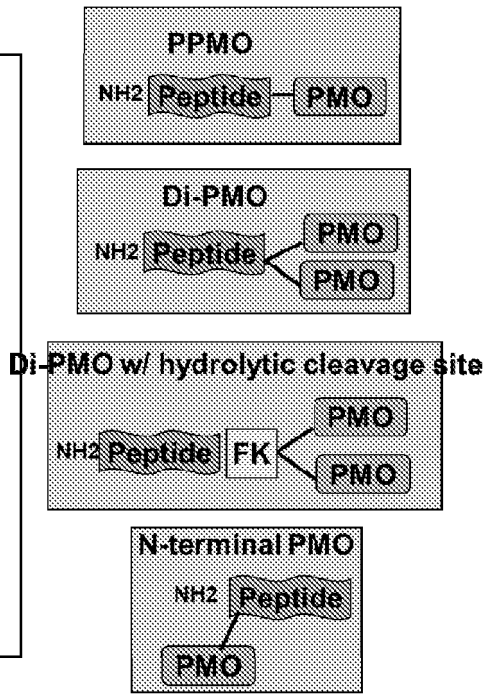
FIGURE 1

K series
Ac-(RXR) 4 XA-PMO - PPMO-K
Ac-(RXR)4 XEG-(PMO)2 – PPMO-Di-K
Ac-(RXR)4 FKEG-(PMO)2 – PPMO-Di-K + "FK" cathepsin site

B series
Ac-(RXRRBR)2XBA-PMO - PPMO-B
E(PMO)(RXRRBR)2XFKG- n-terminal PPMOB
Ac-(RXRRBR)2XEG-(PMO)2 – Di-PMO-B
Ac-(RXRRBR)2 FKEG-(PMO)2 -Di-PMO-K with cathepsin site

X = aminohexanoic acid
E = glutamic acid
FK = cathepsin site
B= beta-alanine



K series SEQ ID NOS from top to bottom

SEQ ID NO.: 92

SEQ ID NO.: 93

SEQ ID NO.: 94

B series SEQ ID NOS from top to bottom

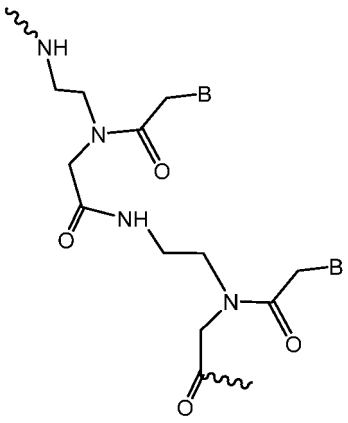
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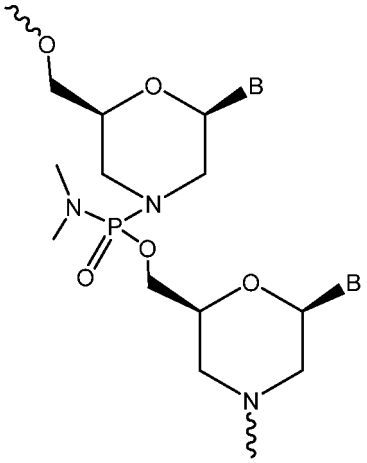
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SEQ ID NO.: 91

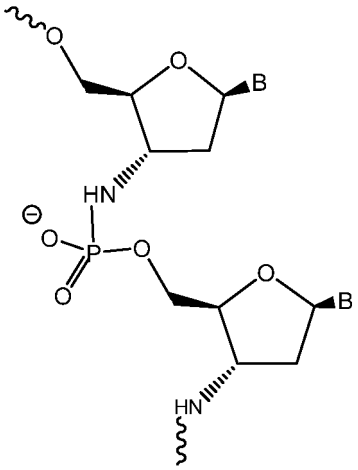
FIGURE 2



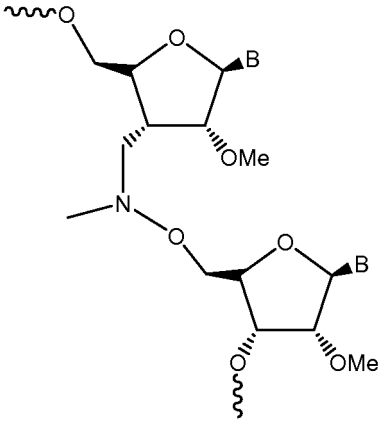
PNA



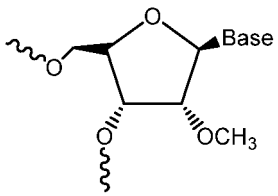
Morpholino



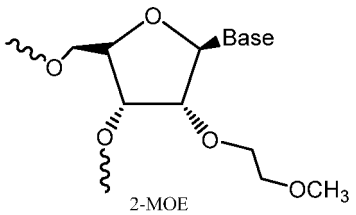
Phosphoramidate



MMI



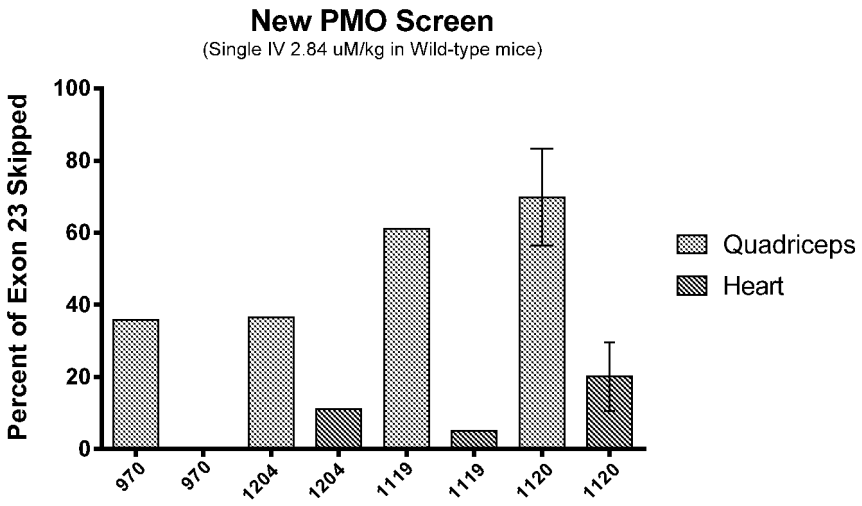
2-OMe



2-MOE

B, Base = nucleotide base: A, G, T, C

FIGURE 3


New PMO Screen
(Single IV 2.84 uM/kg in Wild-type mice)
Percent of Exon 23 Skipped
100
80
60
40
20
0
970
970
1204
1204
1119
1119
1120
1120
Quadriceps
Heart