Figure 1

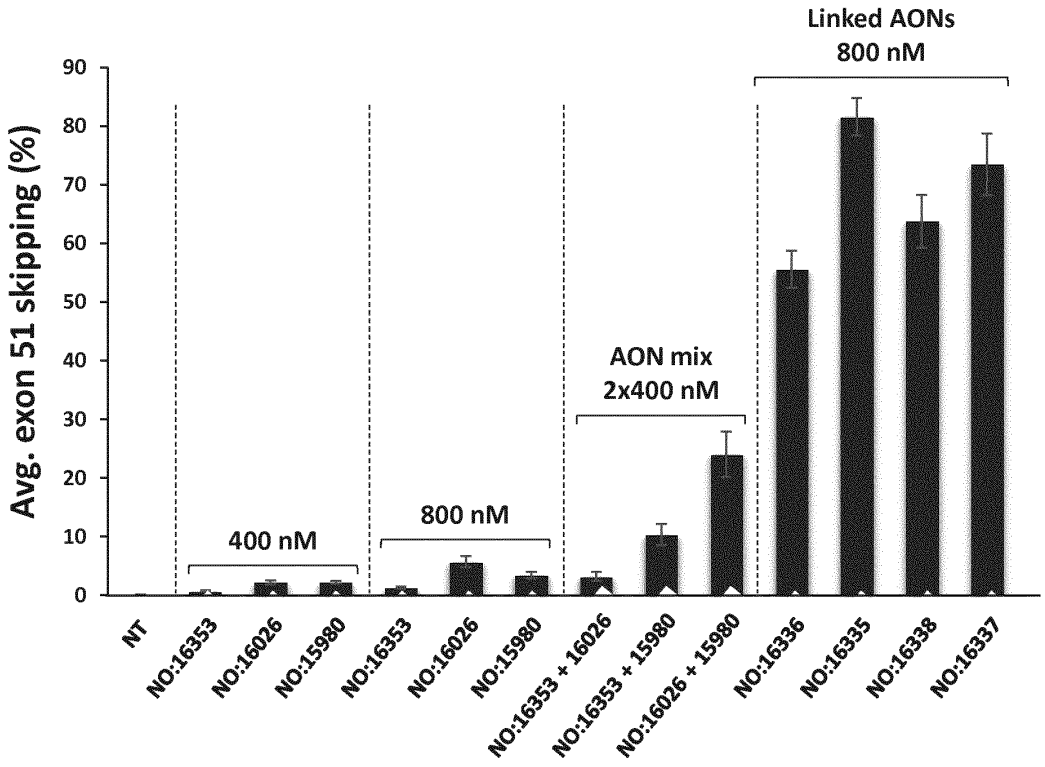
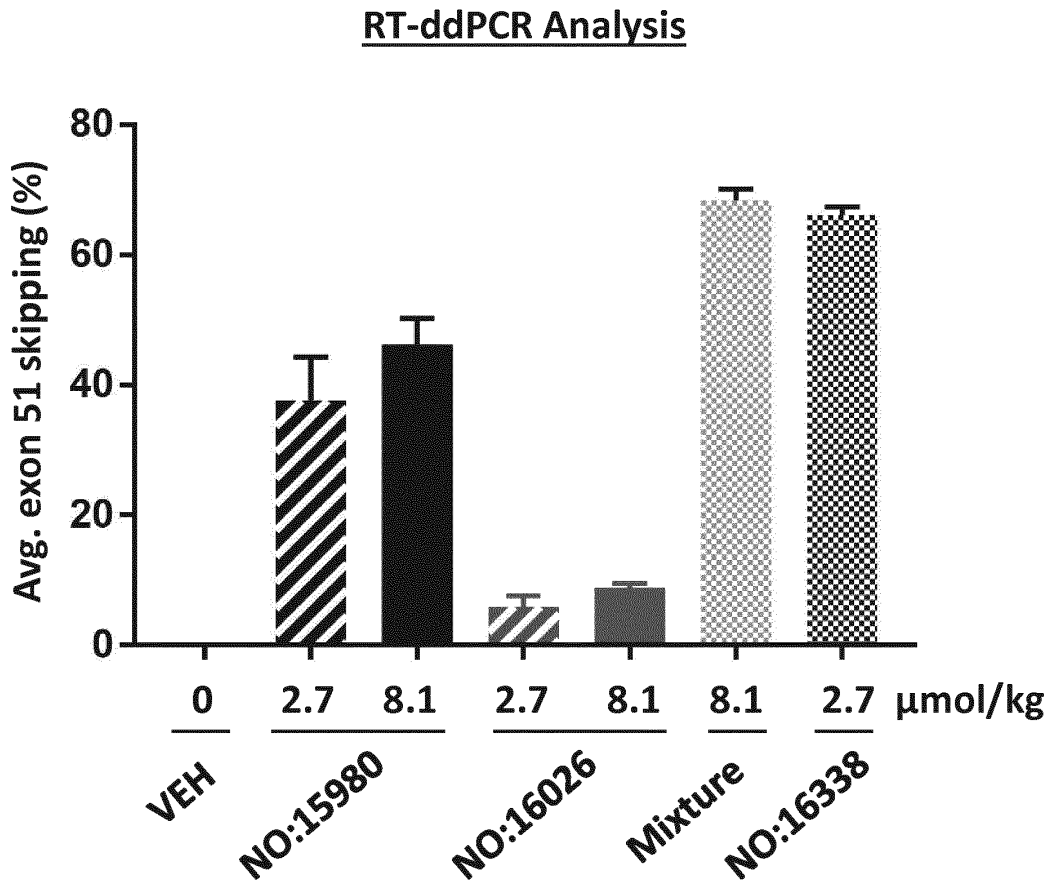

RT-ddPCR Analysis
Avg. exon 51 skipping (%)
90
80
70
60
50
40
30
20
10
0
400 nM
800 nM
AON mix
2x400 nM
Linked AONs
800 nM
NT
NO:16353
NO:16026
NO:15980
NO:16353
NO:16026
NO:15980
NO:16353 + 16026
NO:16353 + 15980
NO:16026 + 15980
NO:16336
NO:16335
NO:16338
NO:16337

Figure 2


RT-ddPCR Analysis
Avg. exon 51 skipping (%)
80
60
40
20
0
0
2.7
8.1
2.7
8.1
8.1
2.7
µmol/kg
VEH
NO:15980
NO:16026
Mixture
NO:16338