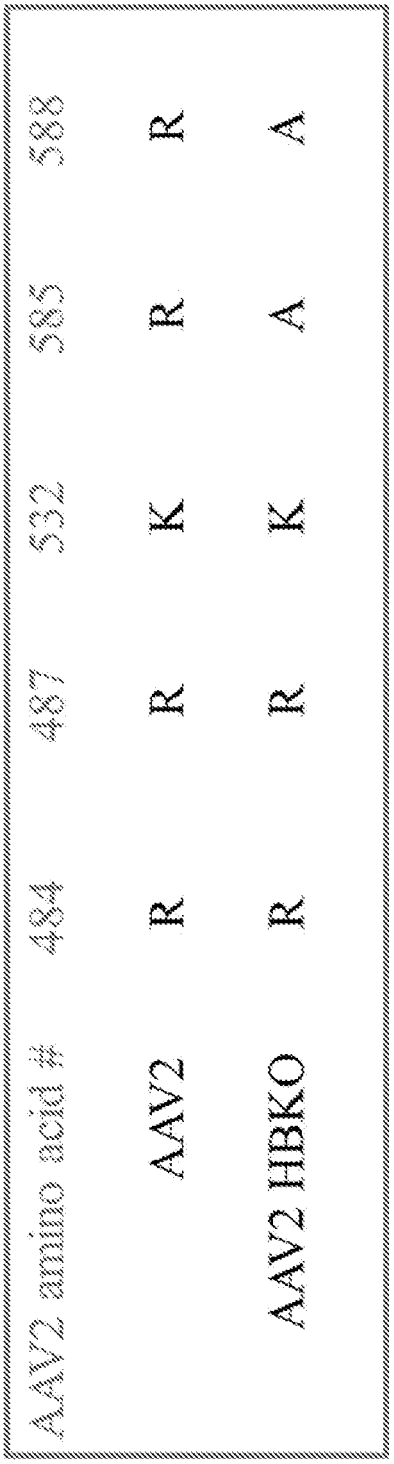


AAV2 amino acid #	484	487	532	583	588
AAV2	R	R	K	R	R
AAV2 HBKO	R	R	K	A	A

FIG. 1

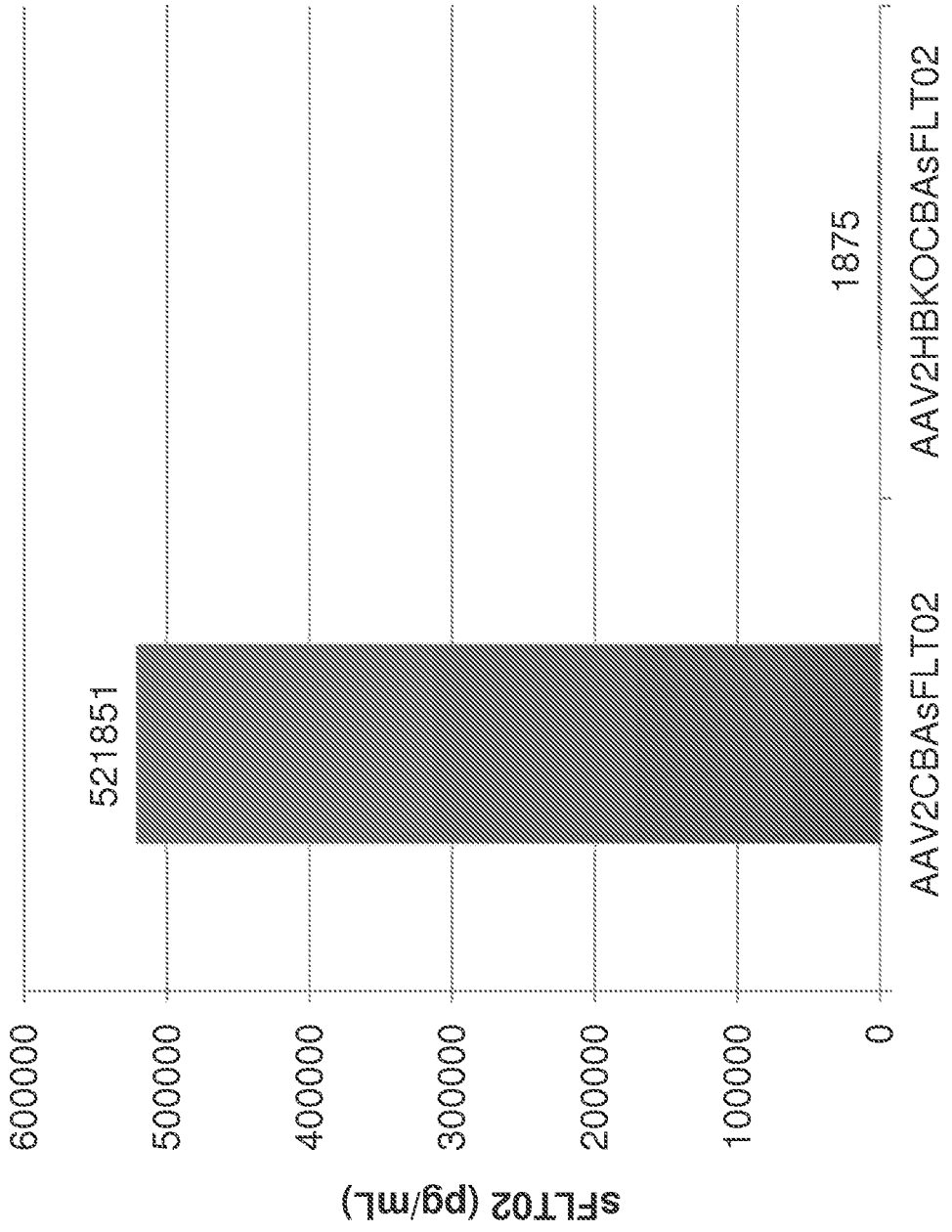

sFLT02 (pg/mL)
600000
500000
400000
300000
200000
100000
0
521851
1875
AAV2CBAsFLT02
AAV2HBKOCBAsFLT02

FIG. 2

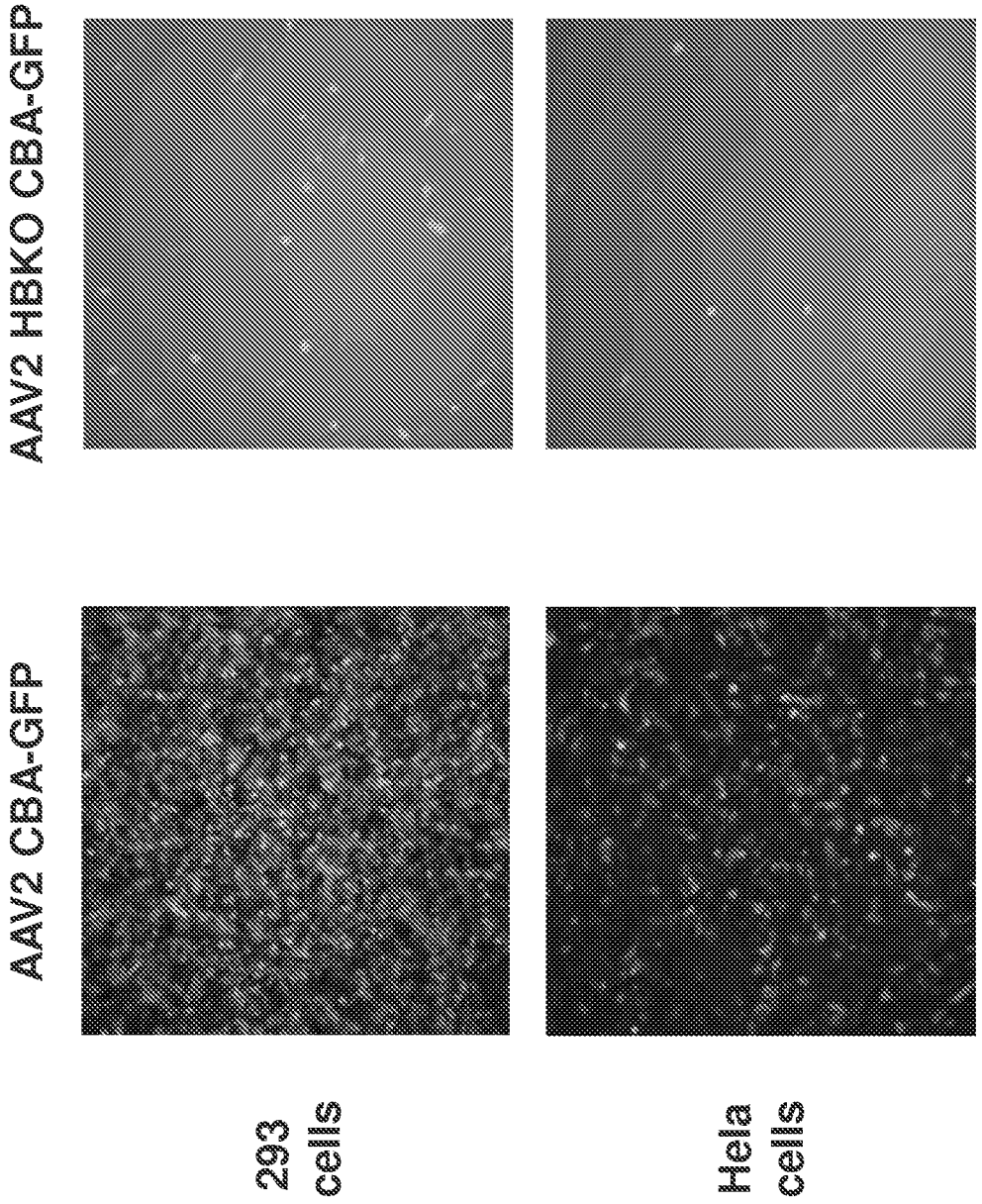
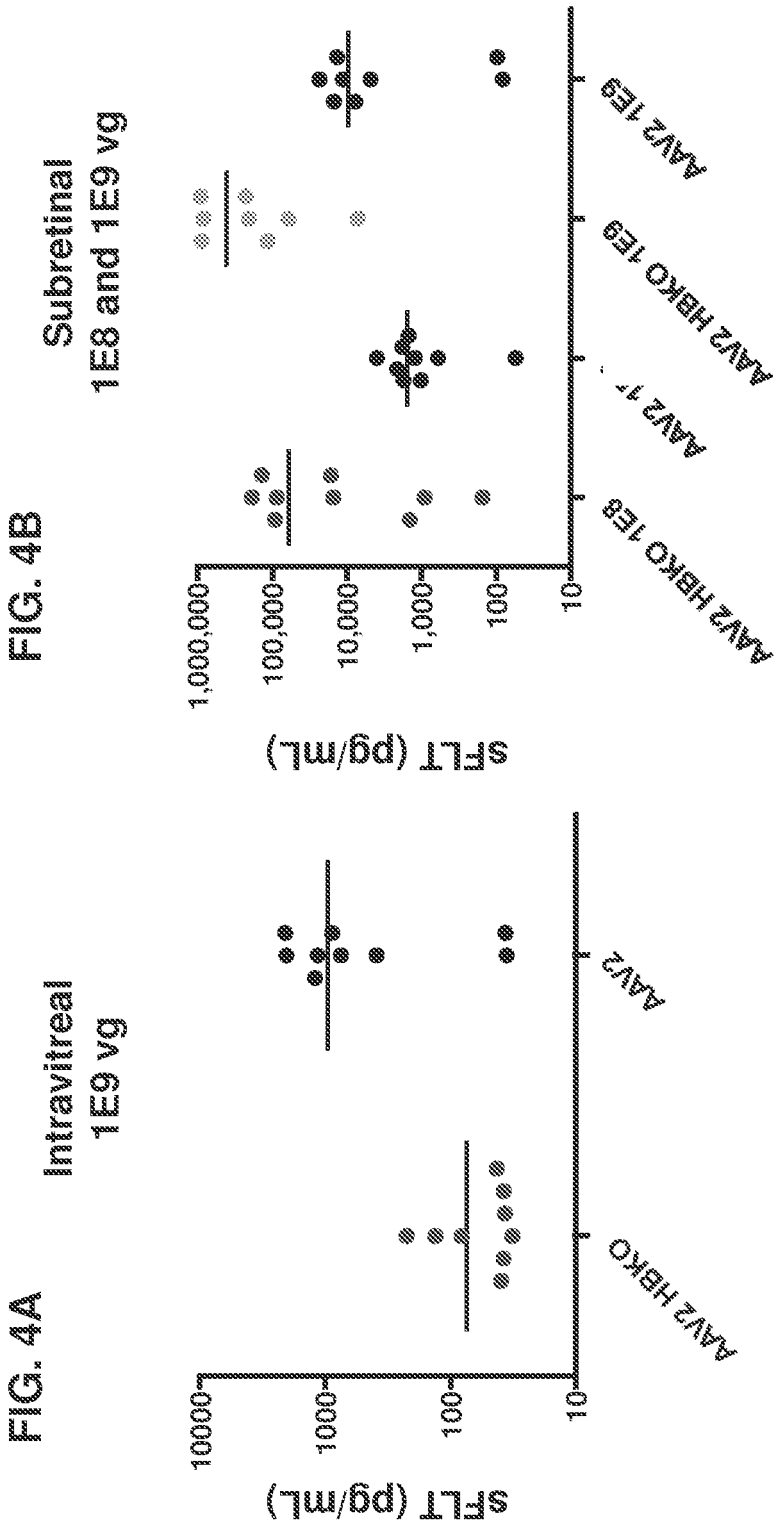


FIG. 3


FIG. 4A
Intravitreal
1E9 vg
sFLT (pg/mL)
10000
1000
100
10
AAV2
AAV2 HBKO
FIG. 4B
Subretinal
1E8 and 1E9 vg
sFLT (pg/mL)
1,000,000
100,000
10,000
1,000
100
10
AAV2 HBKO 1E8
AAV2
AAV2 HBKO 1E9
AAV2 1E9

FIGS. 4A & 4B

Arginine mutations decrease AAV2 intravitreal transduction in mouse eye

AAV2-CBA-GFP

AAV2HBKO(ArginineKO)

No transduction

FIG. 5

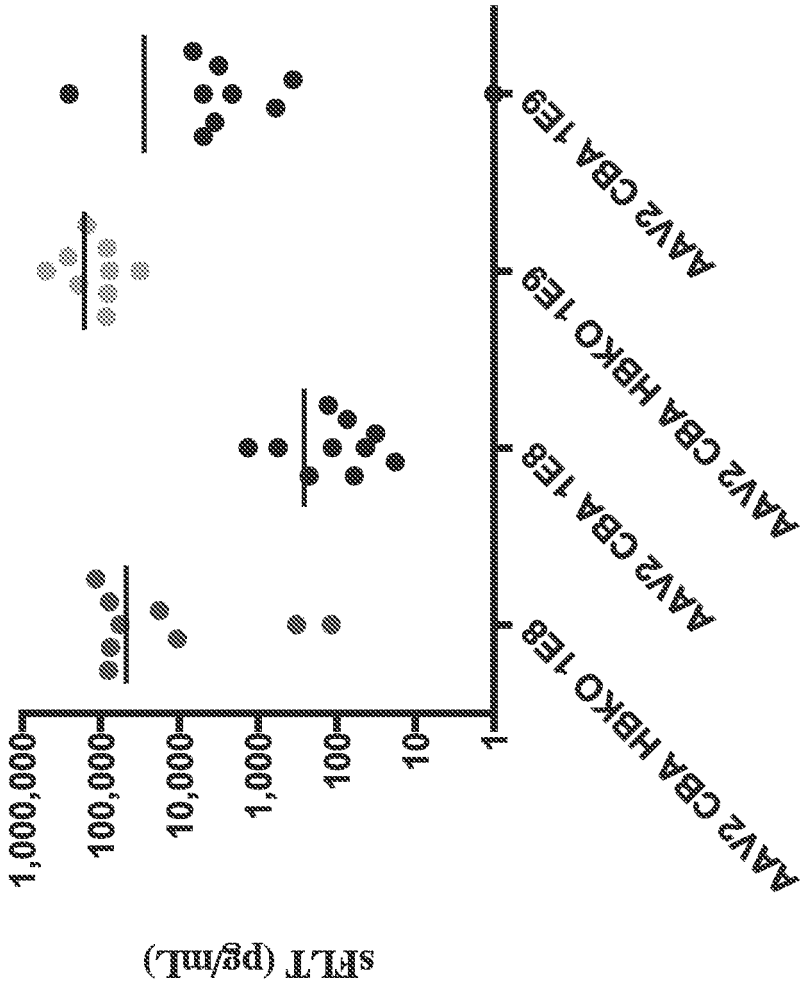

sFLT (pg/mL)
1,000,000
100,000
10,000
1,000
100
10
1
AAV2 CBA HBKO 1E9
AAV2 CBA 1E9
AAV2 CBA HBKO 1E9
AAV2 CBA 1E9

FIG. 6

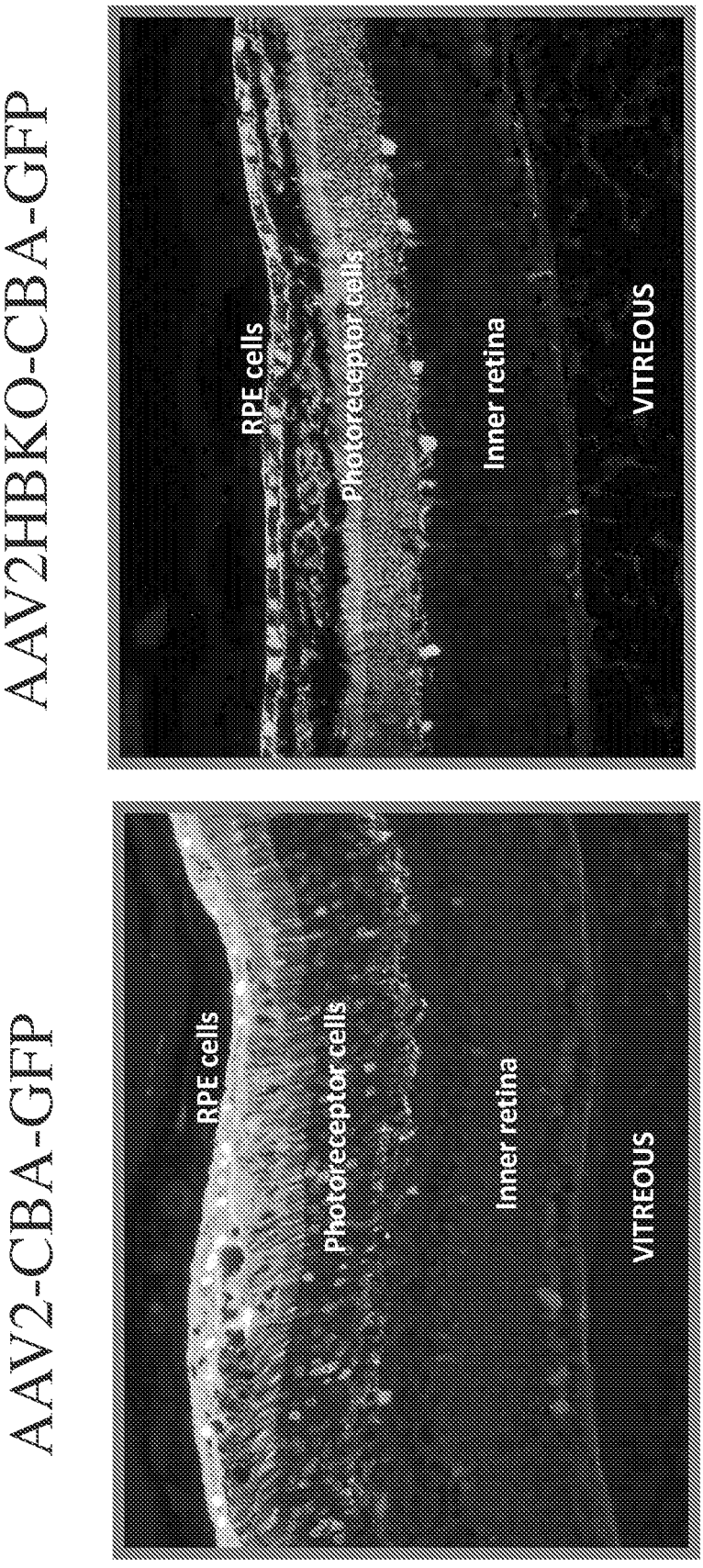

AAV2-CBA-GFP
RPE cells
Photoreceptor cells
Inner retina
VITREOUS
AAV2HBKO-CBA-GFP
RPE cells
Photoreceptor cells
Inner retina
VITREOUS

FIG. 7

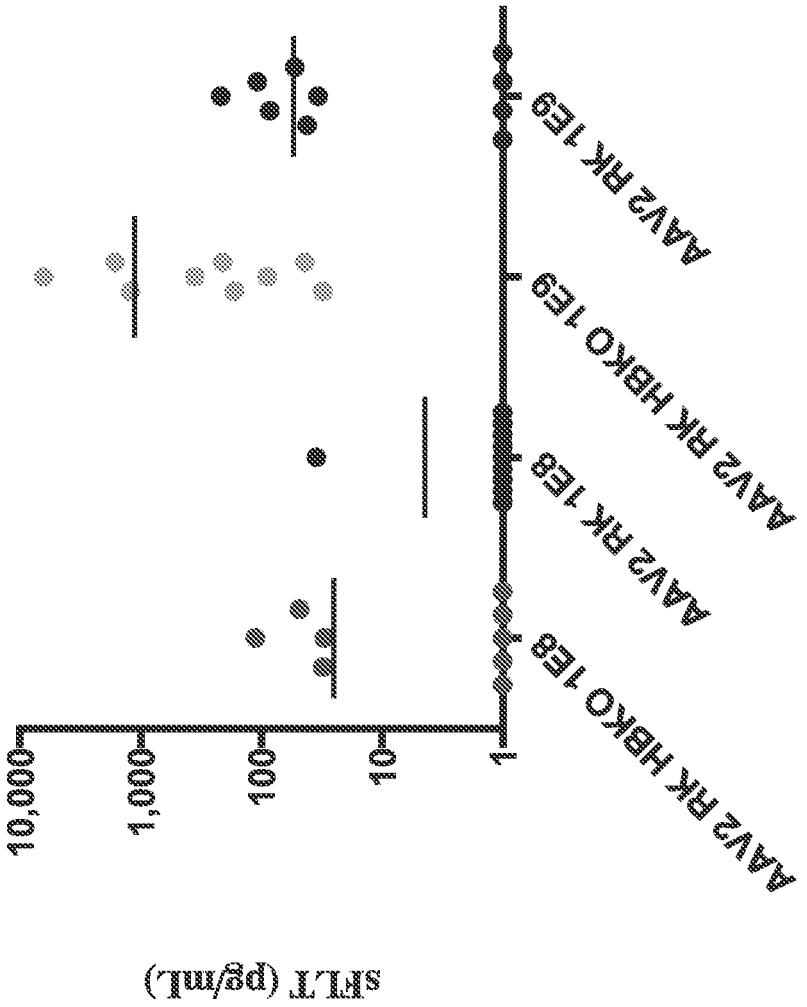

sFLT (pg/mL)
10,000
1,000
100
10
1
AAV2 RK 1E9
AAV2 RK HBKO 1E9
AAV2 RK 1E8
AAV2 RK HBKO 1E8

FIG. 8

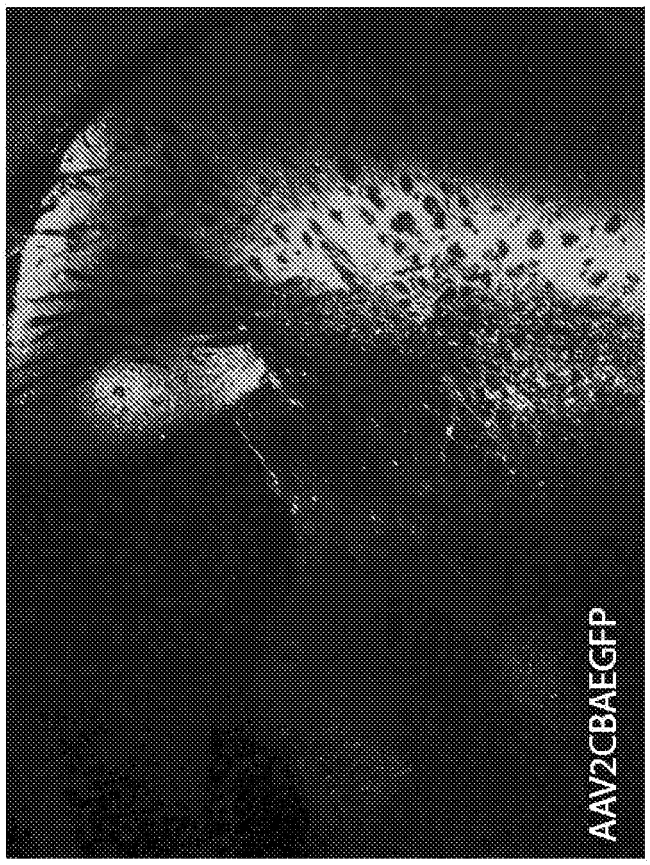


FIG. 9B

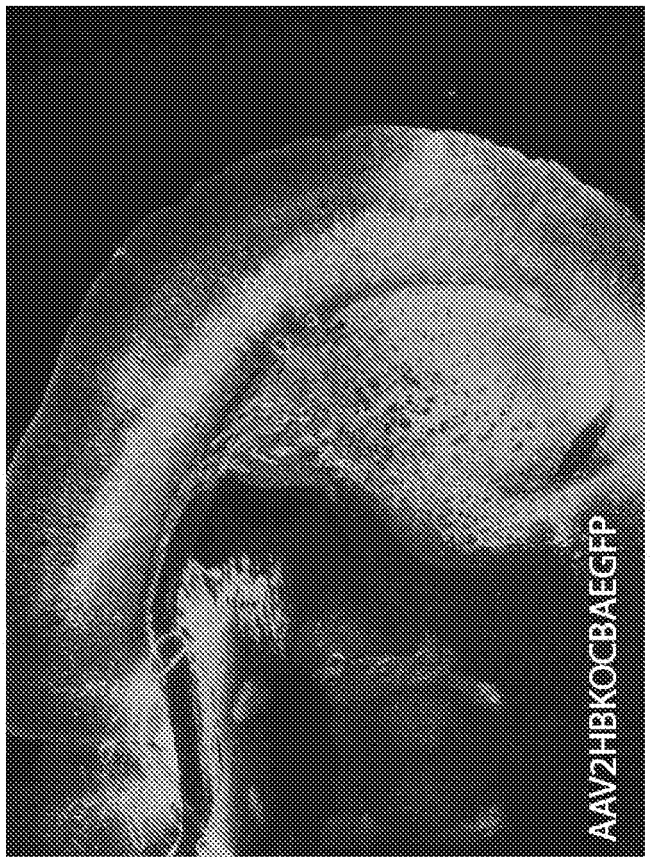
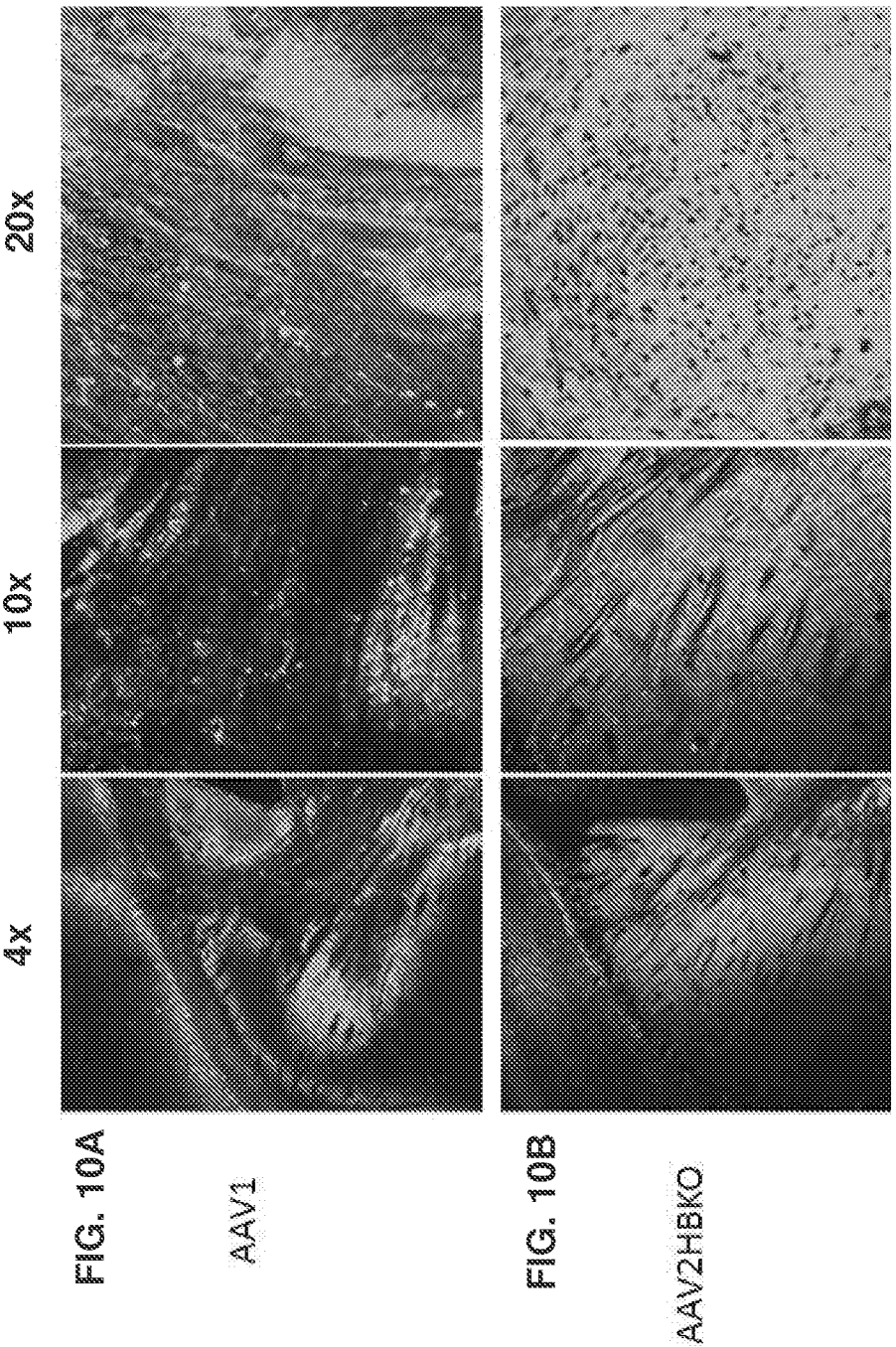


FIG. 9A

FIGS. 9A&9B



FIGS. 10A&10B

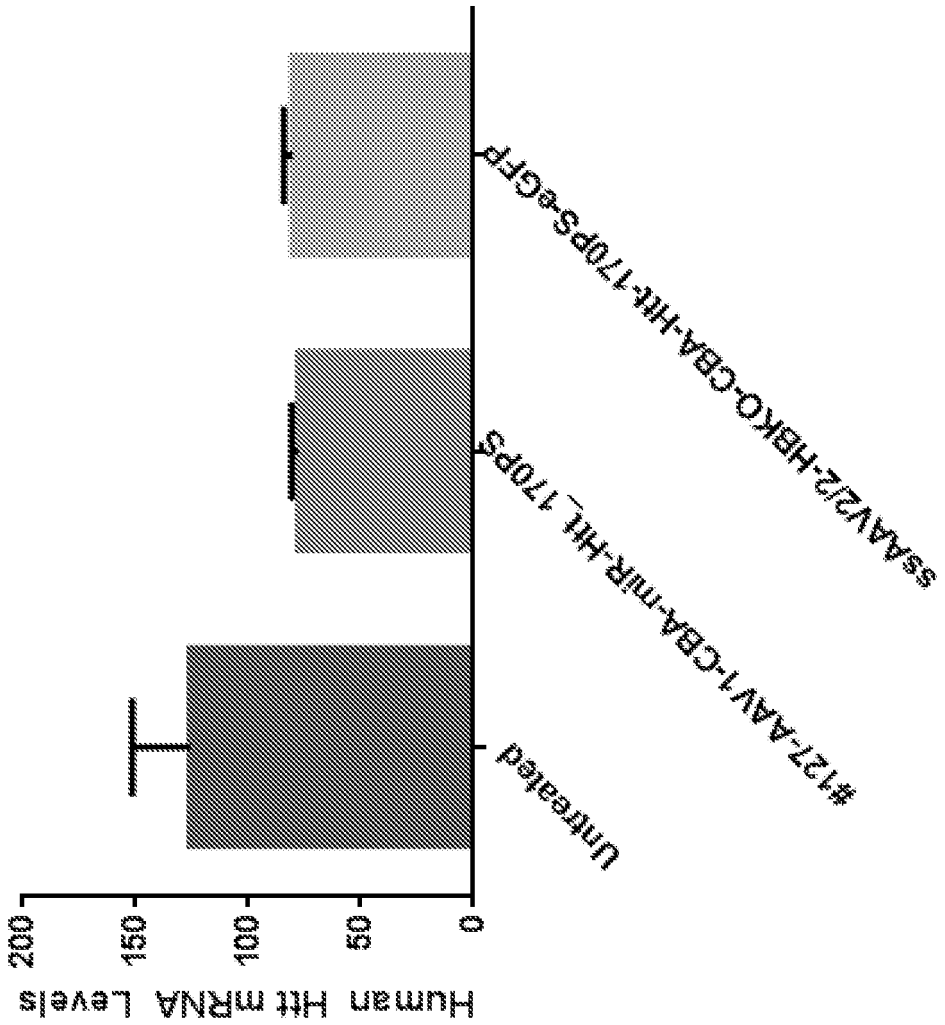

Human Htt mRNA Levels
200
150
100
50
0
Untreated
#127-AAV1-CBA-miR-Htt_170PS
ssAAV2/2-HBKO-CBA-Htt-170PS-eGFP

FIG. 11A

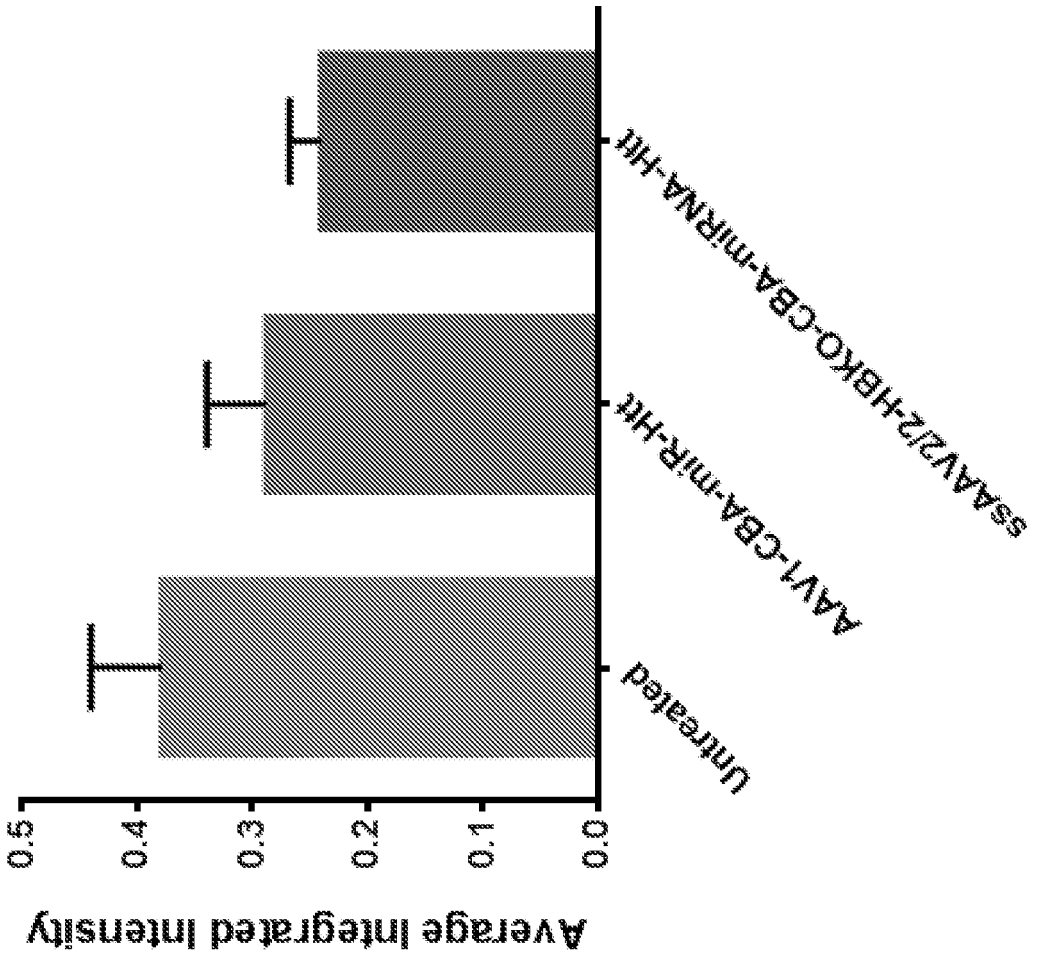

Average integrated intensity
0.0
0.1
0.2
0.3
0.4
0.5
untreated
AAV1-CBA-miR-HH
ssAAV2/2-HBKO-CBA-miRNA-HH

FIG. 11B

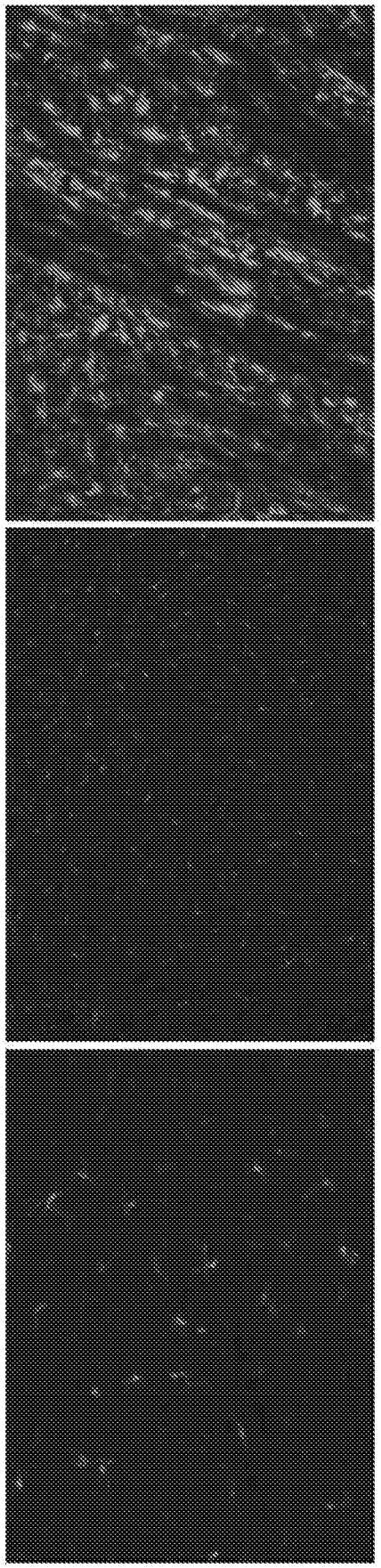
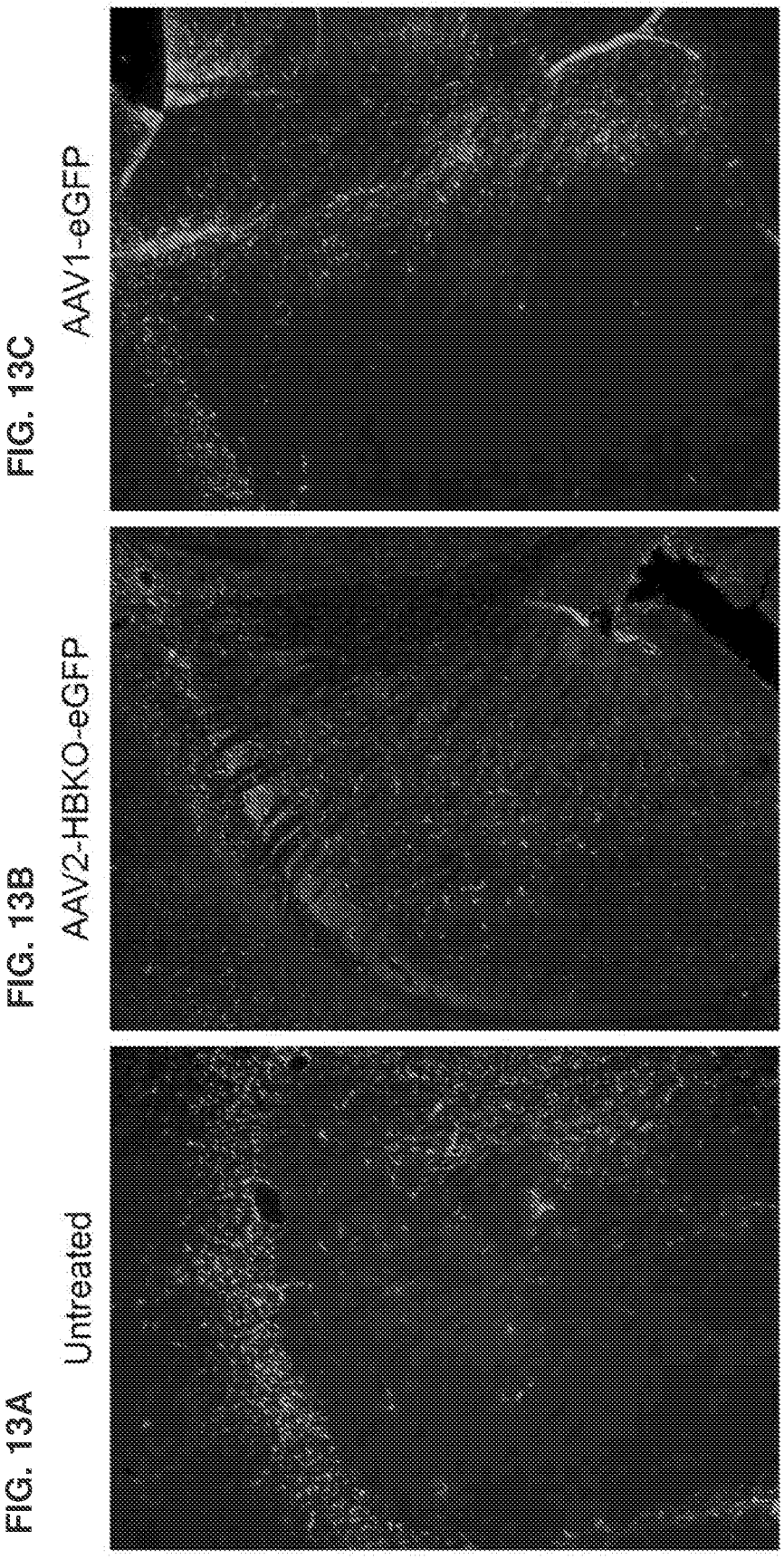


FIG. 12C

FIG. 12B

FIG. 12A

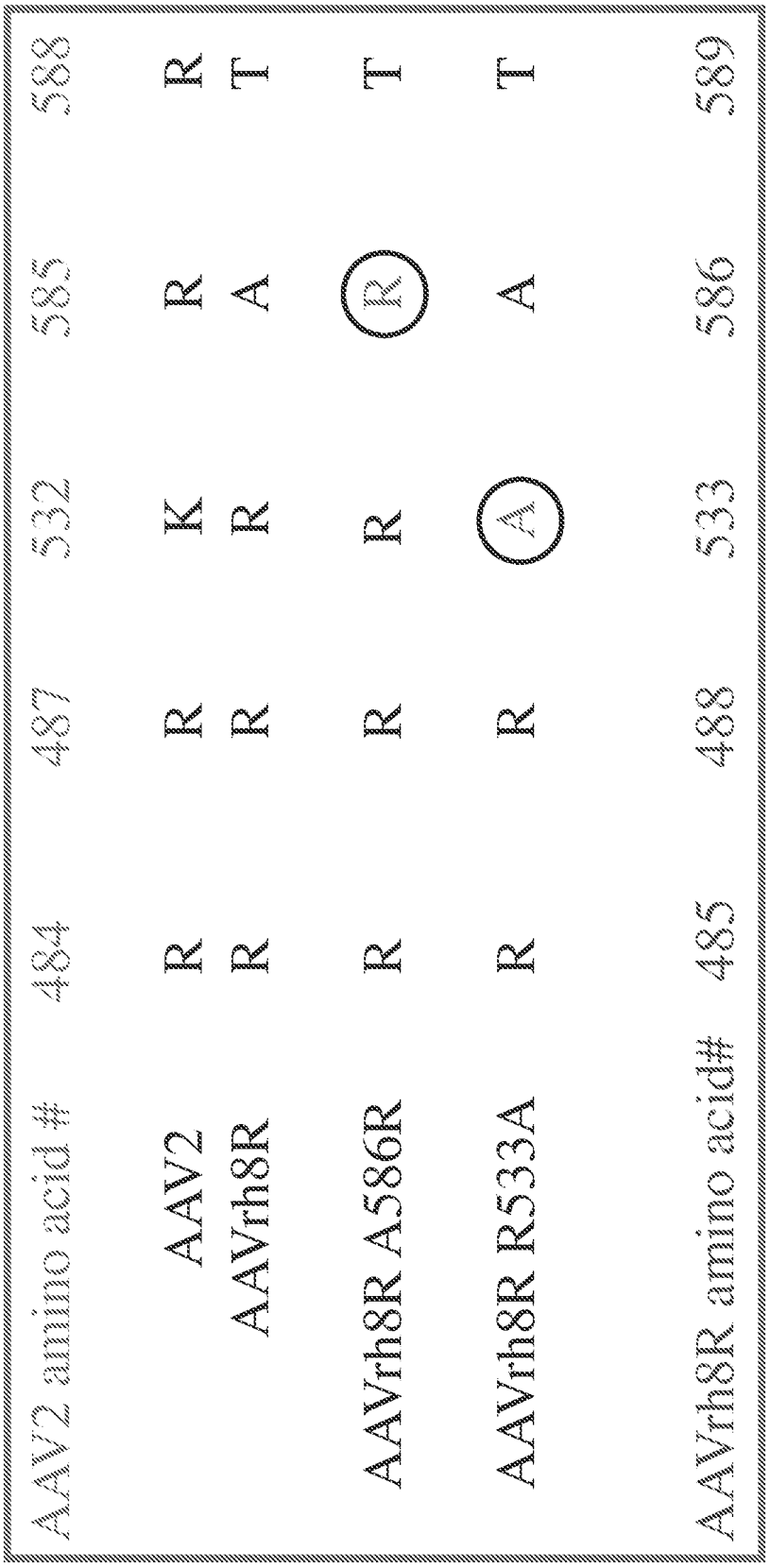
FIGS. 12A-12C



FIGS. 13A-13C

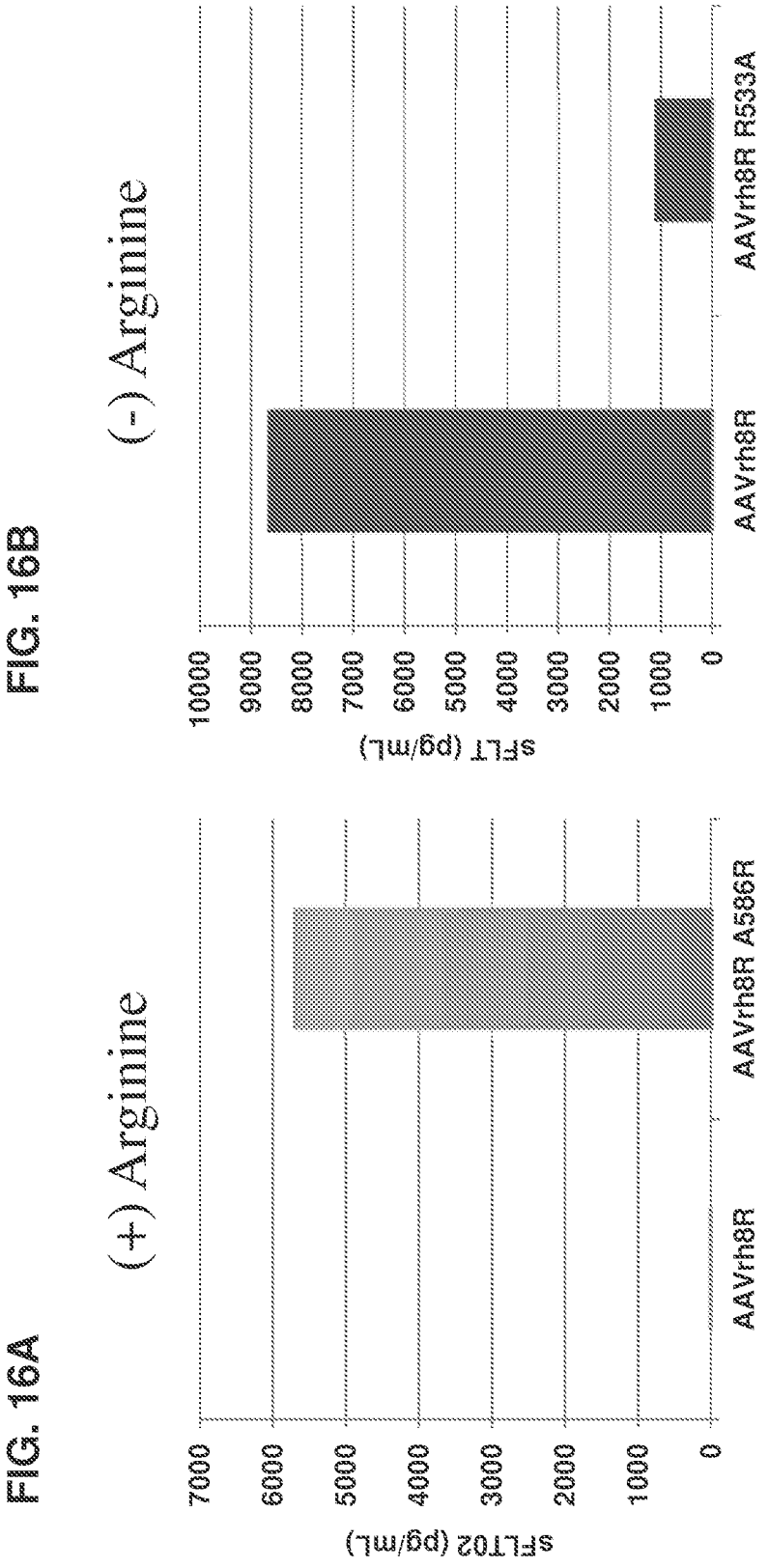
AAV2 amino acid #	484	487	532	585	588
AAV2	R	R	K	R	R
AAVrh8R	R	R	R	A	T
AAVrh8R amino acid#	485	488	533	586	589

FIG. 14

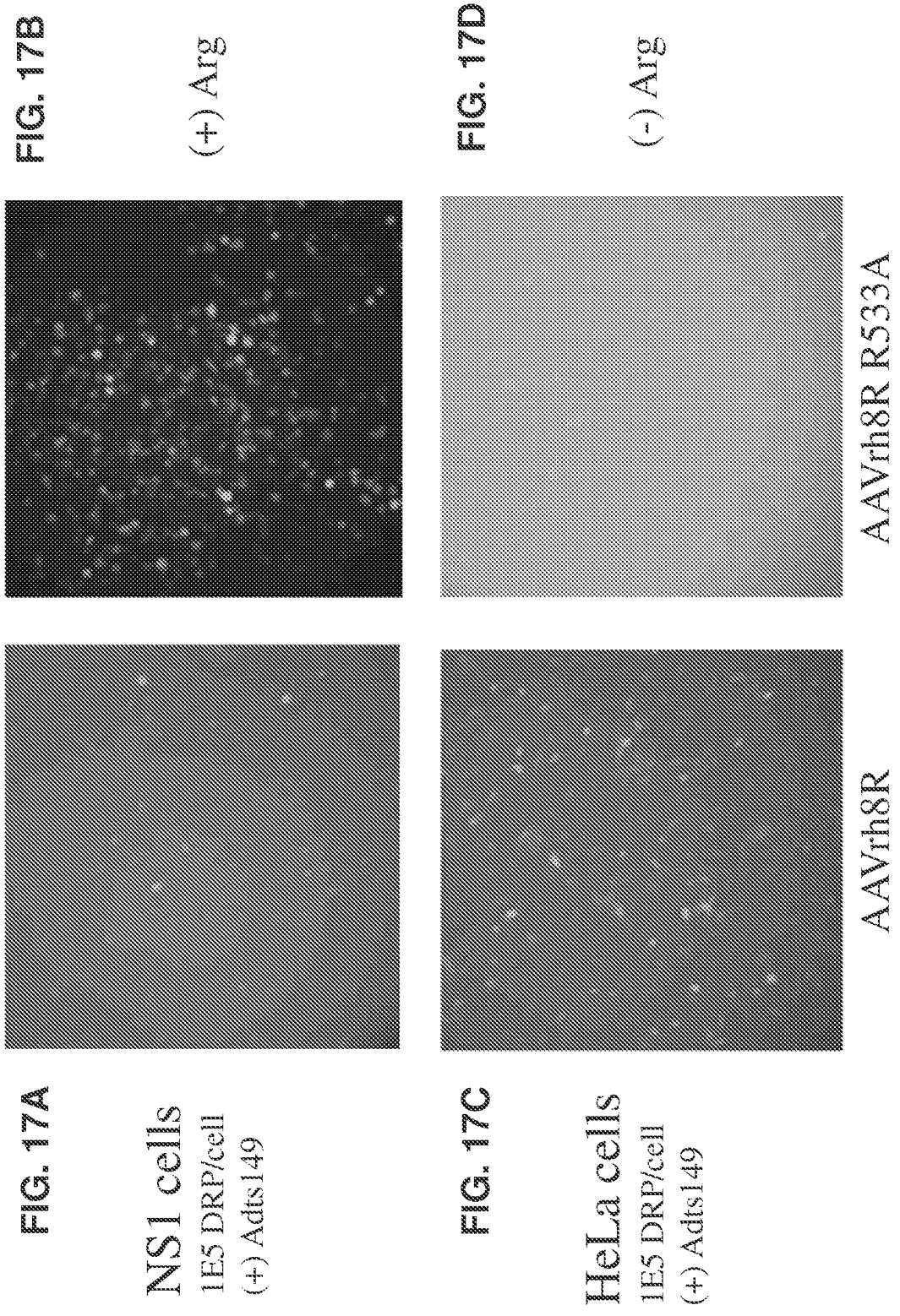


AAV2 amino acid #	484	487	532	585	588
AAV2	R	R	K	R	R
AAVrh8R	R	R	R	A	T
AAVrh8R A586R	R	R	R	R	T
AAVrh8R R533A	R	R	A	A	T
AAVrh8R amino acid#	485	488	533	586	589

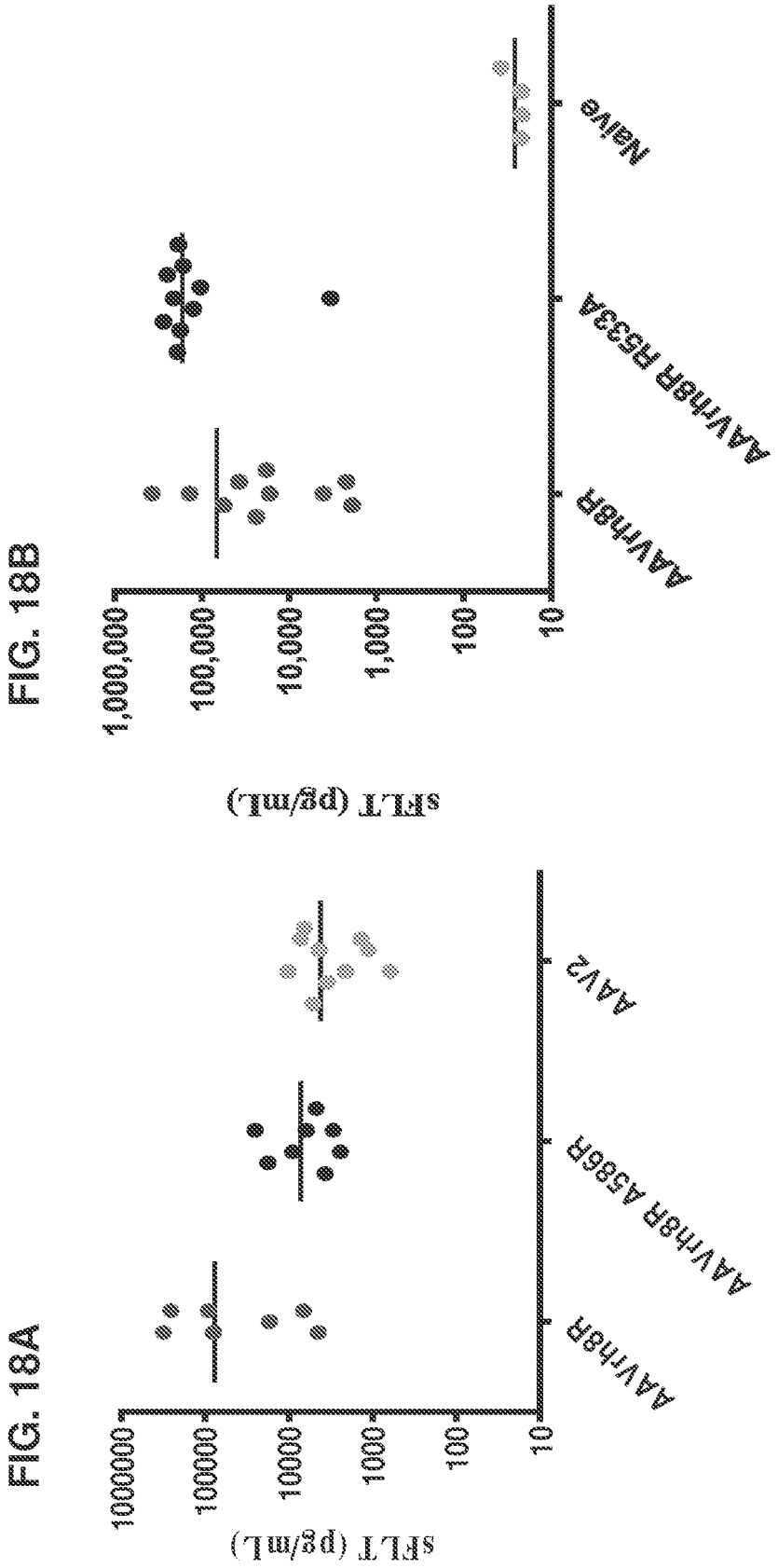
FIG. 15


FIG. 16A
(+) Arginine
sFLT02 (pg/mL)
7000
6000
5000
4000
3000
2000
1000
0
AAVrh8R
AAVrh8R A586R
HeLa
1 x 10⁴ DRP/cell
FIG. 16B
(-) Arginine
sFLT (pg/mL)
10000
9000
8000
7000
6000
5000
4000
3000
2000
1000
0
AAVrh8R
AAVrh8R R533A
HeLaRC32
1 x 10⁴ DRP/cell

FIGS. 16A & 16B


FIG. 17A
AAVrh8R
NS1 cells
1E5 DRP/cell
(+) Adts149
FIG. 17B
AAVrh8R A586R
(+) Arg
FIG. 17C
AAVrh8R
HeLa cells
1E5 DRP/cell
(+) Adts149
FIG. 17D
AAVrh8R R533A
(-) Arg

FIGS. 17A-17D


FIG. 18B
sFLT (pg/mL)
1,000,000
100,000
10,000
1,000
100
10
AAVrh8R
AAVrh8R R533A
Naive
FIG. 18A
sFLT (pg/mL)
1000000
100000
10000
1000
100
10
AAVrh8R
AAVrh8R A586R
AAV2

FIGS. 18A & 18B

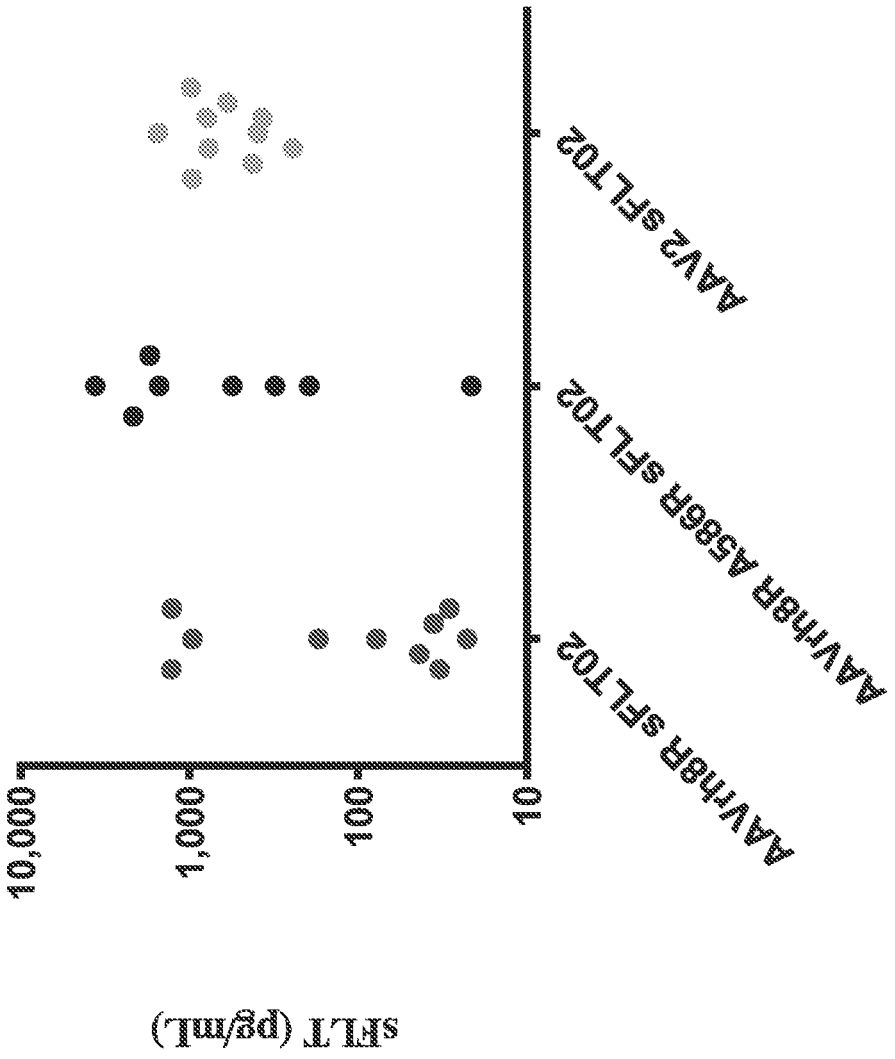

sFLT (pg/mL)
10,000
1,000
100
10
AAVhBR sFLT02
AAVhBR A580R sFLT02
AAV2 sFLT02

FIG. 19

AAV	amino acid #	484	487	527	532	585	588
	AAV2	R	R	K	K	R	R
AAV	amino acid #	485	488	528	533	586	589
	AAV1	R	R	K	K	S	T
	AAV6	R	R	K	K	S	T
	AAV9	R	R	K	R	S	A
	AAVrh8R	R	R	K	R	A	T
AAV	amino acid #	487	490	530	535	588	591
	AAV8	R	R	K	R	Q	T
	AAVrh10	R	R	K	R	Q	A

FIG. 20