AAV2 amino acid #	484	487	532	585	588
AAV2	R	R	K	R	R
AAV2 HBKO	R	R	K	A	A

FIG. 1

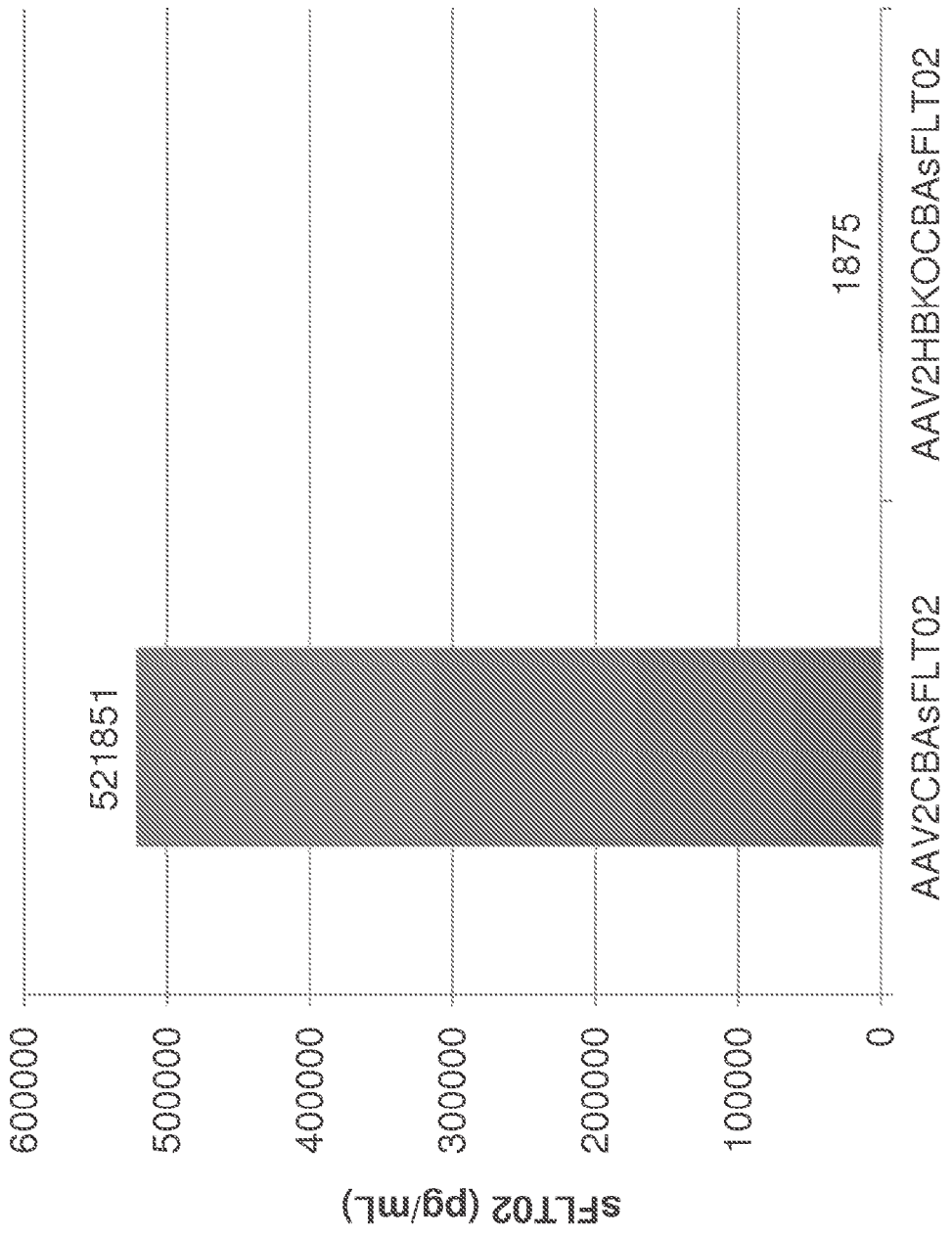

sFLT02 (pg/mL)
600000
500000
400000
300000
200000
100000
0
521851
1875
AAV2CBAsFLT02
AAV2HBKOCBAsFLT02

FIG. 2

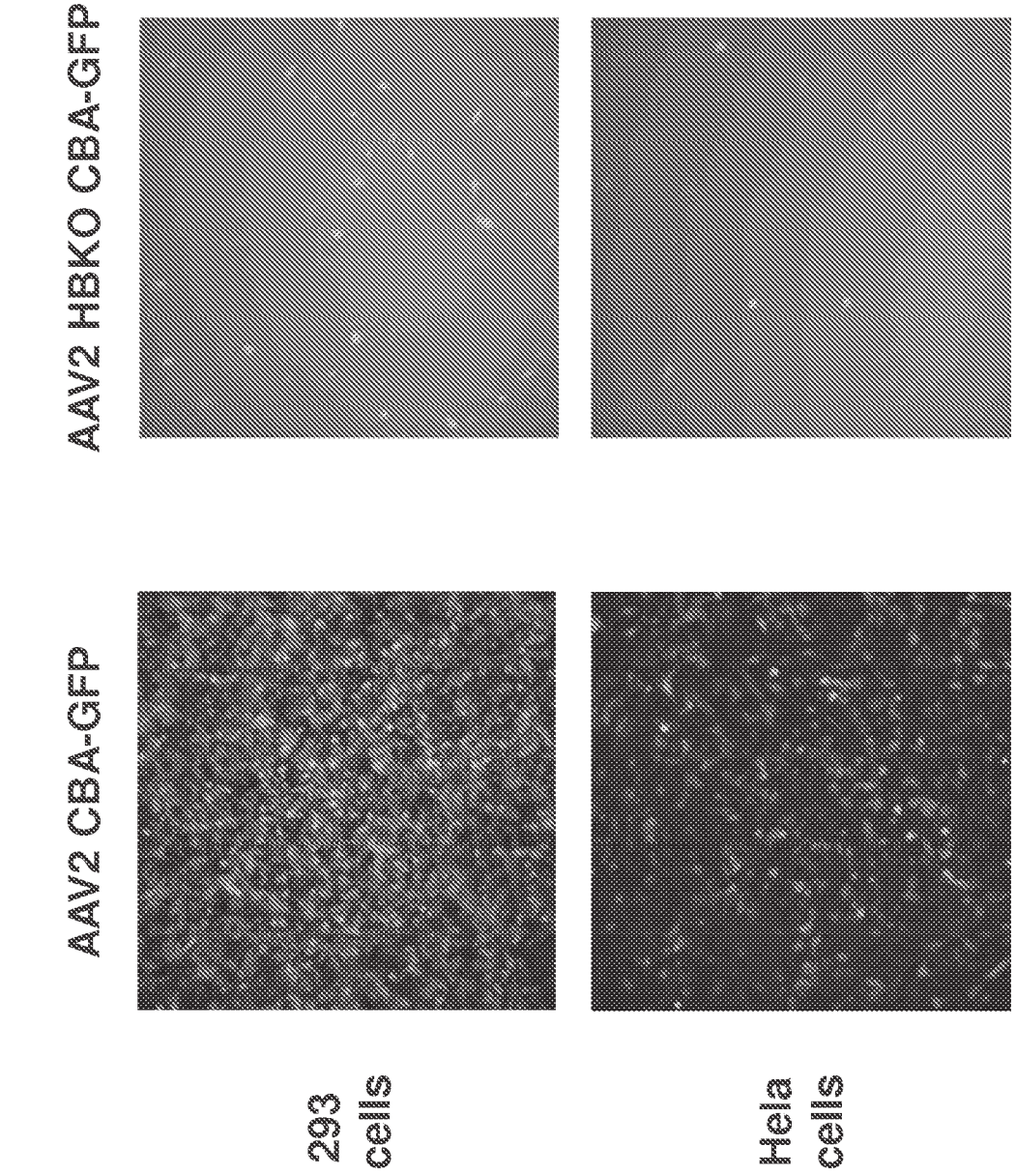


FIG. 3

FIG. 4A

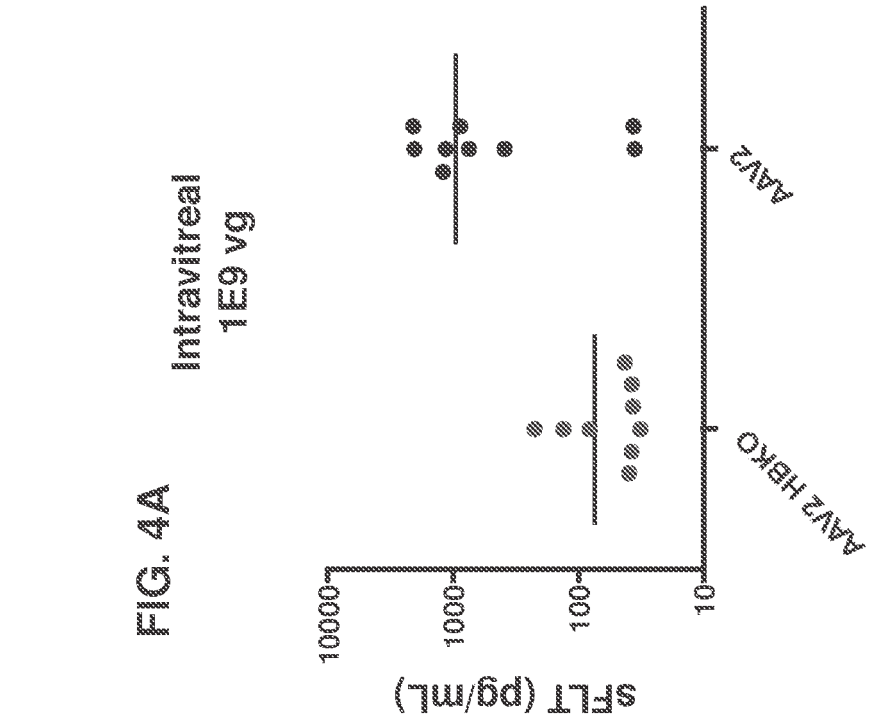
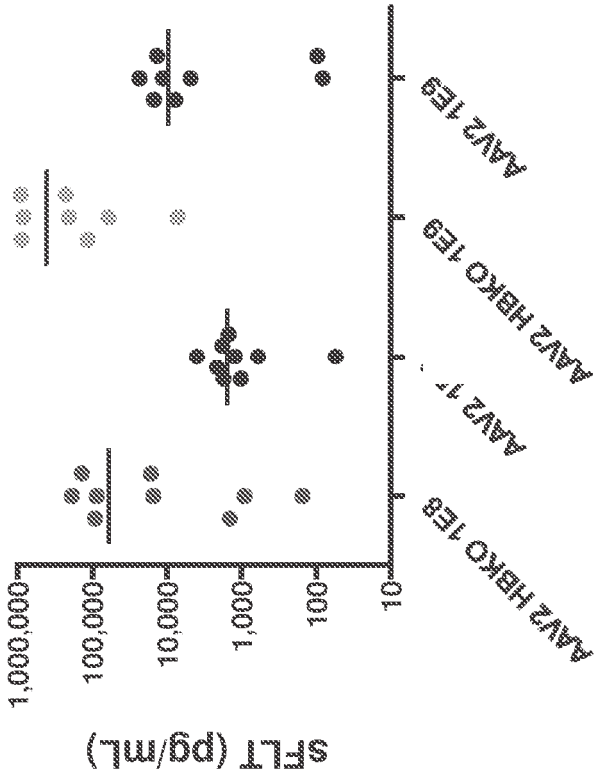
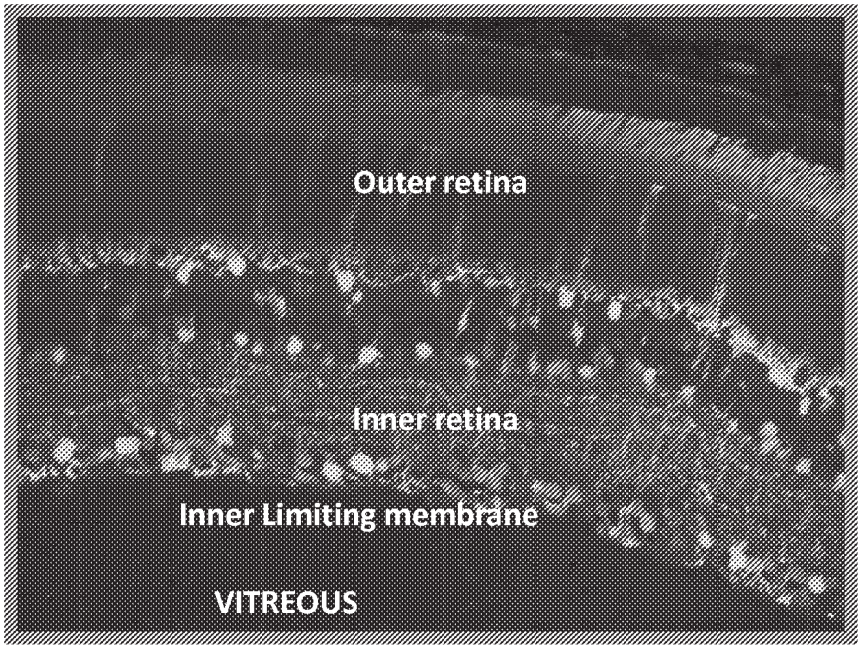

Intravitreal
1E9 vg
sFLT (pg/mL)
10000
1000
100
10
AAV2
AAV2 HBKO

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 1E8
AAV2 HBKO 1E9
AAV2 1E9

Arginine mutations decrease AAV2 intravitreal transduction in mouse eye

AAV2-CBA-GFP



AAV2HBKO(ArginineKO)

No transduction

FIG. 5

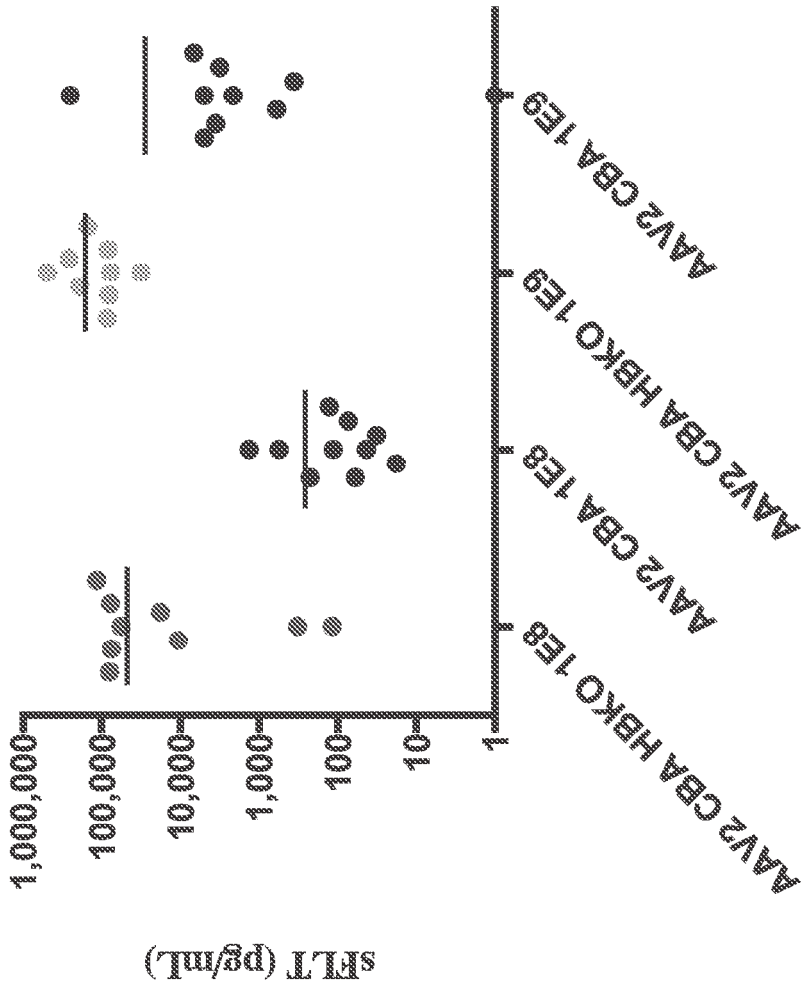
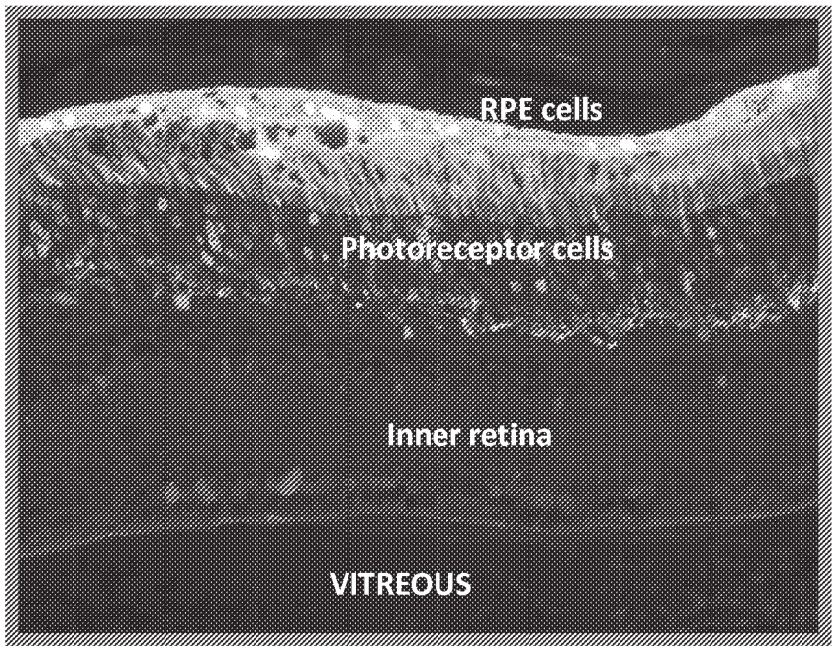

sFLT (pg/mL)
1,000,000
100,000
10,000
1,000
100
10
1
AAV2 CBA 1E9
AAV2 CBA HBKO 1E9
AAV2 CBA 1E8
AAV2 CBA HBKO 1E8

FIG. 6

AAV2-CBA-GFP



AAV2HBKO-CBA-GFP

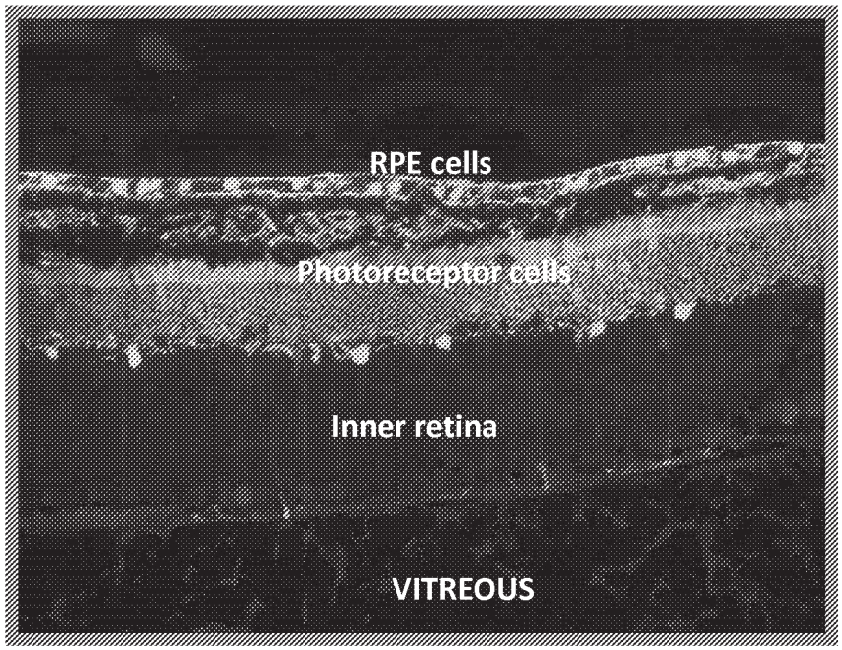


FIG. 7

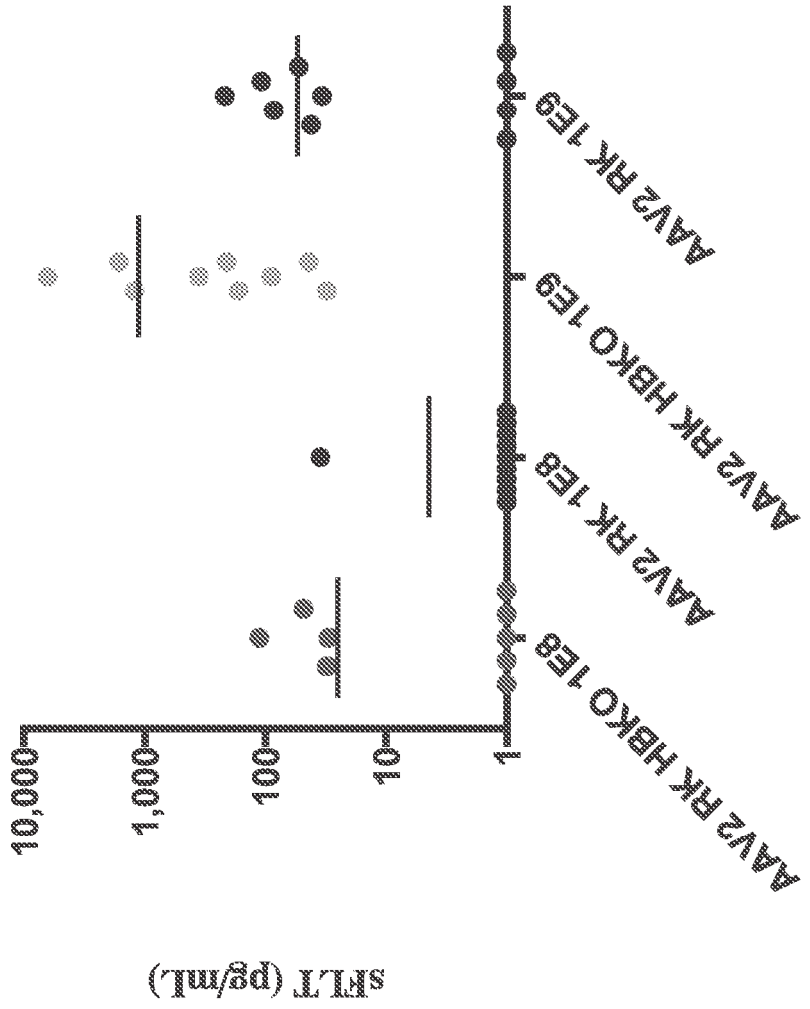

sFLT (pg/mL)
10,000
1,000
100
10
1
AAV2 RK HBKO 1E8
AAV2 RK 1E8
AAV2 RK HBKO 1E9
AAV2 RK 1E9

FIG. 8

FIG. 9A

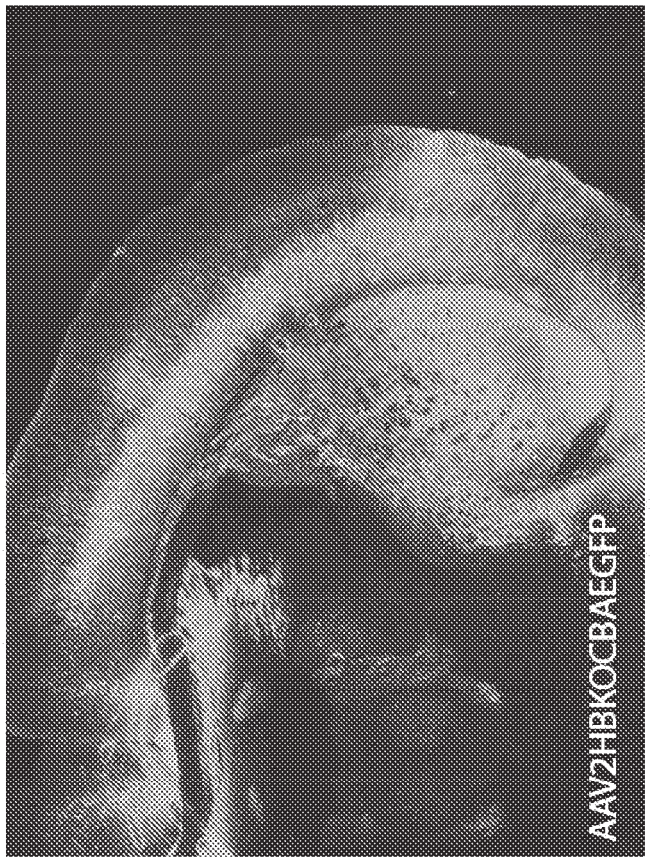
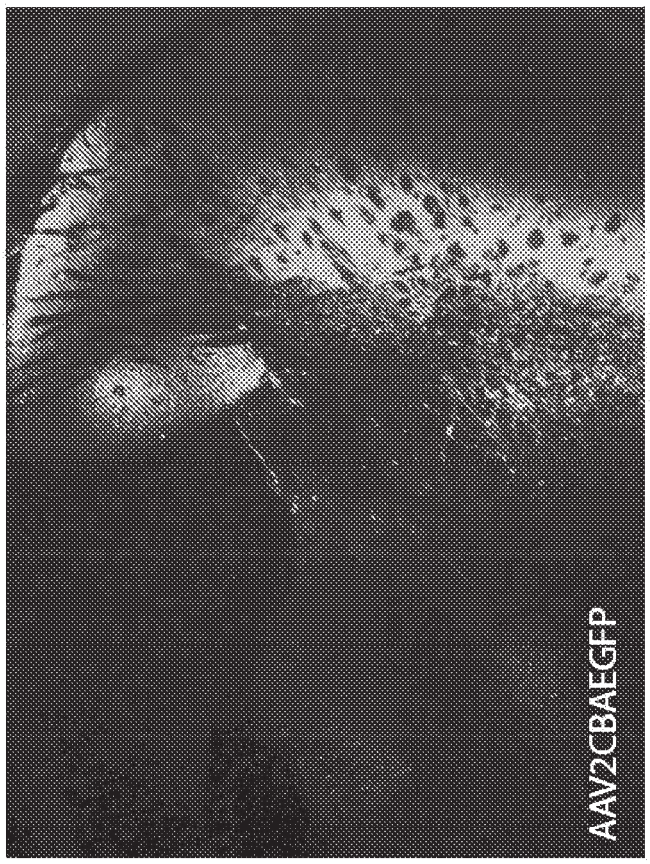


FIG. 9B



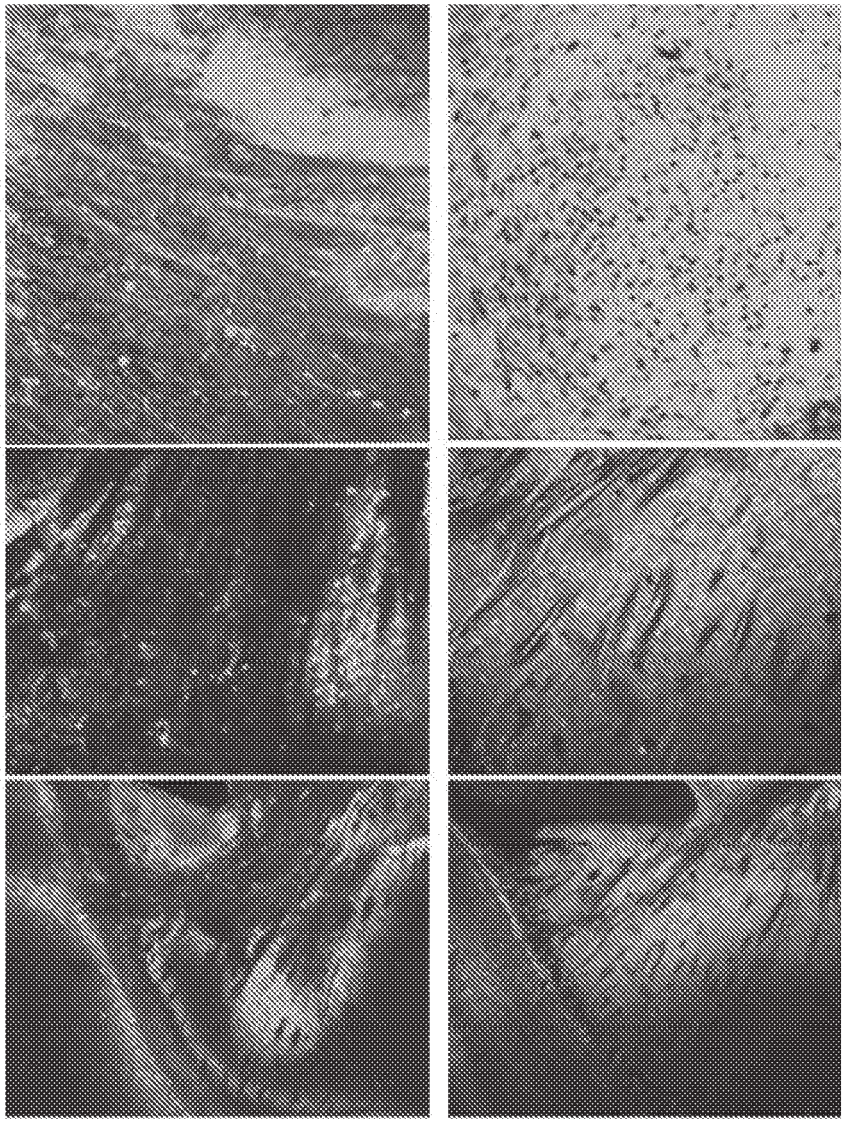


FIG. 10A

FIG. 10B

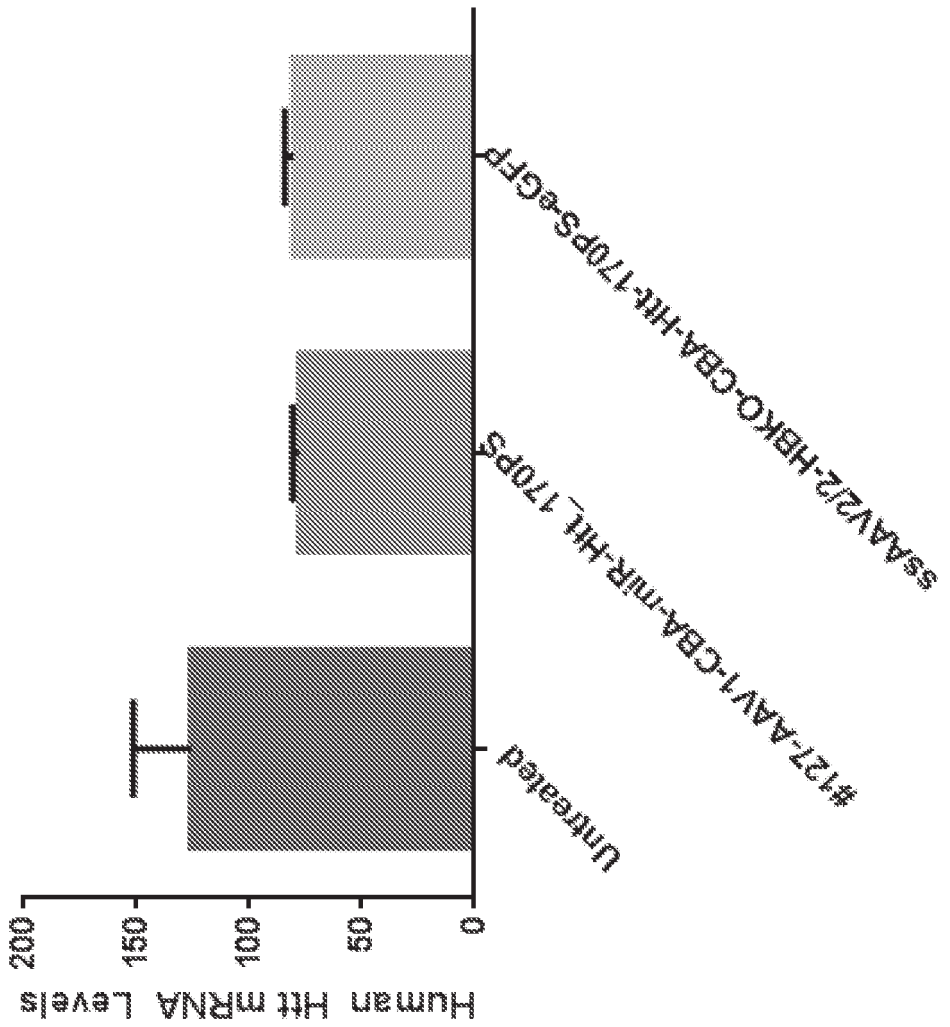

Human Htt mRNA Levels
200
150
100
50
0
untreated
#127-AAV1-CBA-miR-hHL_170P5
ssAAV2/2-HBKO-CBA-hHL-170P5-eGFP

FIG. 11A

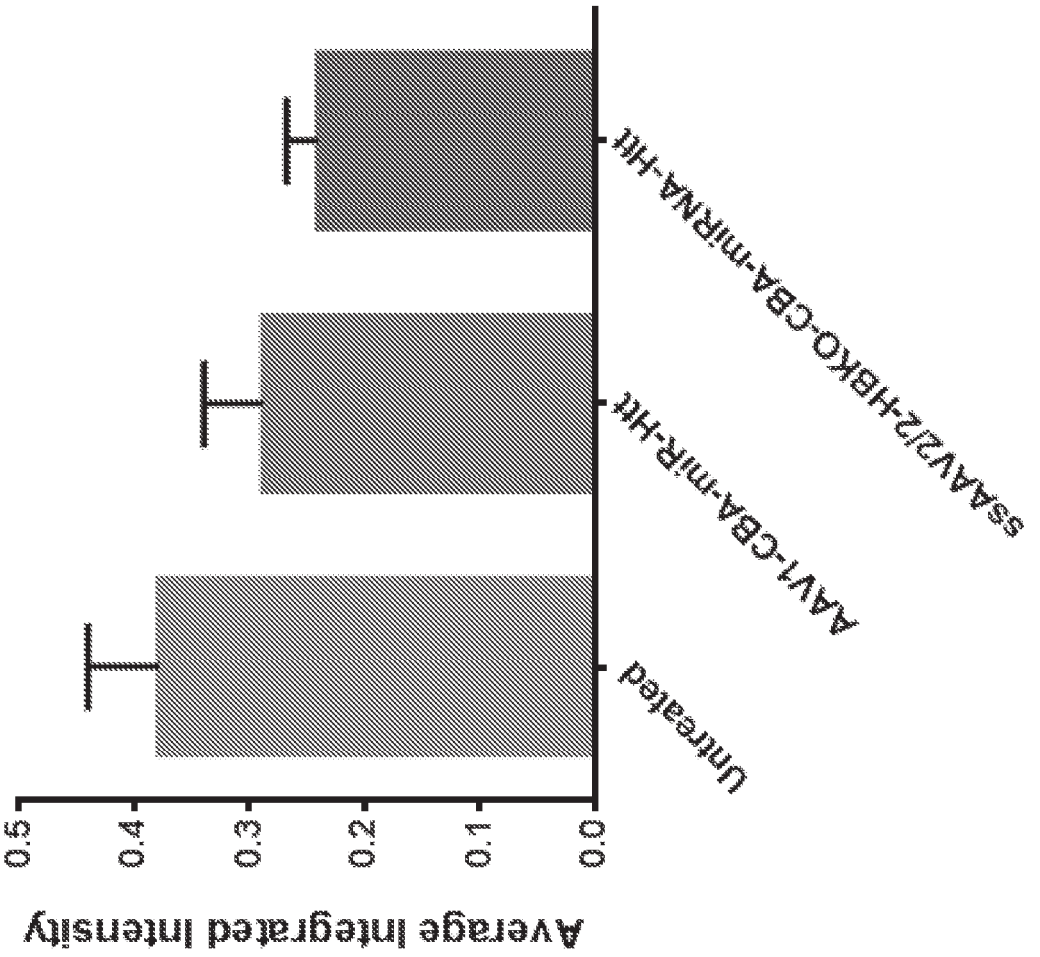

Average integrated intensity
0.0
0.1
0.2
0.3
0.4
0.5
Untreated
AAV1-CBA-miR-Htt
ssAAV2i2-HBKO-CBA-miRNA-Htt

FIG. 11B

FIG. 12A



FIG. 12B



FIG. 12C

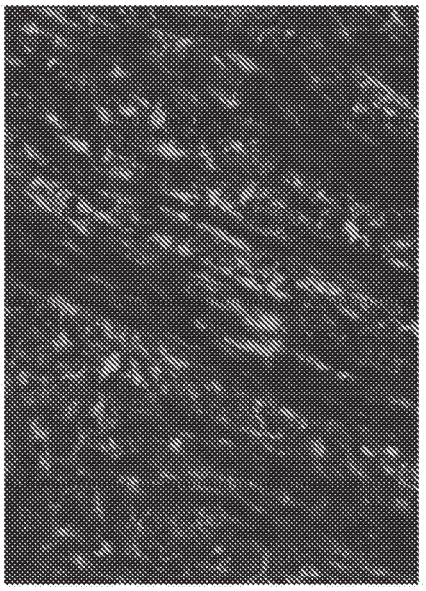


FIG. 13A

Untreated

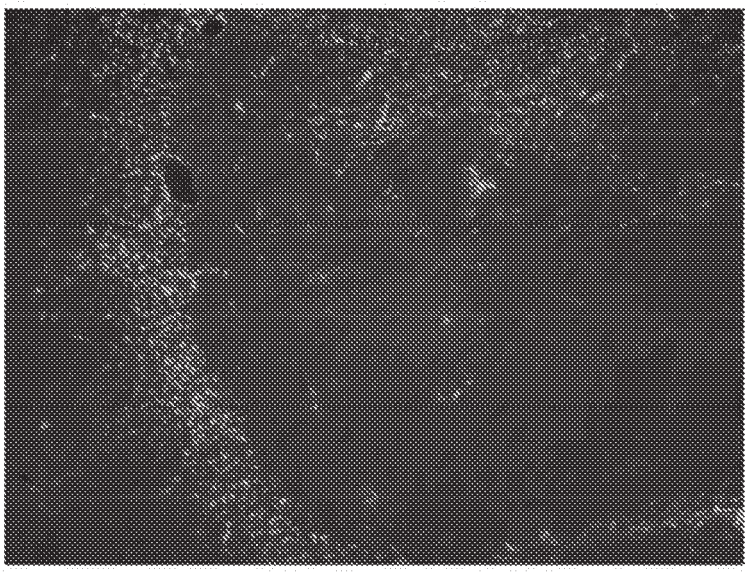


FIG. 13B

AAV2-HBKO-eGFP

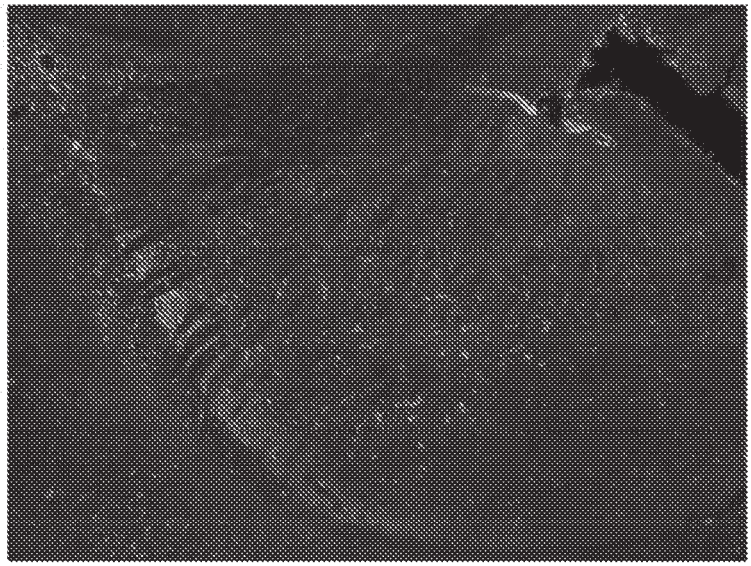
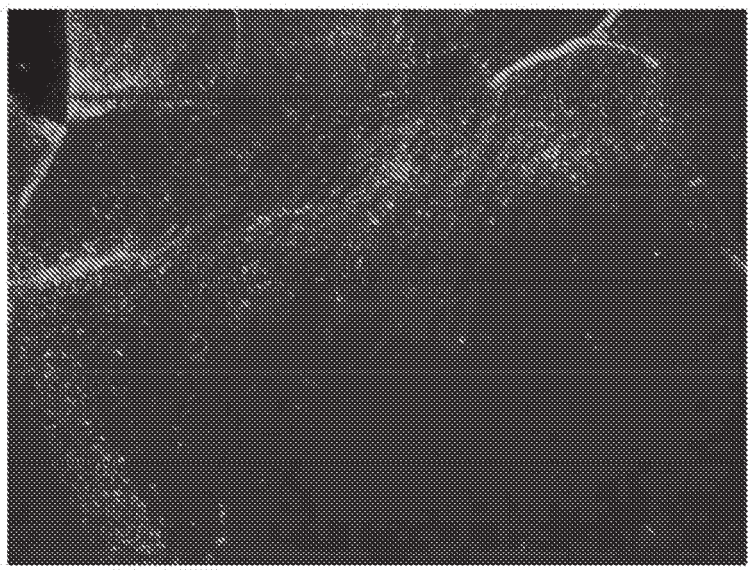


FIG. 13C

AAV1-eGFP



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

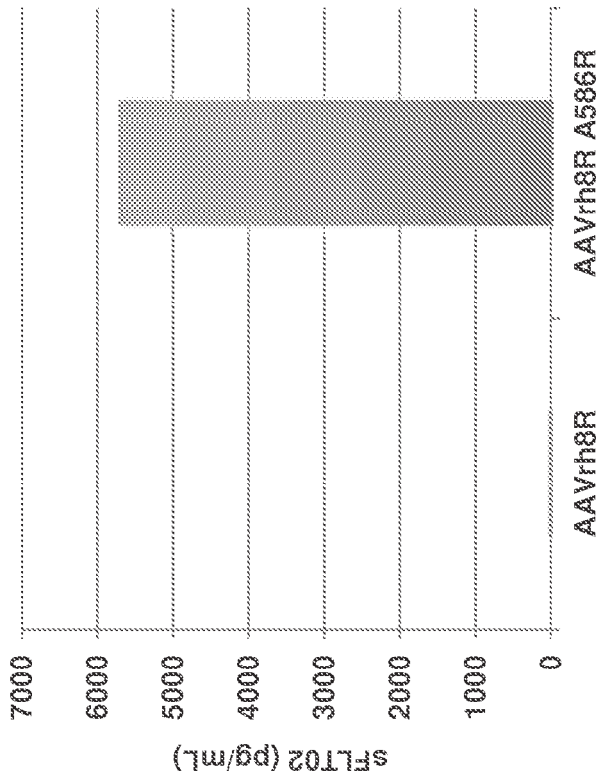
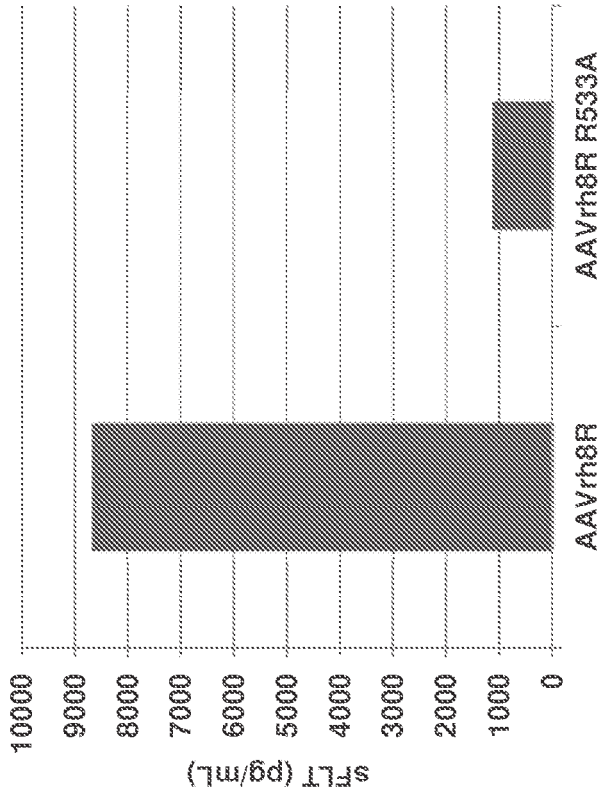


FIG. 16B

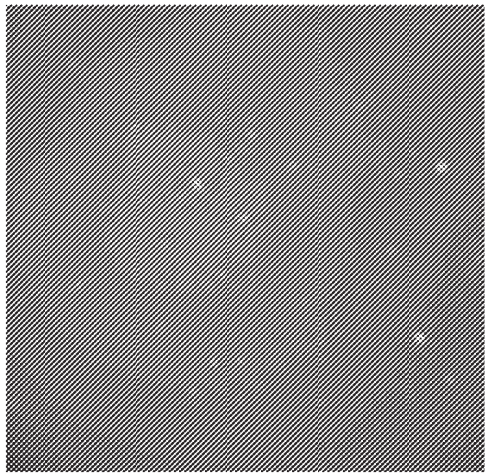


AAVrh8R

AAVrh8R A586R

FIG. 17A

NS1 cells
1E5 DRP/cell
(+) Adts149



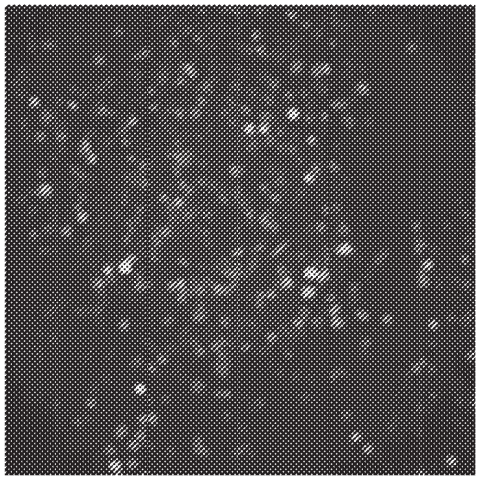
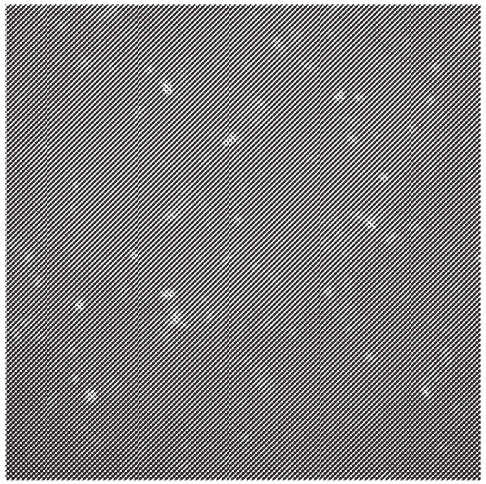


FIG. 17B

(+) Arg

FIG. 17C

HeLa cells
1E5 DRP/cell
(+) Adts149



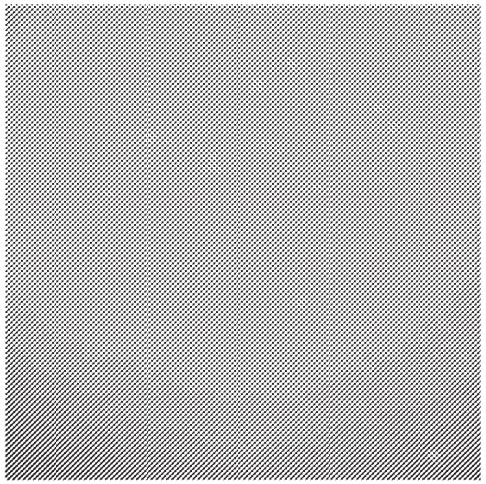


FIG. 17D

(-) Arg

AAVrh8R

AAVrh8R R533A

FIG. 18A

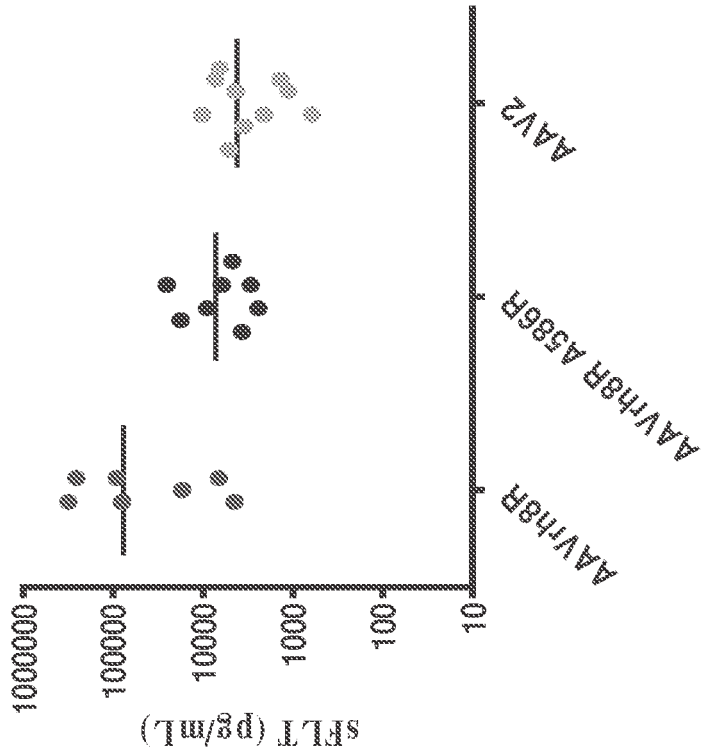
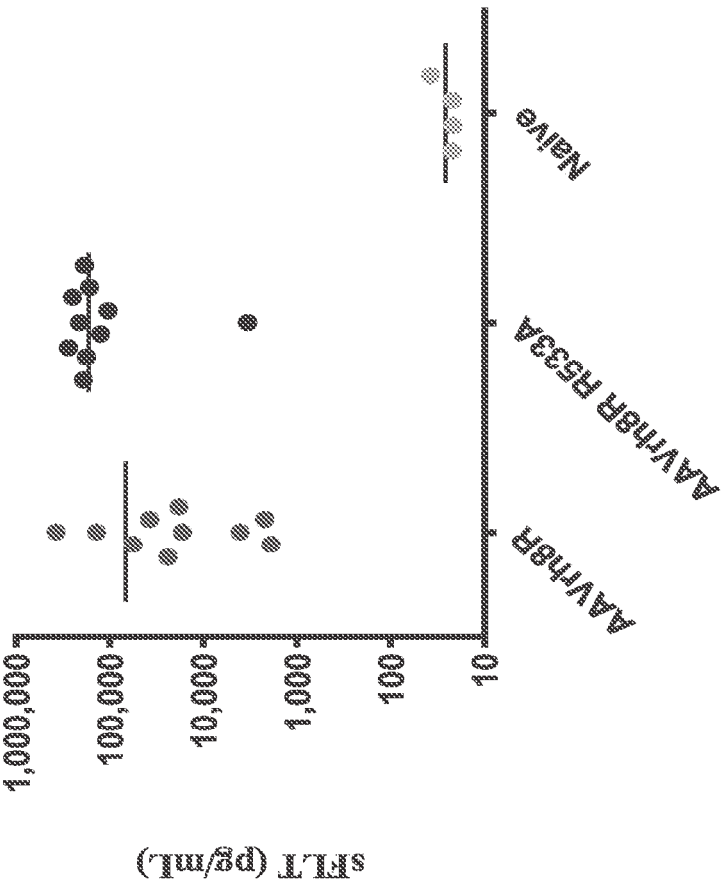


FIG. 18B



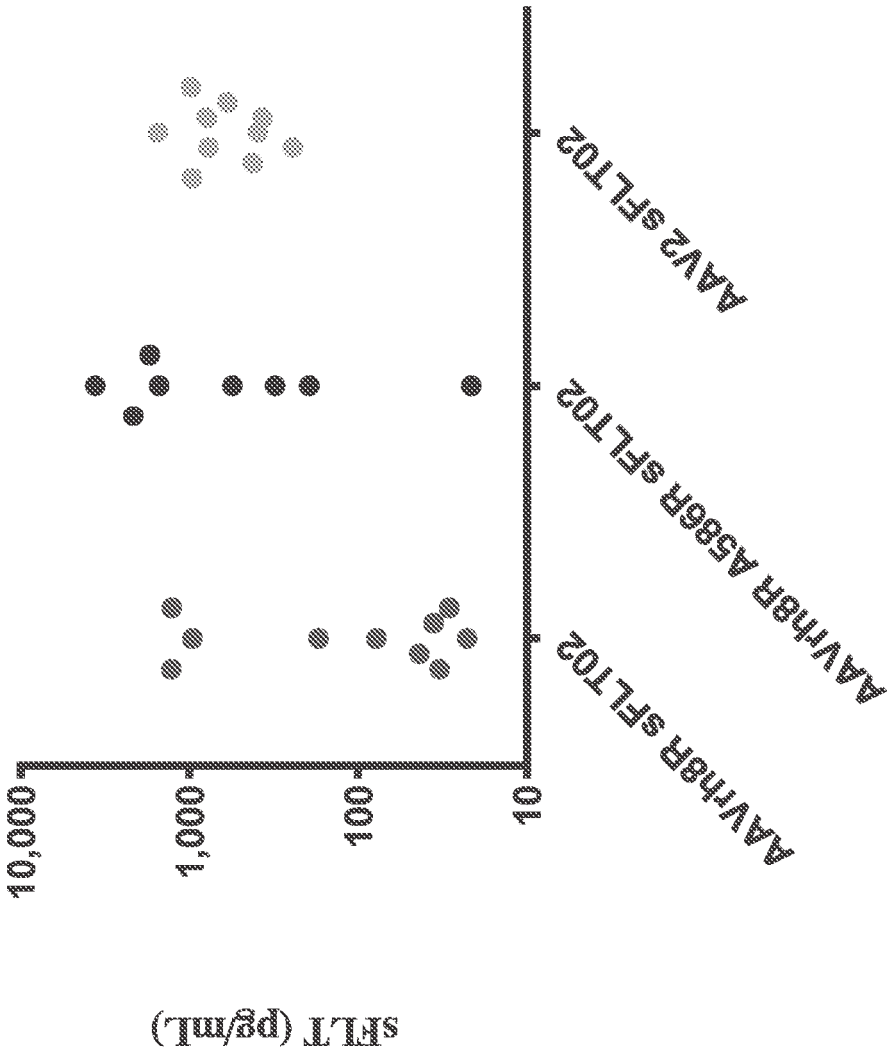

sFLT (pg/mL)
10,000
1,000
100
10
AAVrh8R sFLT02
AAVrh8R A580R sFLT02
AAV2 sFLT02

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