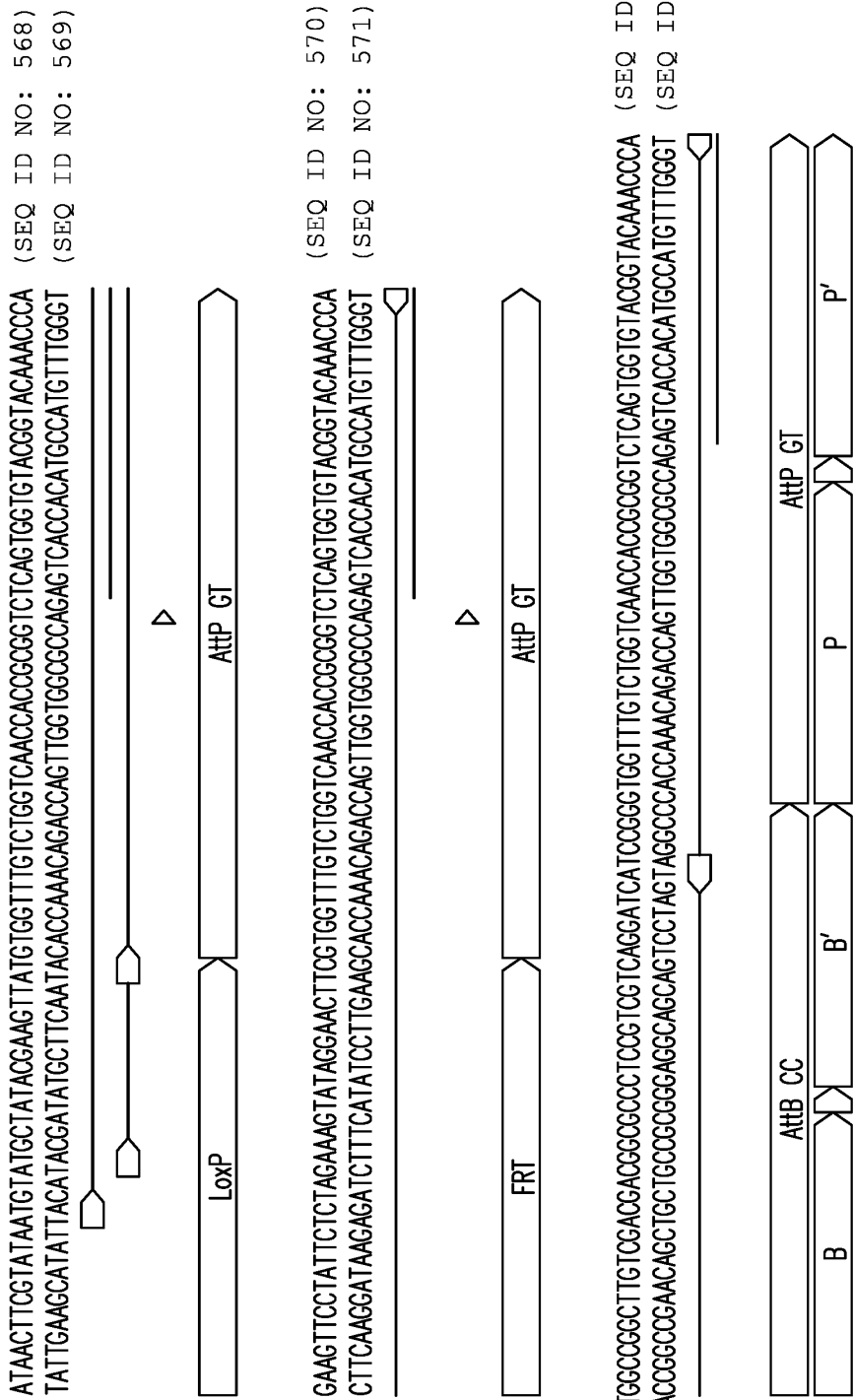


FIG.6B

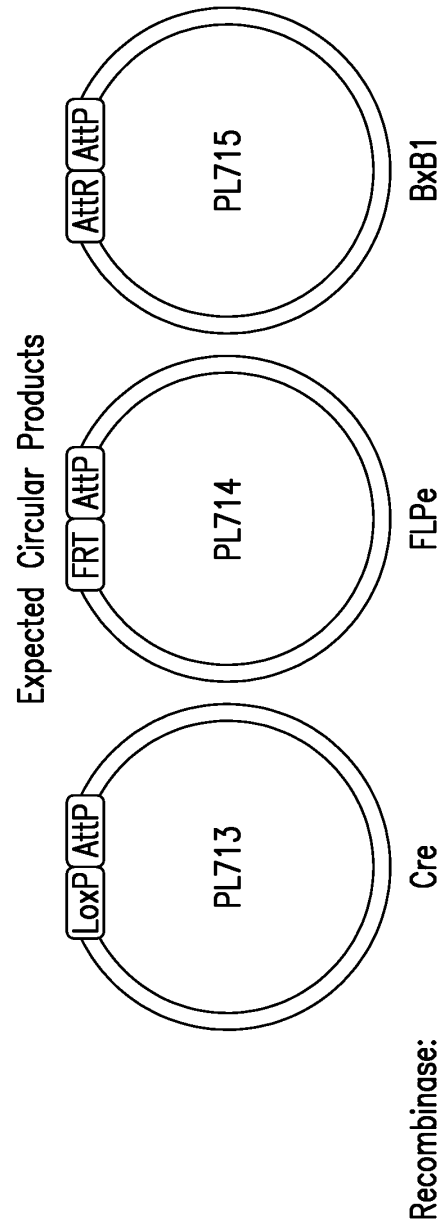


FIG.7

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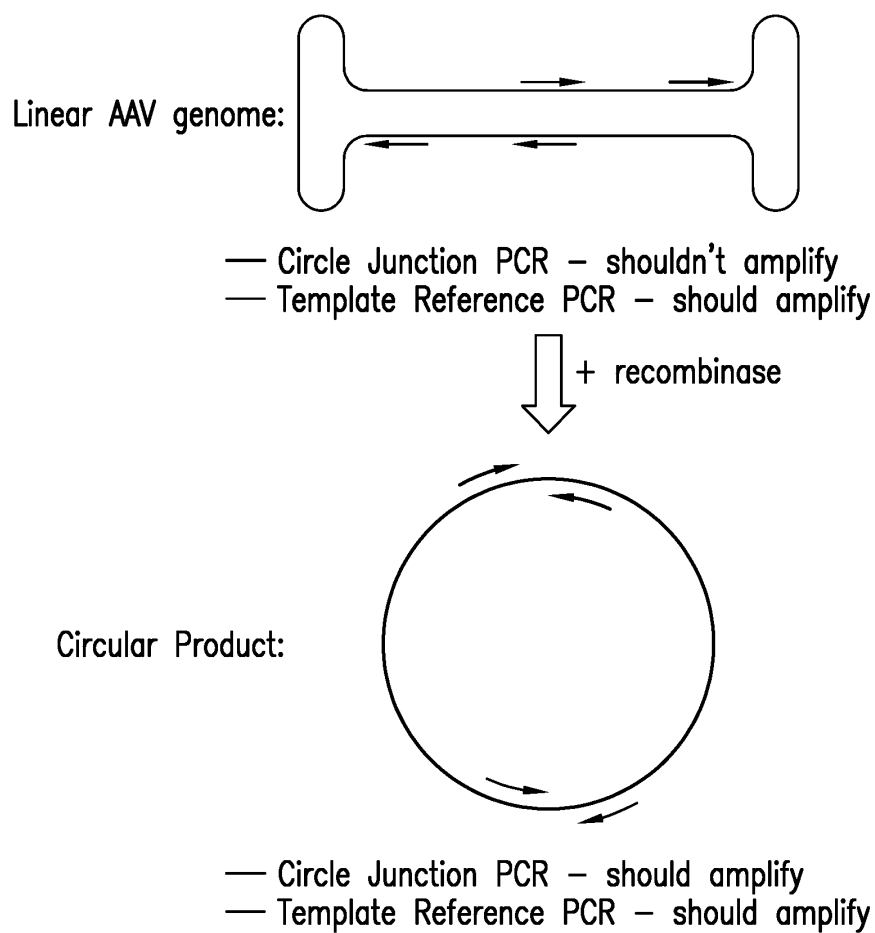
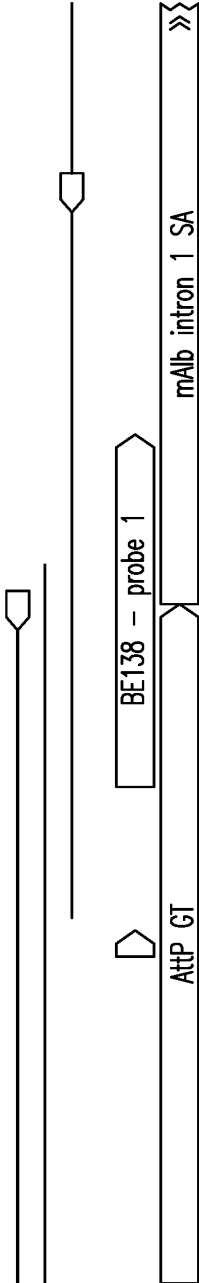


FIG.8A

Universal ddPCR Probe

GTGGTTTGTCTGGTCAACACCGCGTCTCAGTGGTACGGTACAAACCATAGGTCAGTCAAGAGAGAACAAGCAGCATATTACAGTTAGTT (SEQ ID NO: 574)
CACCAAACAGACCAGTTGGTGGCGCCAGAGTCACCACATGCCATGTTGGGTATCCAGTCACCTCTCTCTGTTGTTTCGCTGATATAATGTCATCAATCAA (SEQ ID NO: 575)



- Can bind linear or circle genomes for Cre, FLPe and BxB1 substrates

AttR Probe

GTGGTTTGTCTGGTCAACACCGCGCGCTCCGTCGTGAGGATCATCCGG (SEQ ID NO: 576)
CACCAAACAGACCAGTTGGTGGCGCGCGGAGGAGCAGTCTCTAGTAGGCC (SEQ ID NO: 577)



- Should only bind to Bxb1 recombination scar in circular product

FIG.8B

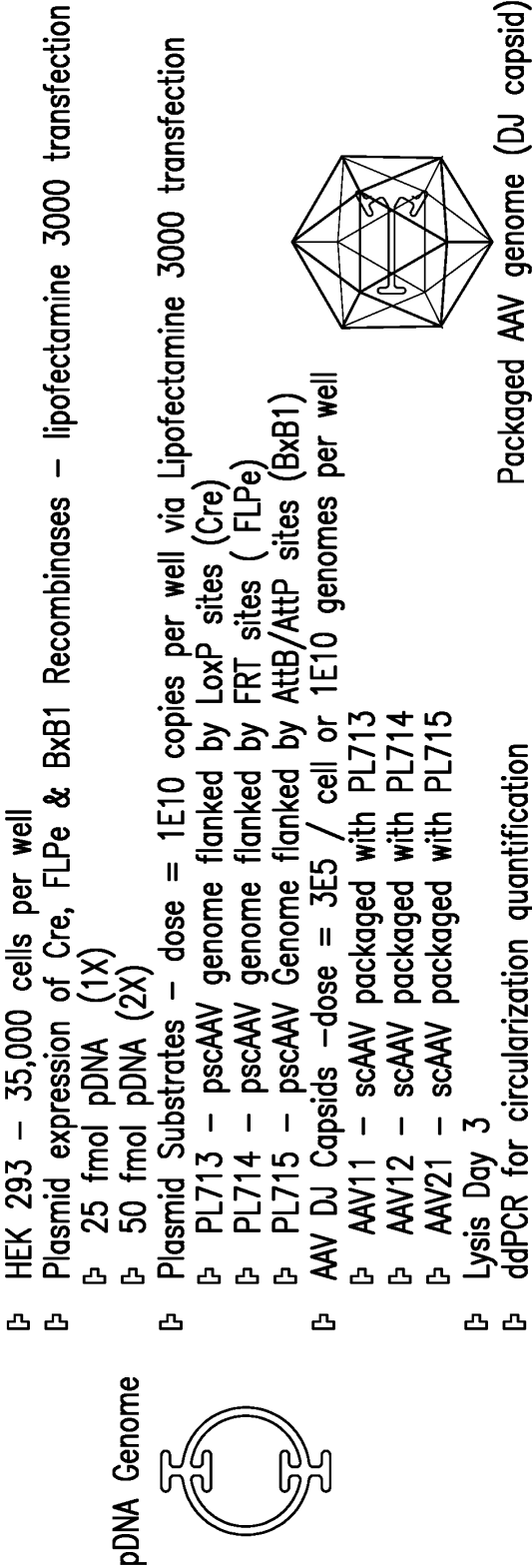


FIG.9

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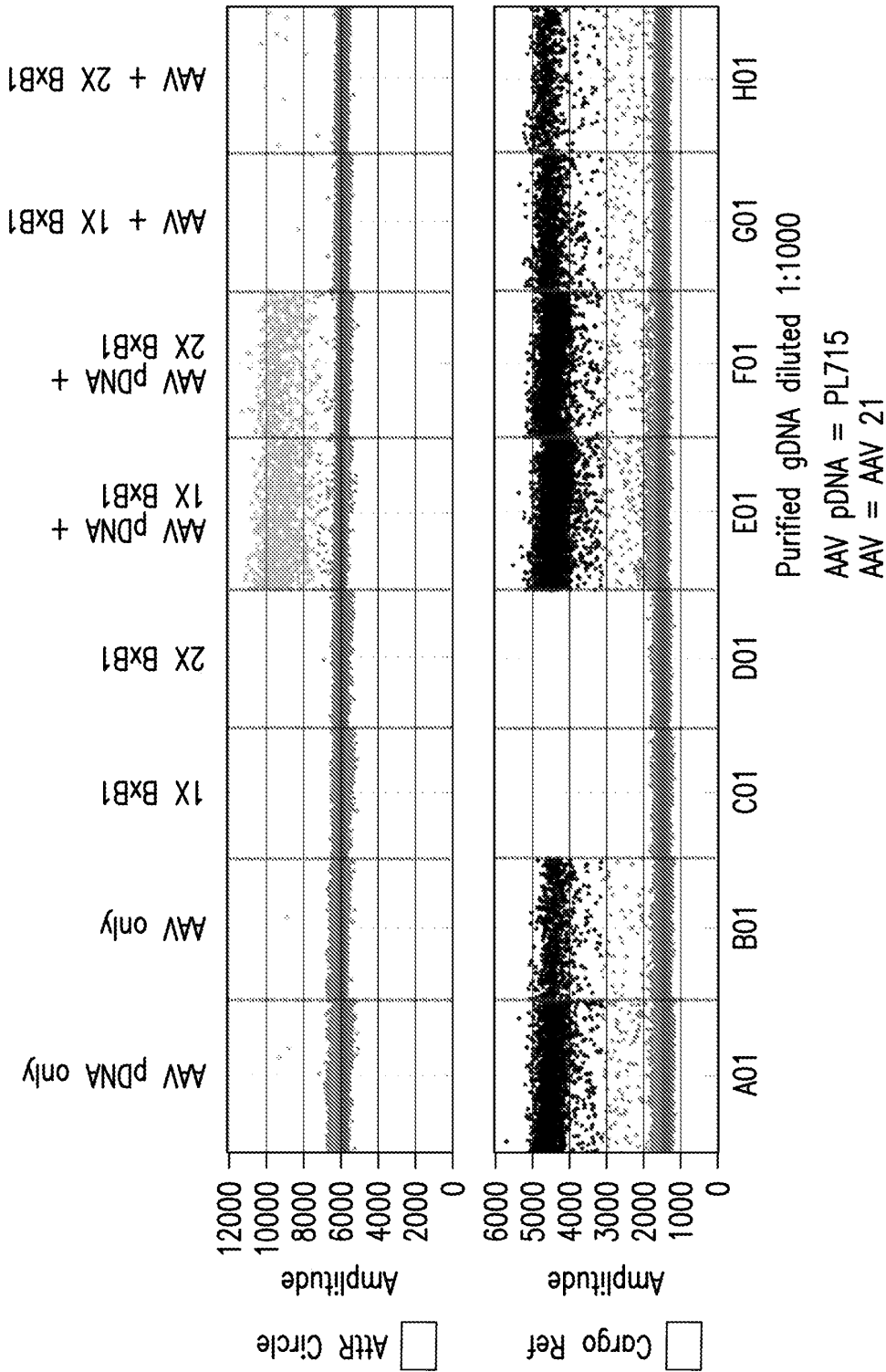


FIG.10

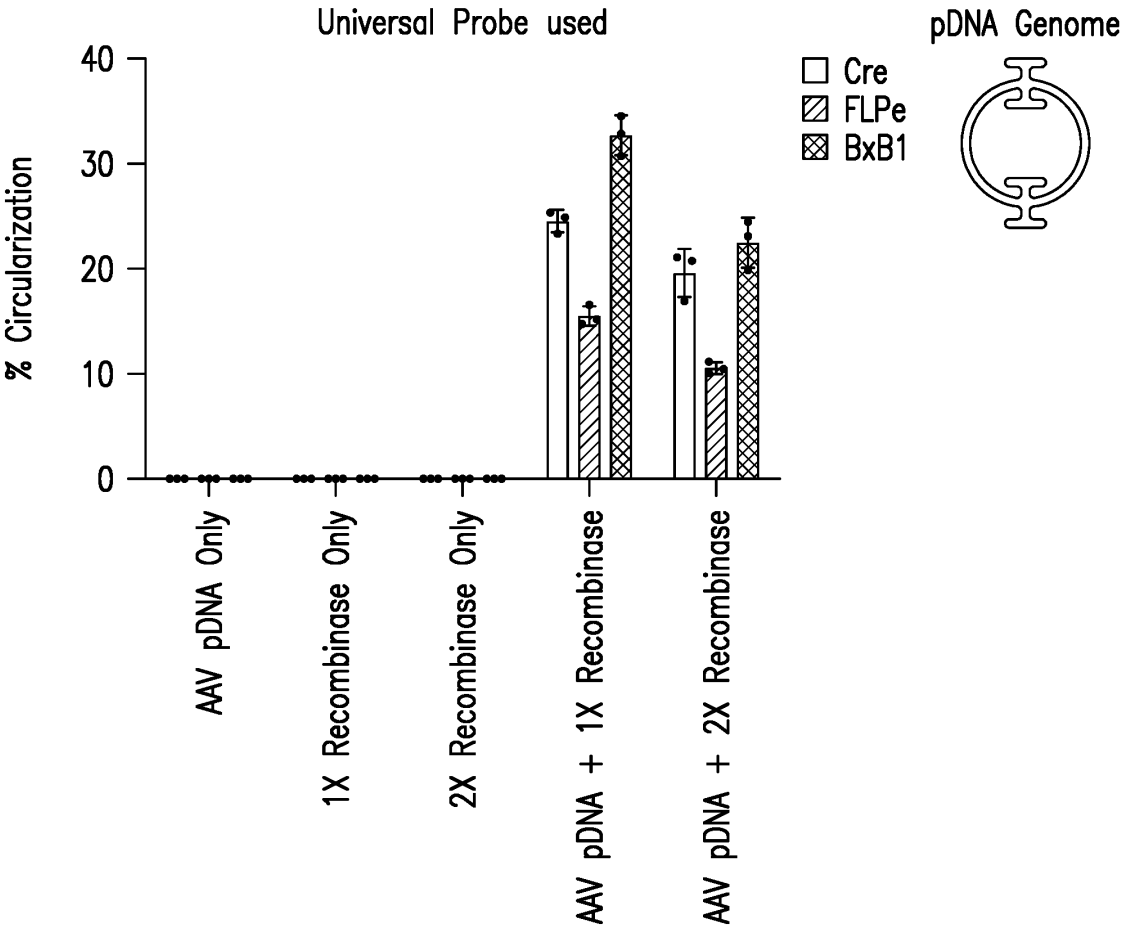


FIG.11

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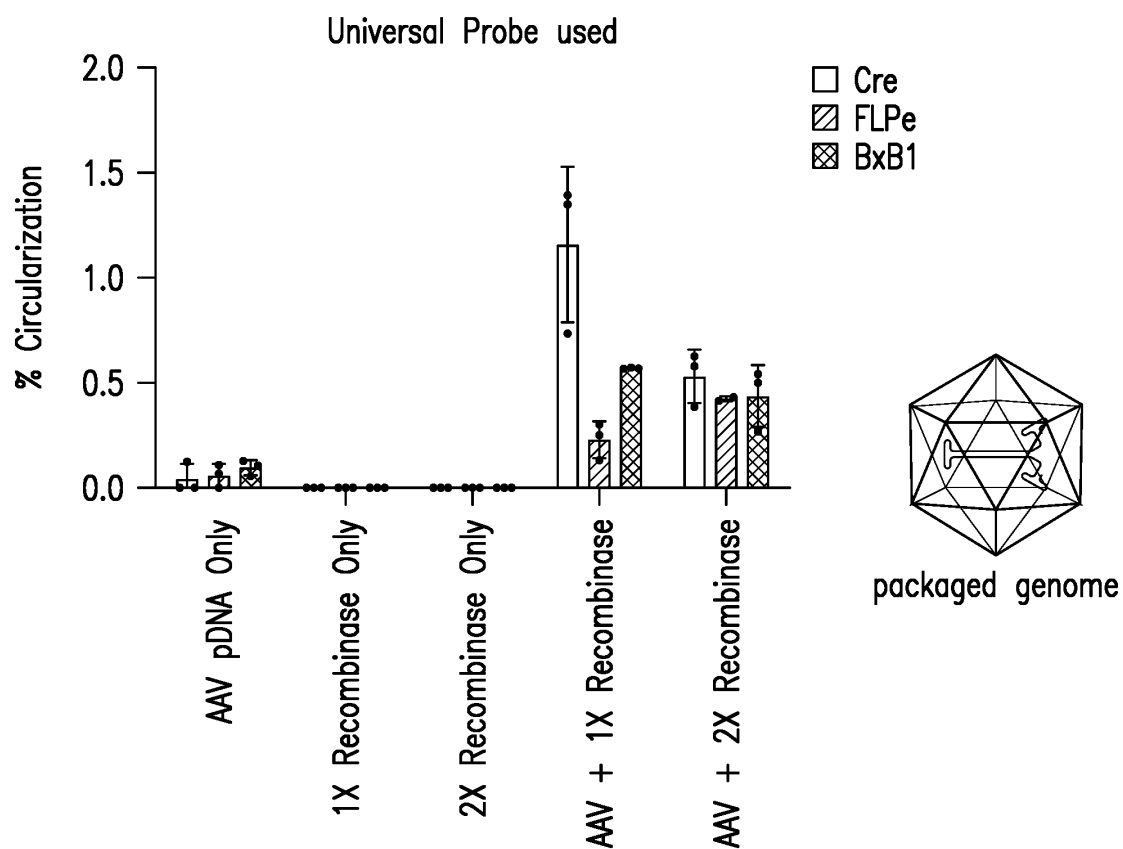


FIG.12

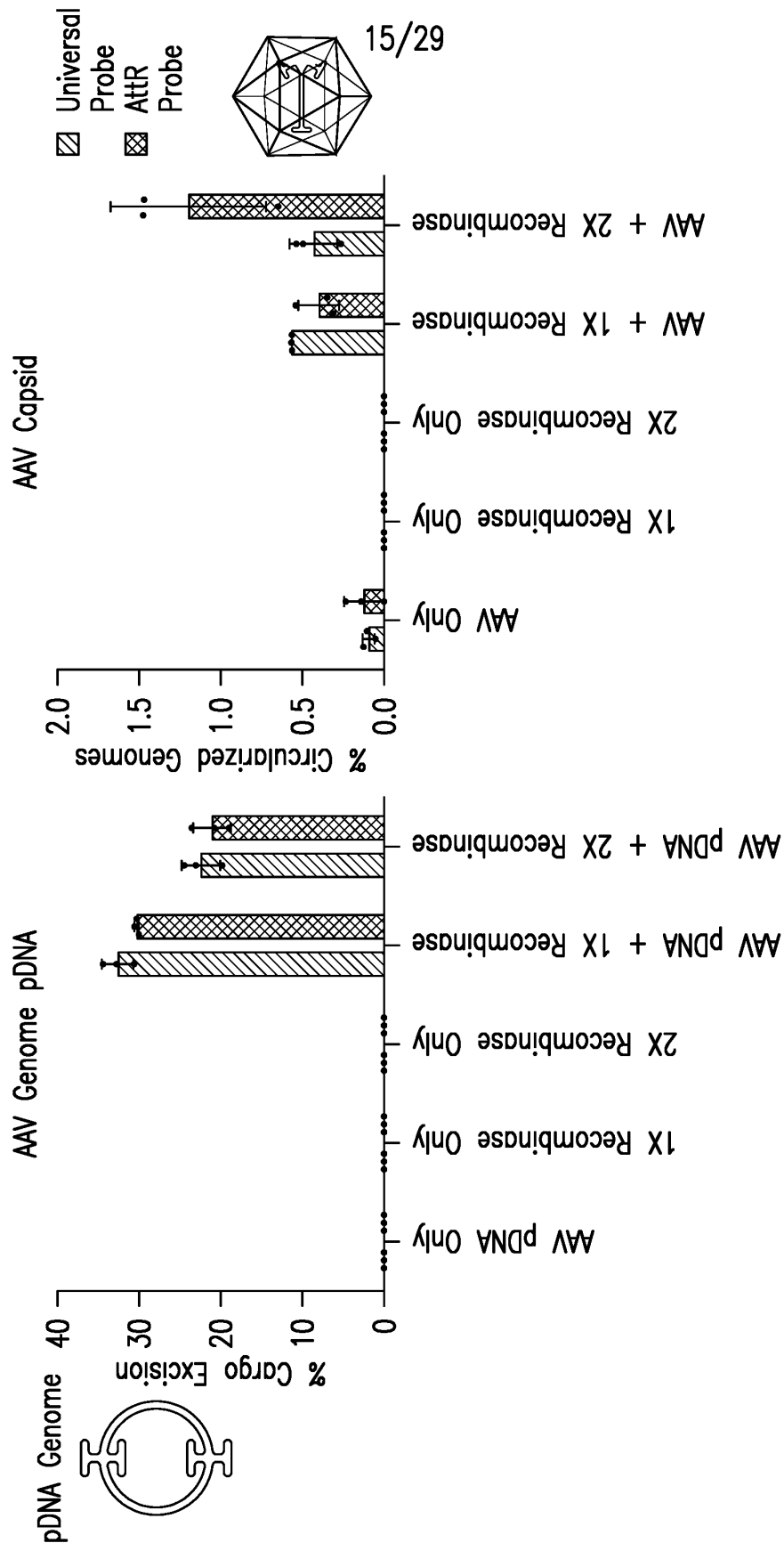


FIG.13

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AttP mutants for improved kinetics

WT AttP

GTGGTTTGTCTGGTCAACCACCGCGGTCTCAGTGGTGTACGGTACAAACCCA

AttP1

GTGGTTTGTGGATGAACAACCTGCAGTCTCAGTGGTGTACGGTACAAACCCA

AttP2

GTGGTTTGTGGTGAACCACAGCGGTCTCAGTGGTGTACGGTACAAACCCA

AttP3

GTGGTTTGTGGATGAACCACTGAAGTCTCAGTGGTGTACGGTACAAACCCA

FIG.14A

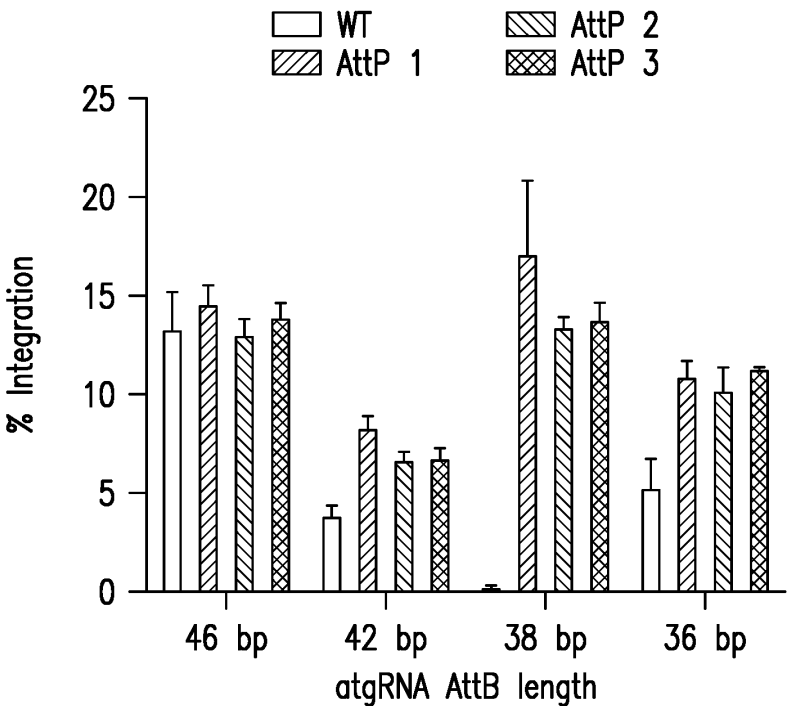


FIG.14B

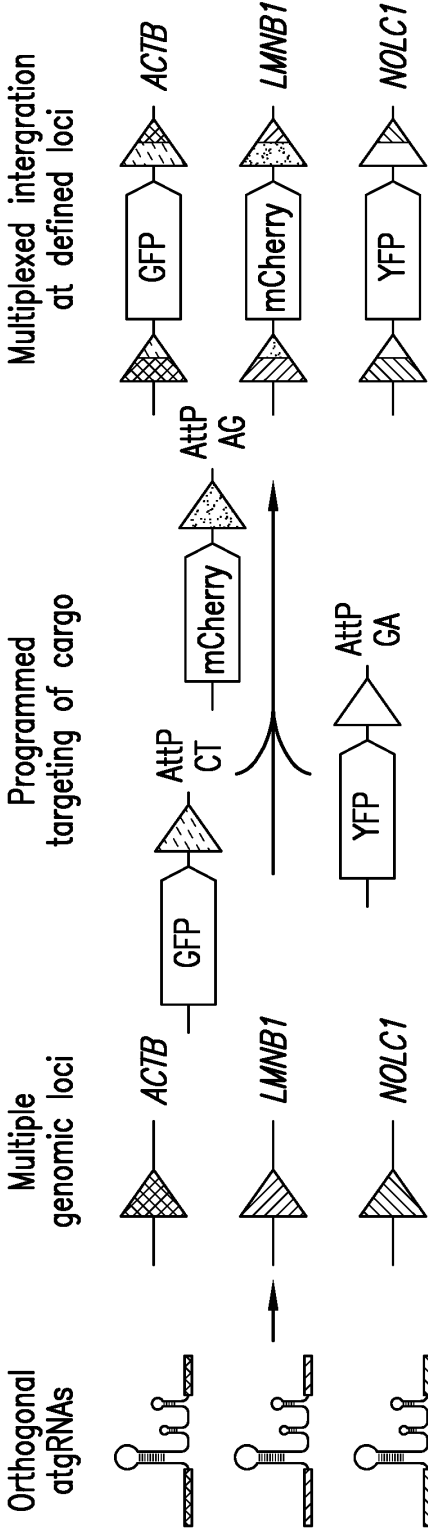


FIG. 14C

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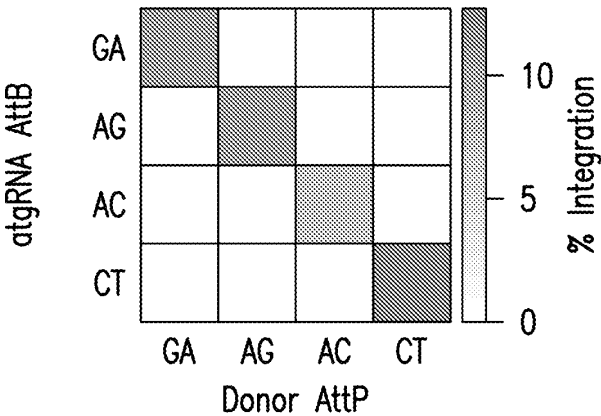


FIG.14D

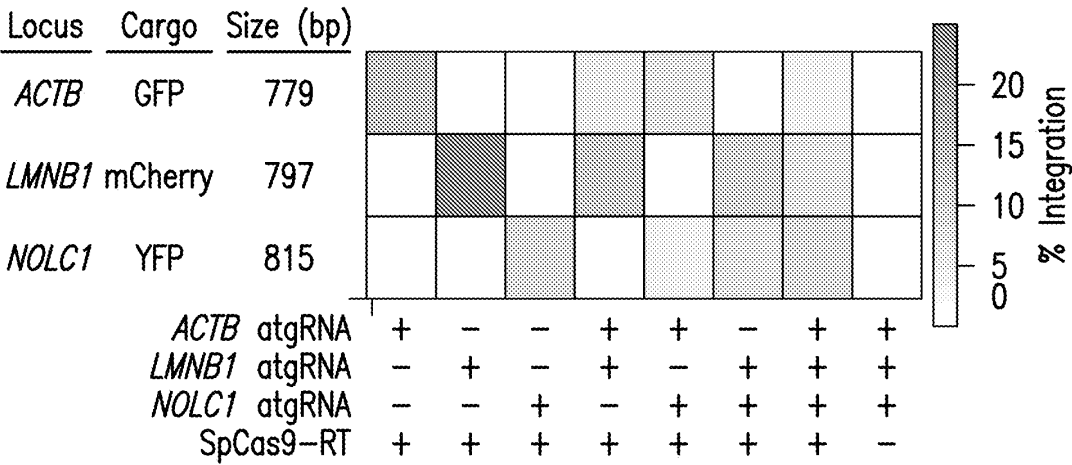


FIG.14E

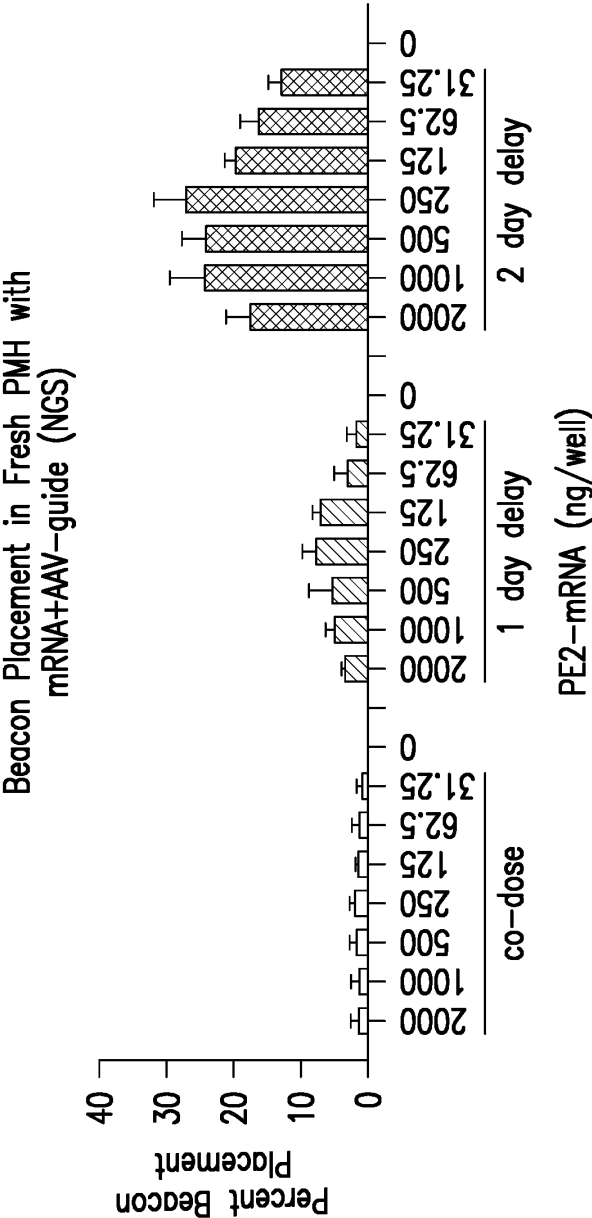


FIG.16

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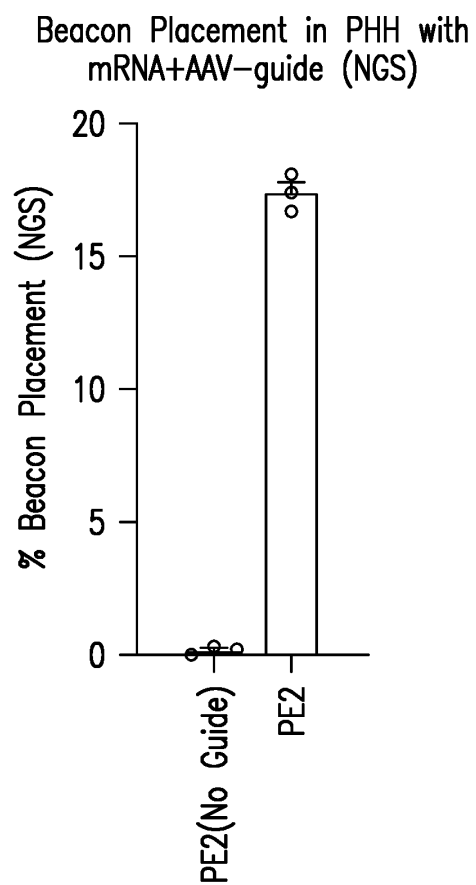


FIG. 17

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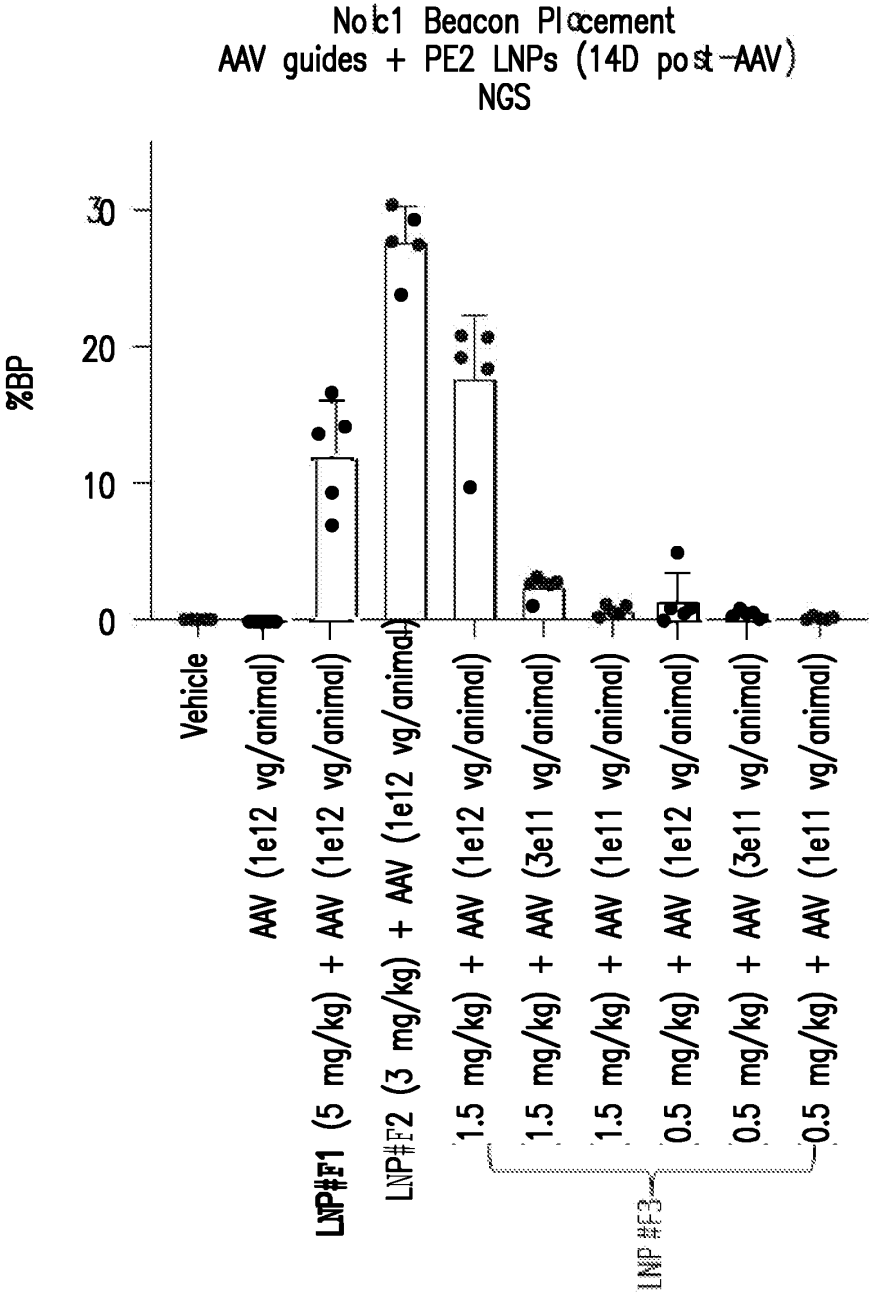


FIG.18

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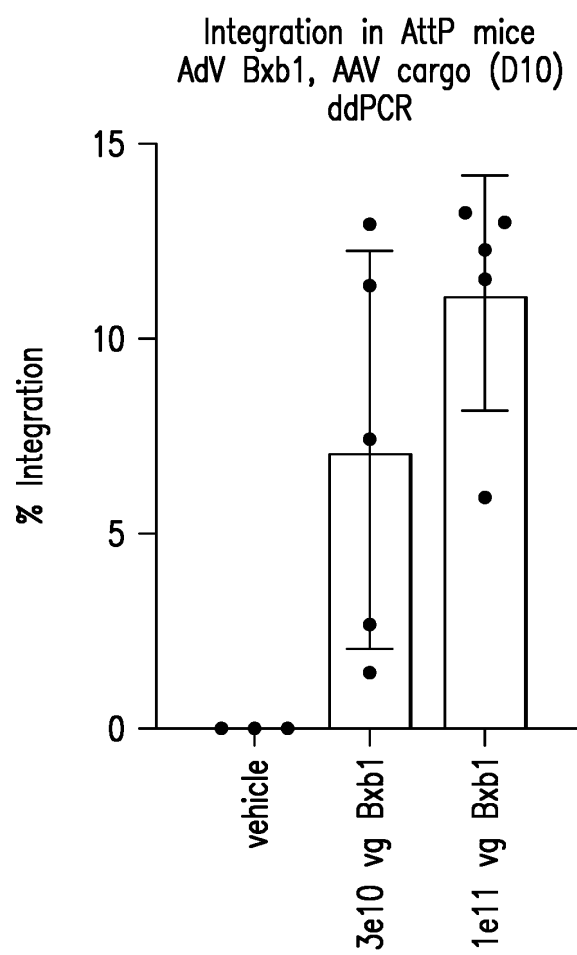


FIG. 19

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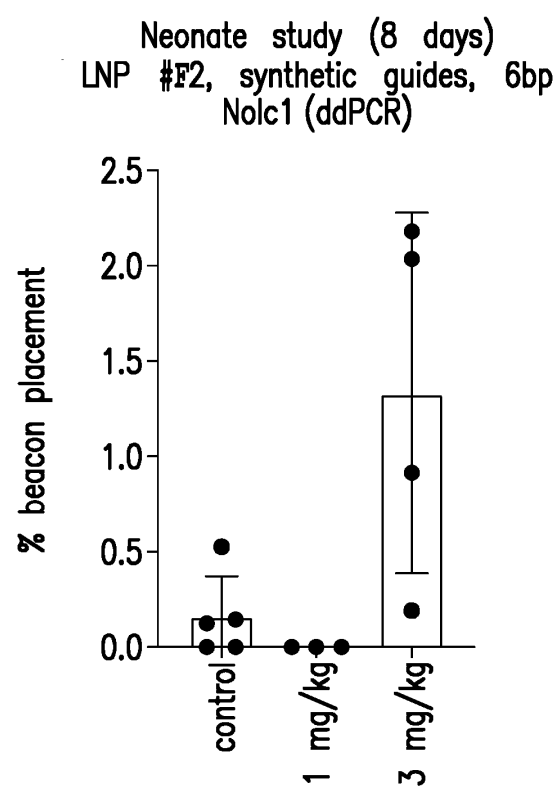


FIG.20A

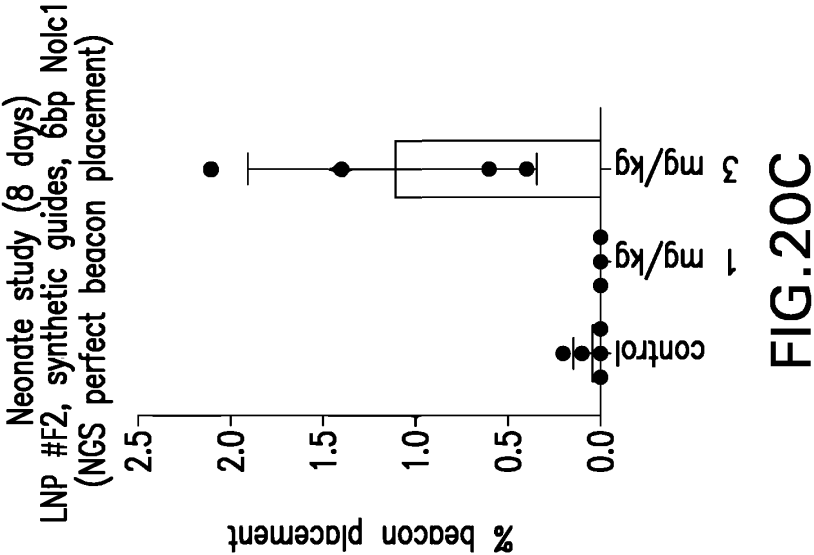


FIG.20C

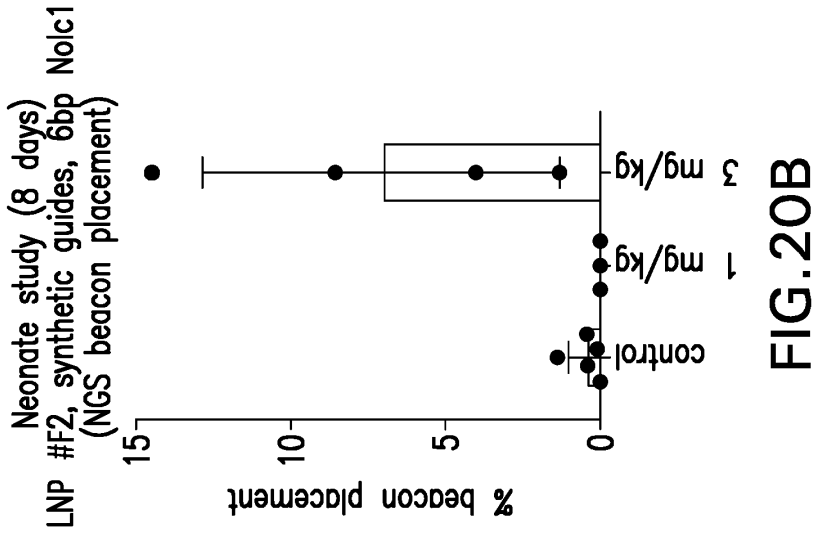


FIG.20B

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Neonate study (6 weeks)
LNP #F2, synthetic guides, 6bp Nlc1
(ddPCR)

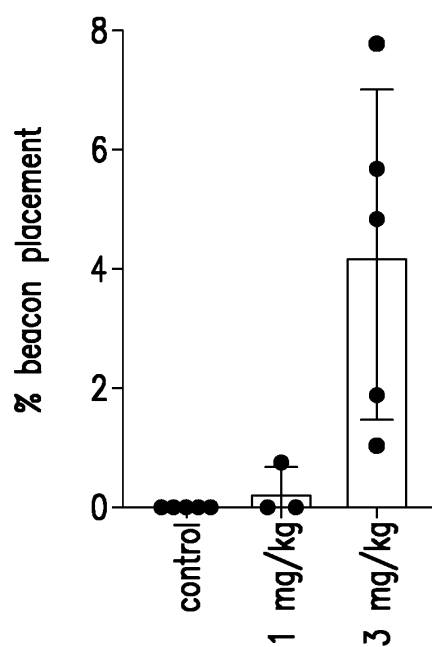


FIG.21A

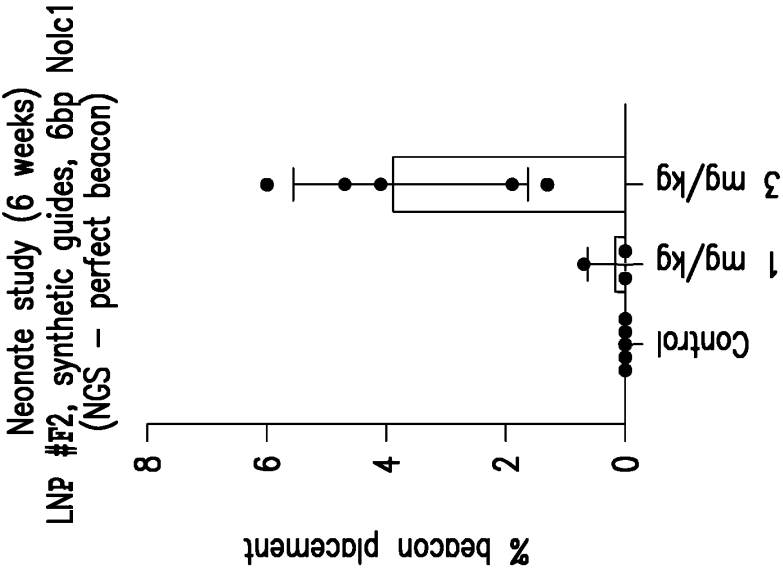


FIG.21C

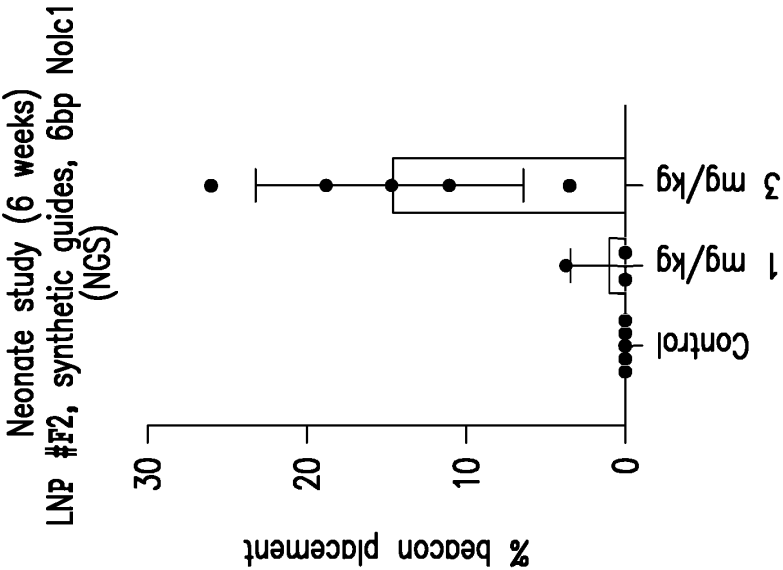


FIG.21B

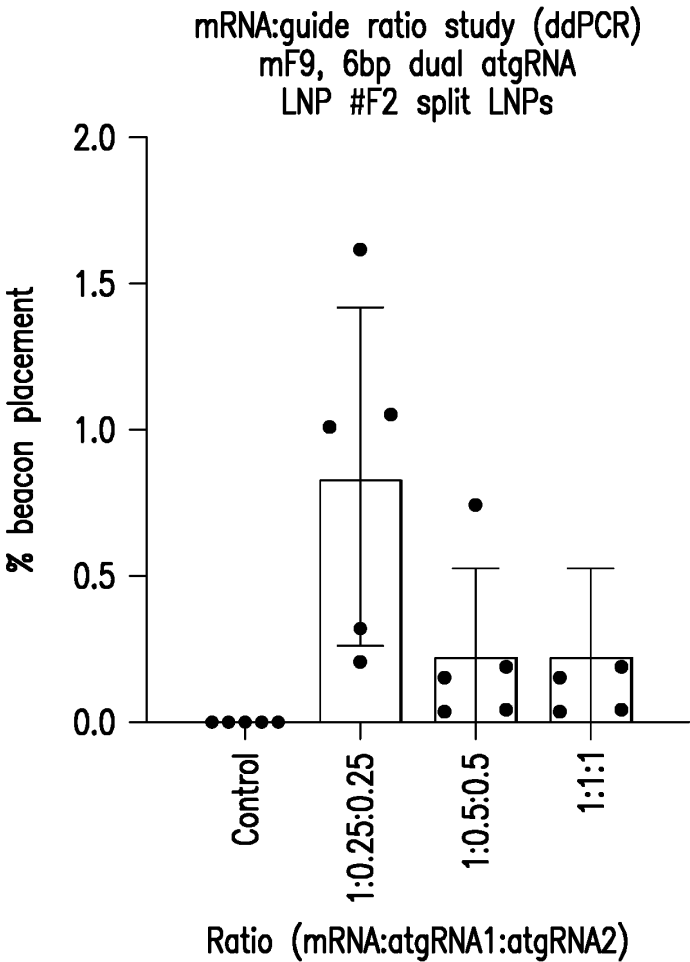


FIG.22A

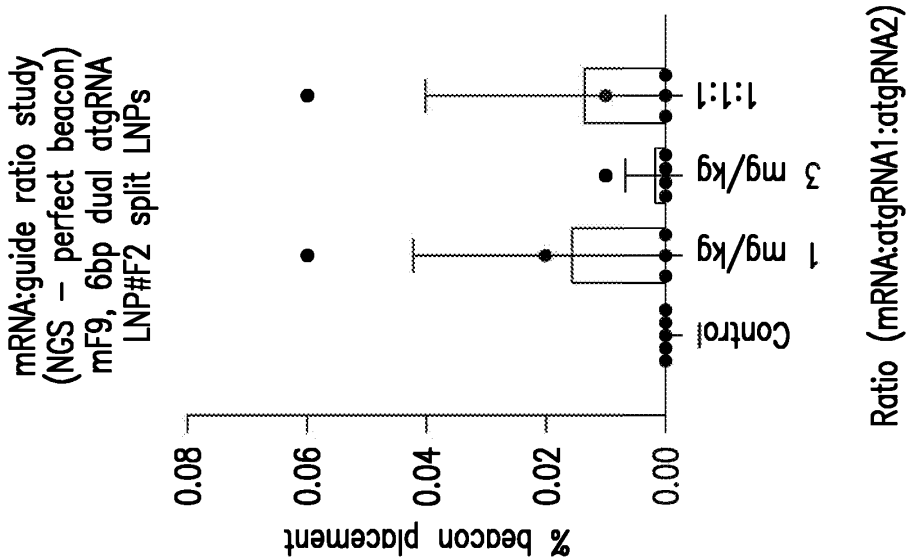


FIG.22C

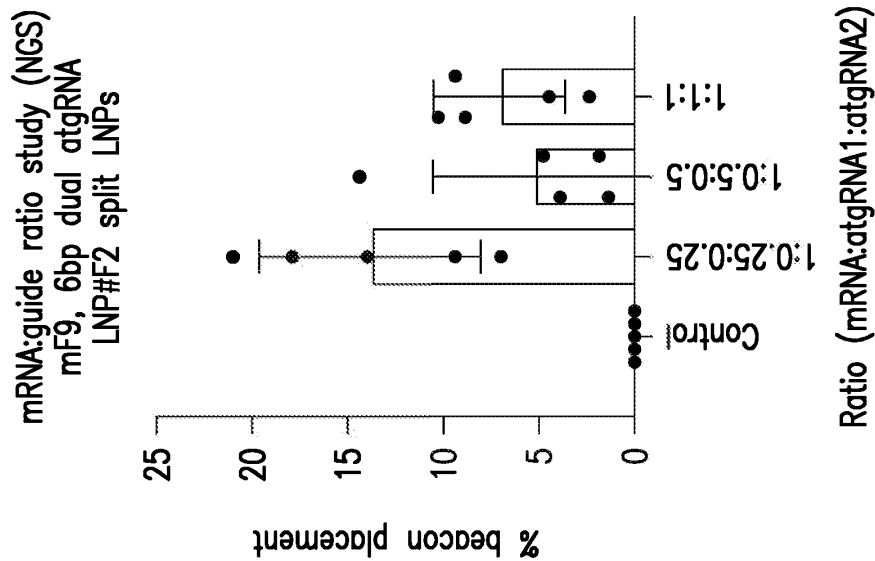


FIG.22B