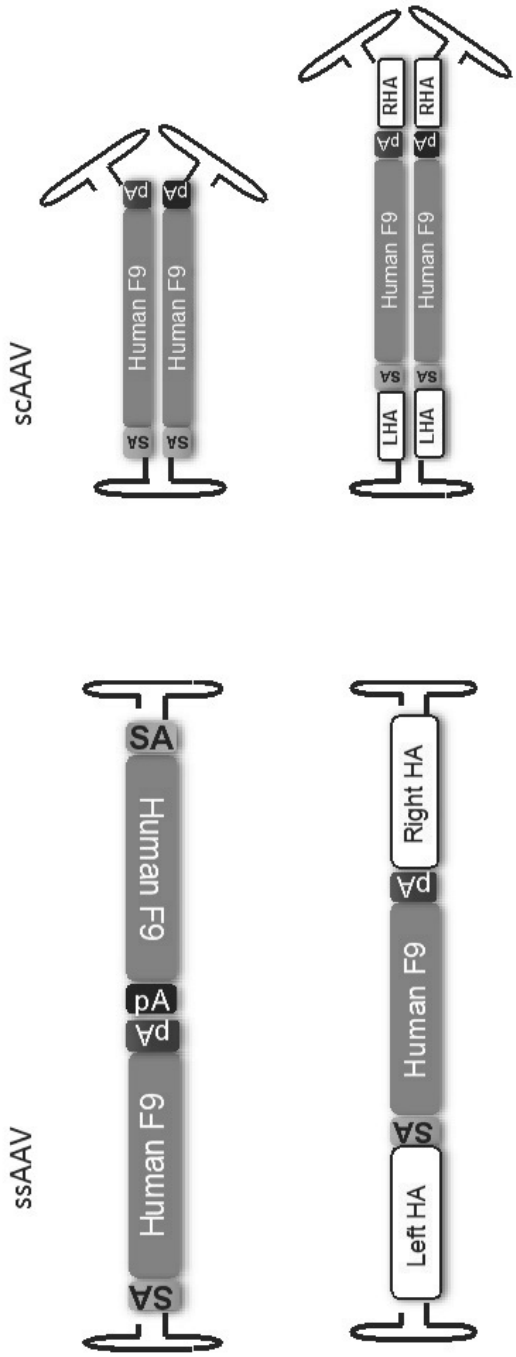

scAAV
Human F9
Human F9
pA
SA
LHA
RHA
ssAAV
Human F9
pA
SA
Left HA
Right HA

FIG. 1

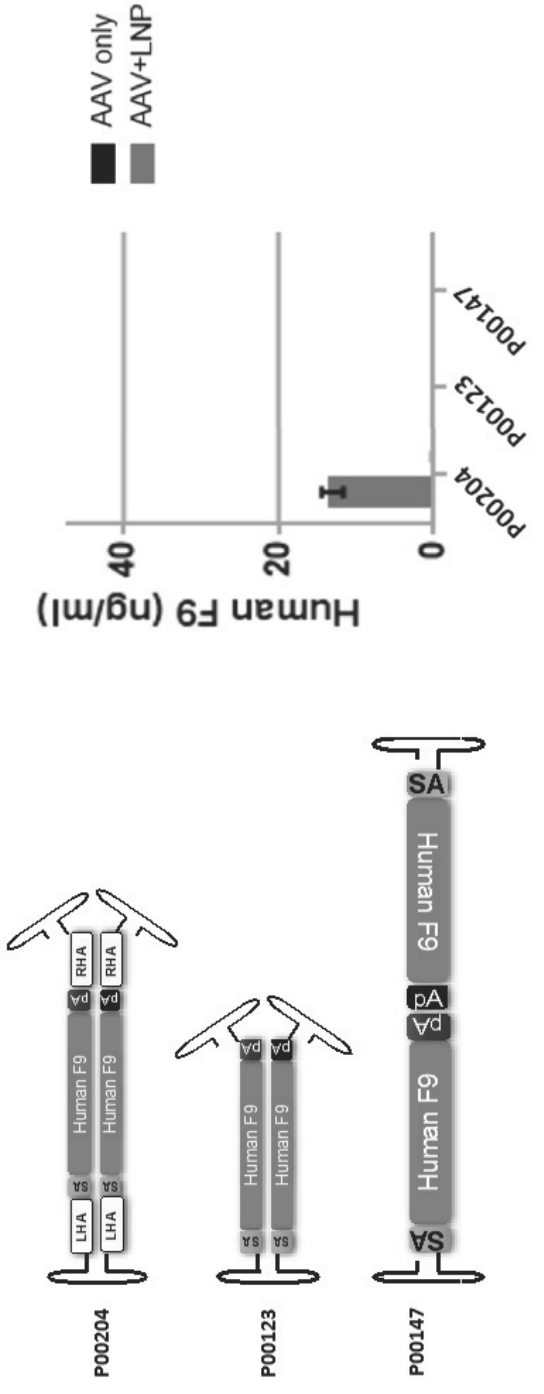

AAV only
AAV+LNP
Human F9 (ng/ml)
0
20
40
P00204
P00123
P00147
LHA
RHA
SA
Human F9
pA

FIG. 2

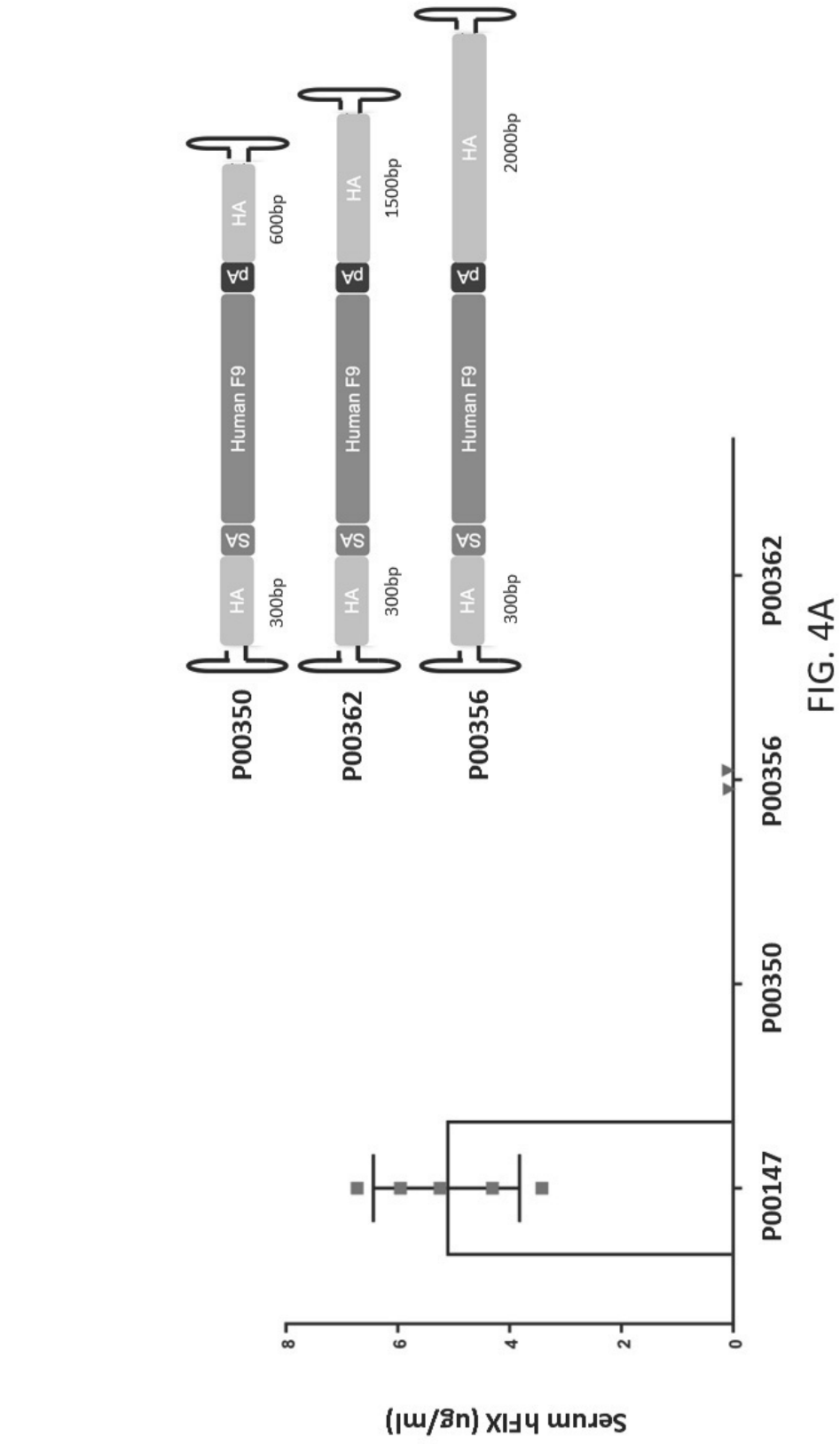

P00350
HA
300bp
SA
Human F9
pA
HA
600bp
P00362
HA
300bp
SA
Human F9
pA
HA
1500bp
P00356
HA
300bp
SA
Human F9
pA
HA
2000bp
Serum hFIX (ug/ml)
0
2
4
6
8
P00147
P00350
P00356
P00362

FIG. 4A

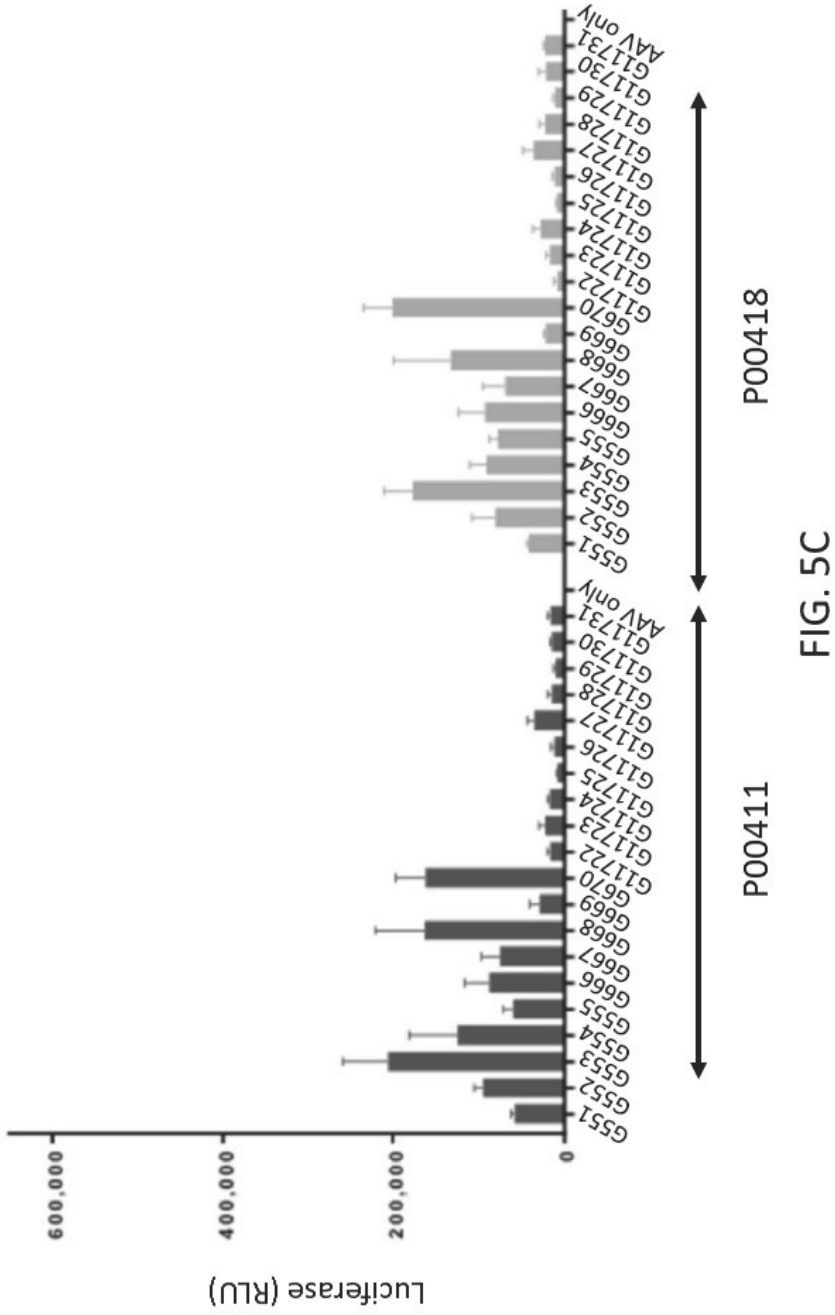
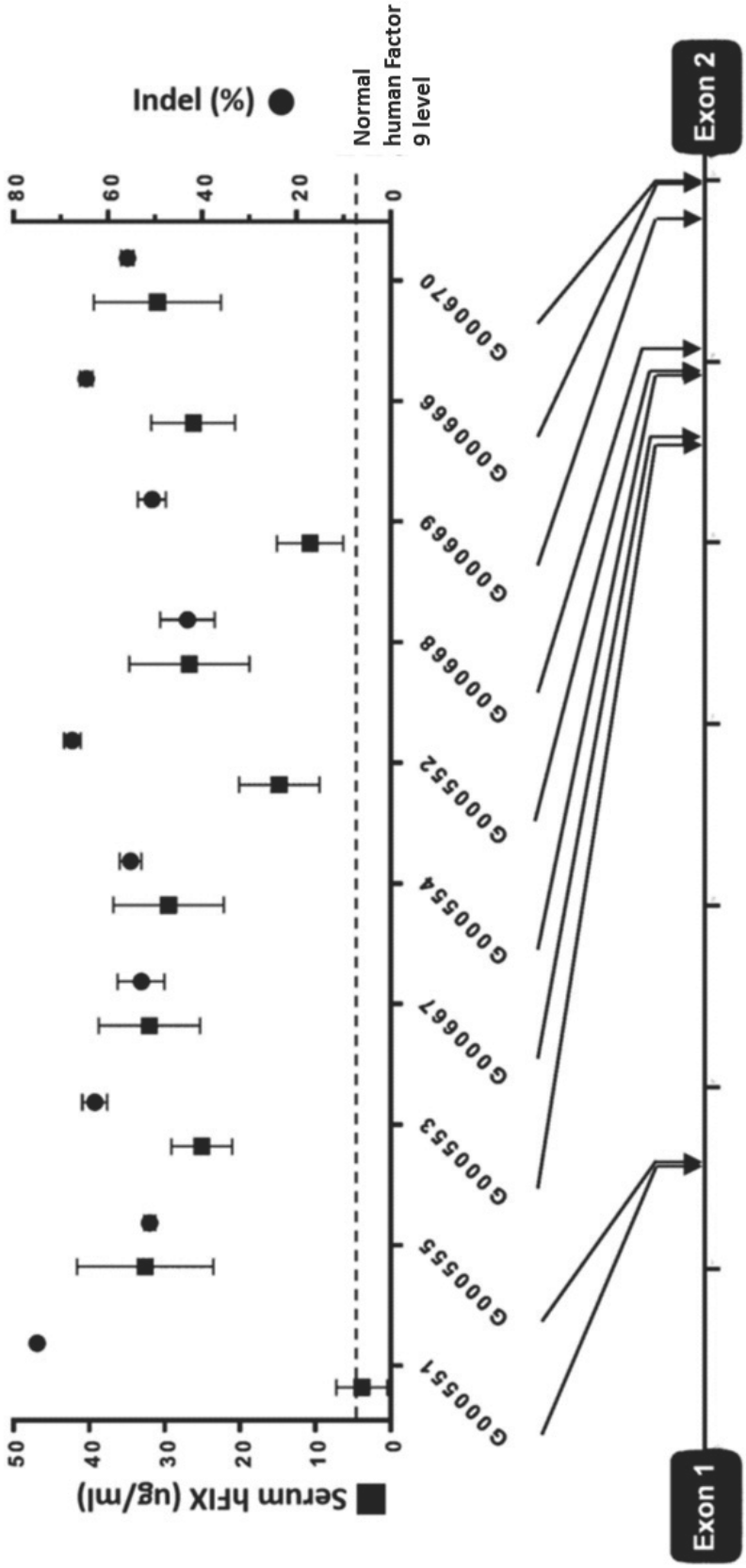

Luciferase (RLU)
600,000
400,000
200,000
0
G551
G552
G553
G554
G555
G666
G667
G668
G669
G670
G1722
G1723
G1724
G1725
G1726
G1727
G1728
G1729
G1730
G1731
AAV only
P00411
P00418

FIG. 5C

FIG. 6


Exon 1
Exon 2
G000551
G000555
G000553
G000567
G000554
G000552
G000568
G000569
G000566
G000570
Normal human Factor 9 level
Indel (%)
Serum hFIX (ug/ml)
0
10
20
30
40
50
0
20
40
60
80