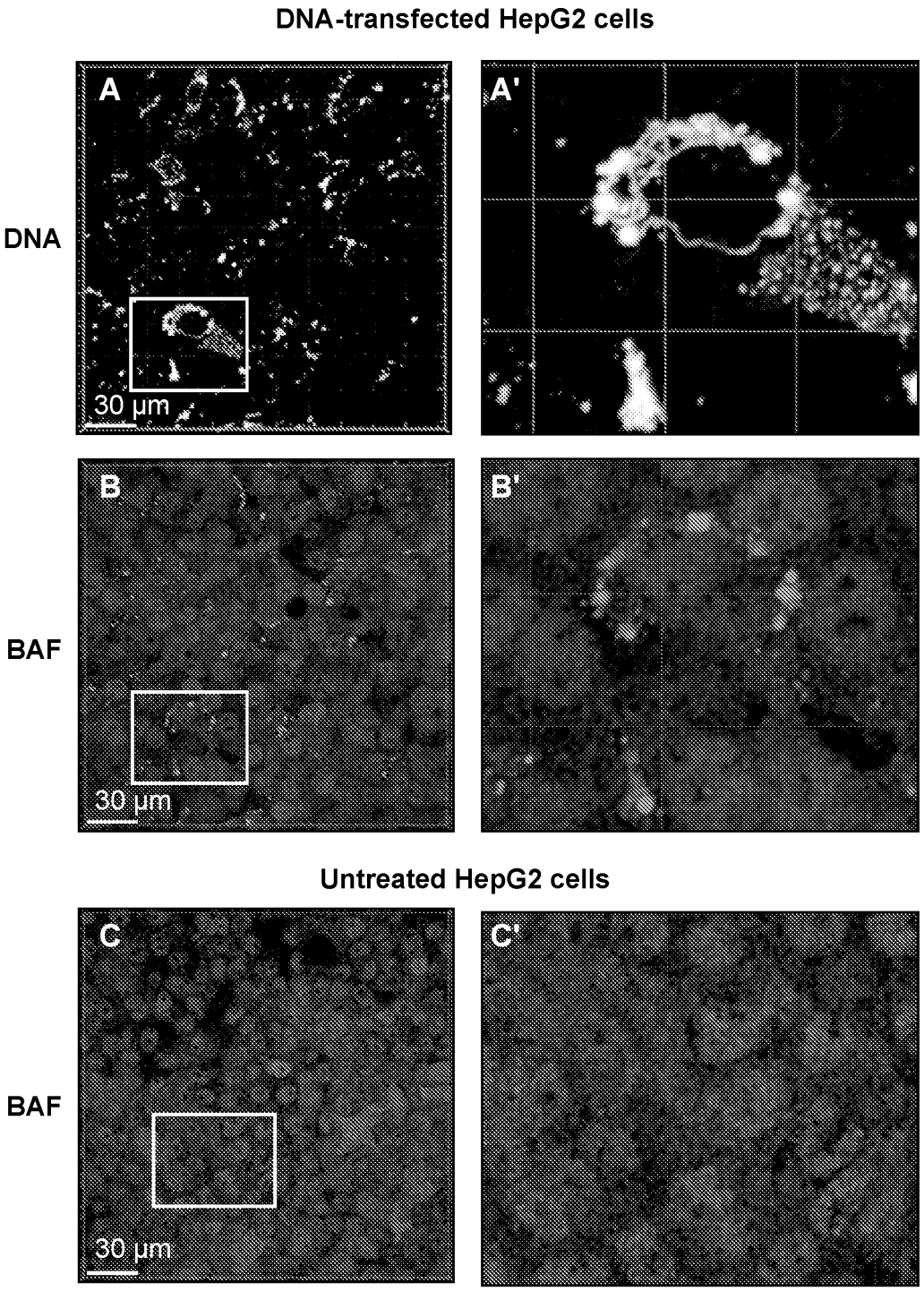
DNA-transfected HepG2 cells
DNA
BAF
Untreated HepG2 cells
BAF
A
A'
B
B'
C
C'
30 µm
30 µm
30 µm

FIG. 1

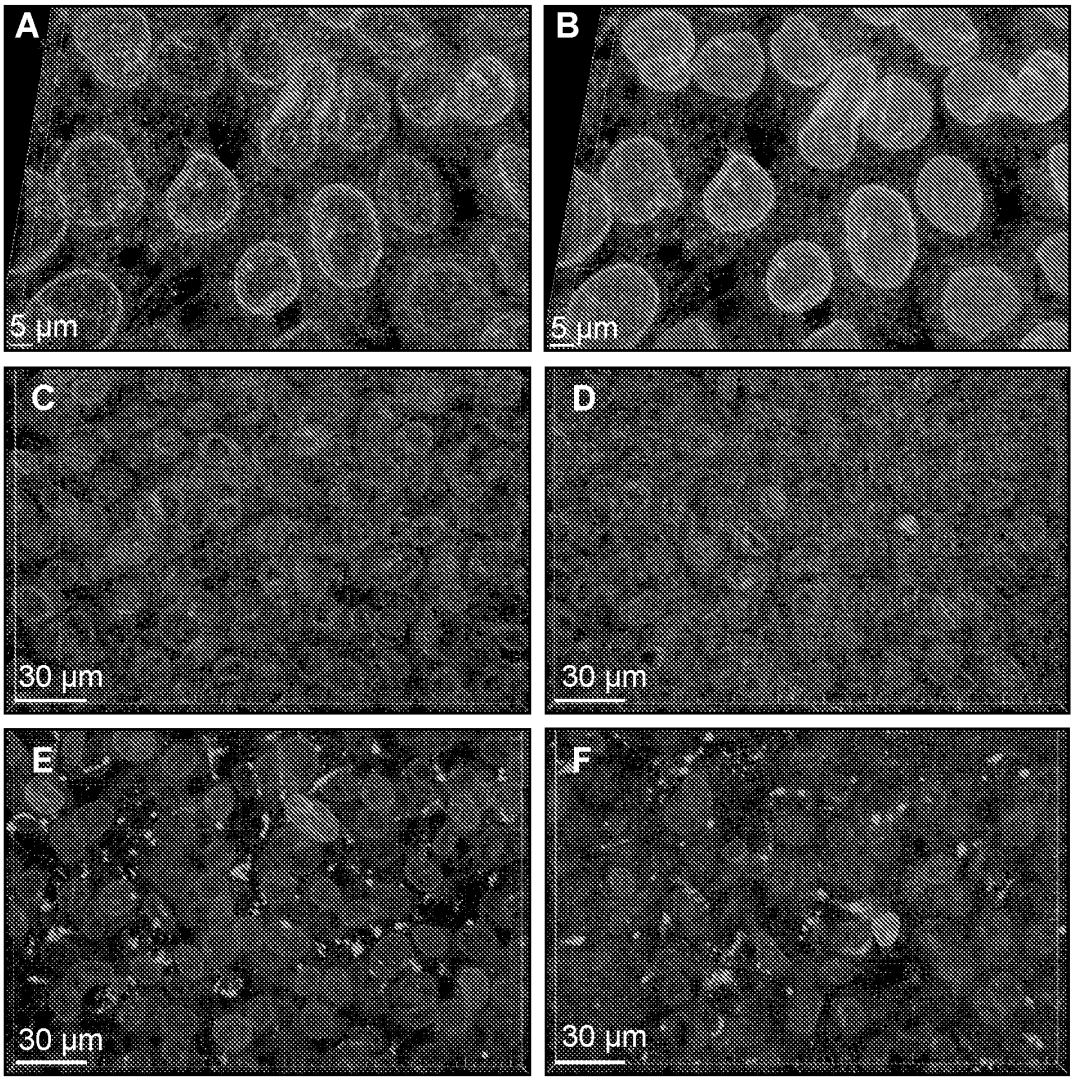

FIG. 2

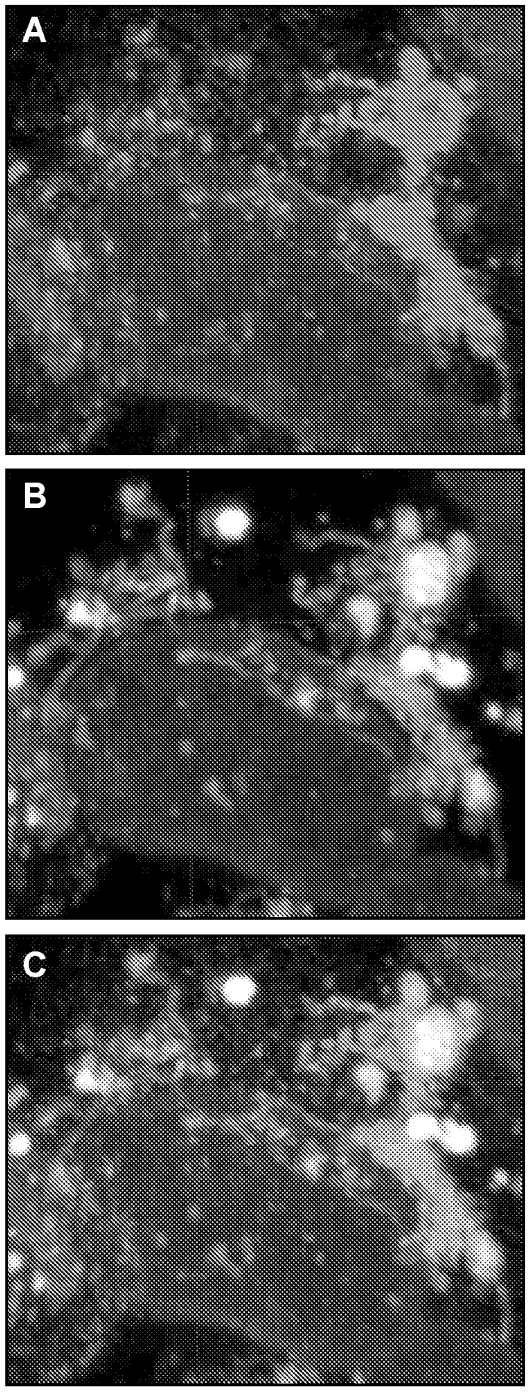

FIG. 3

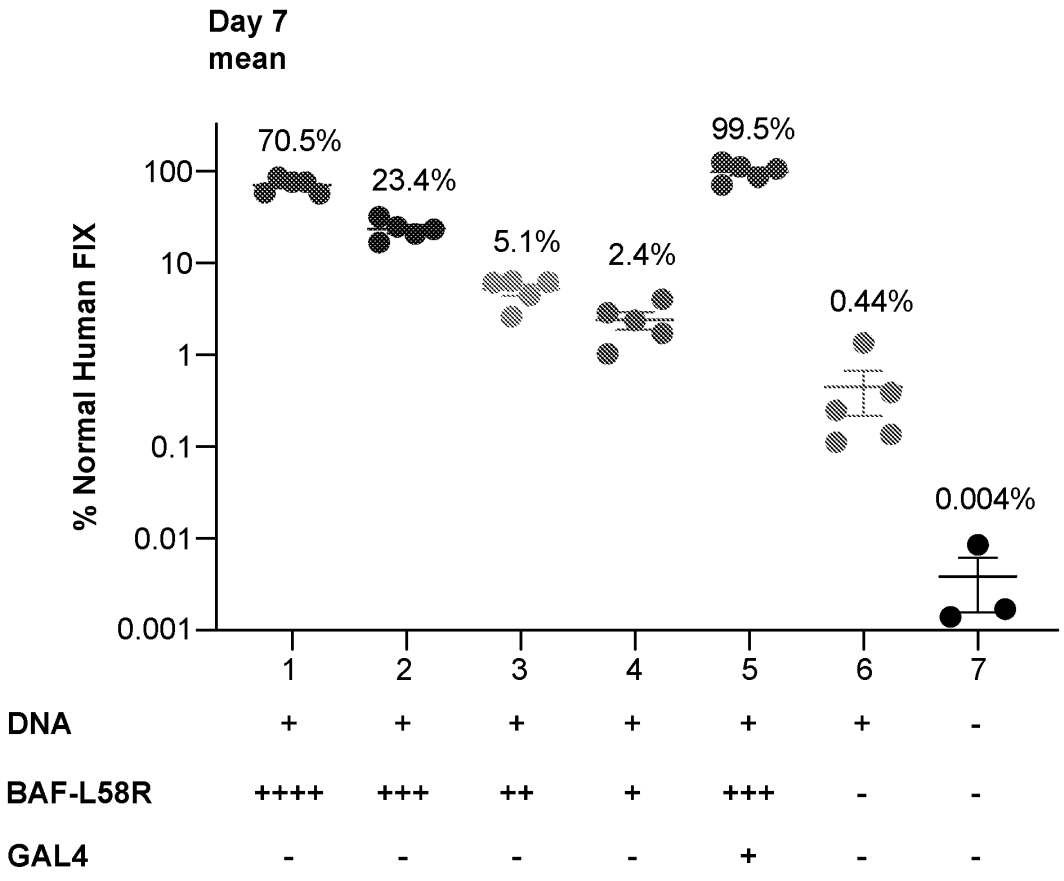
Day 7
mean
% Normal Human FIX
100
10
1
0.1
0.01
0.001
70.5%
23.4%
5.1%
2.4%
99.5%
0.44%
0.004%
1
2
3
4
5
6
7
DNA + + + + + + -
BAF-L58R ++++ +++ ++ + +++ - -
GAL4 - - - - + - -

FIG. 4

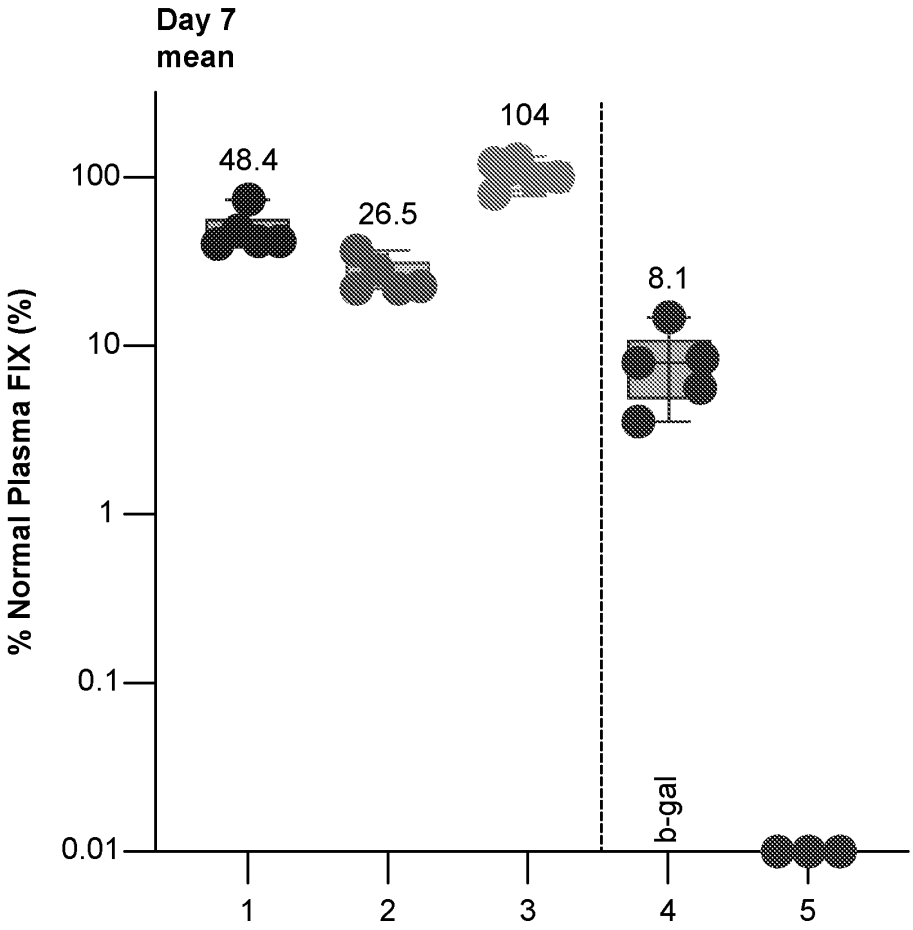
Day 7
mean
% Normal Plasma FIX (%)
100
10
1
0.1
0.01
48.4
26.5
104
8.1
b-gal
1
2
3
4
5

FIG. 5

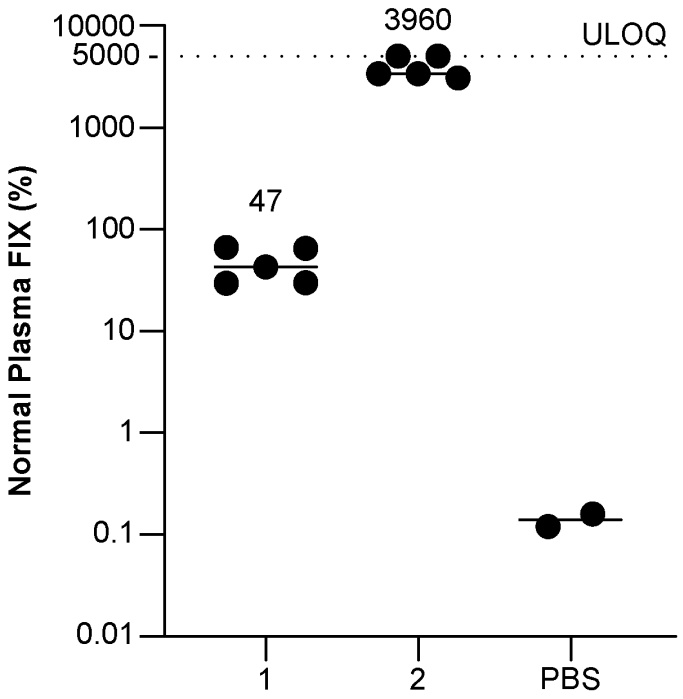
10000
5000
1000
100
10
1
0.1
0.01
Normal Plasma FIX (%)
3960
ULOQ
47
1
2
PBS

FIG. 6

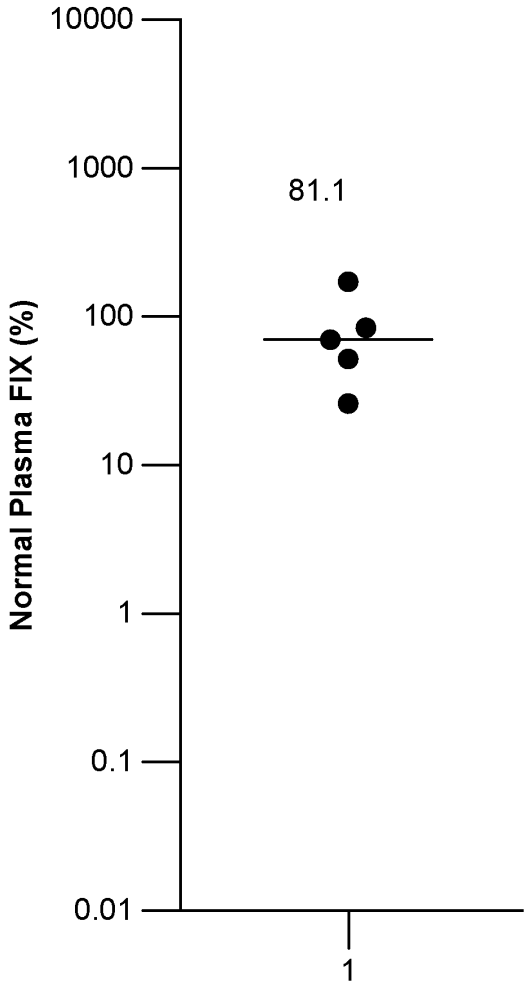

FIG. 7

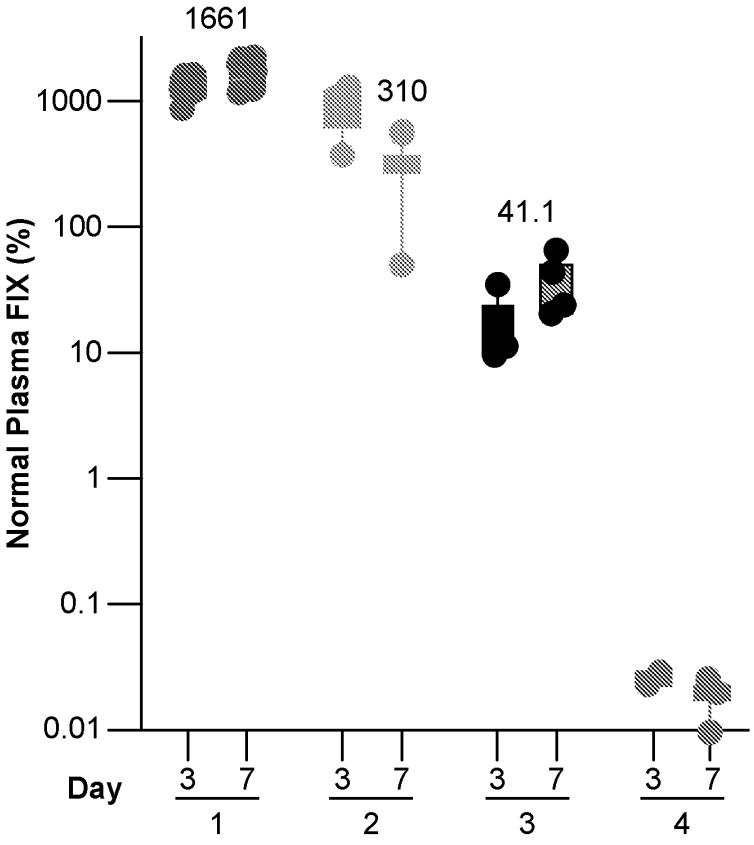
1661
310
41.1
1000
100
10
1
0.1
0.01
Normal Plasma FIX (%)
Day
3 7 3 7 3 7 3 7
1 2 3 4

FIG. 8

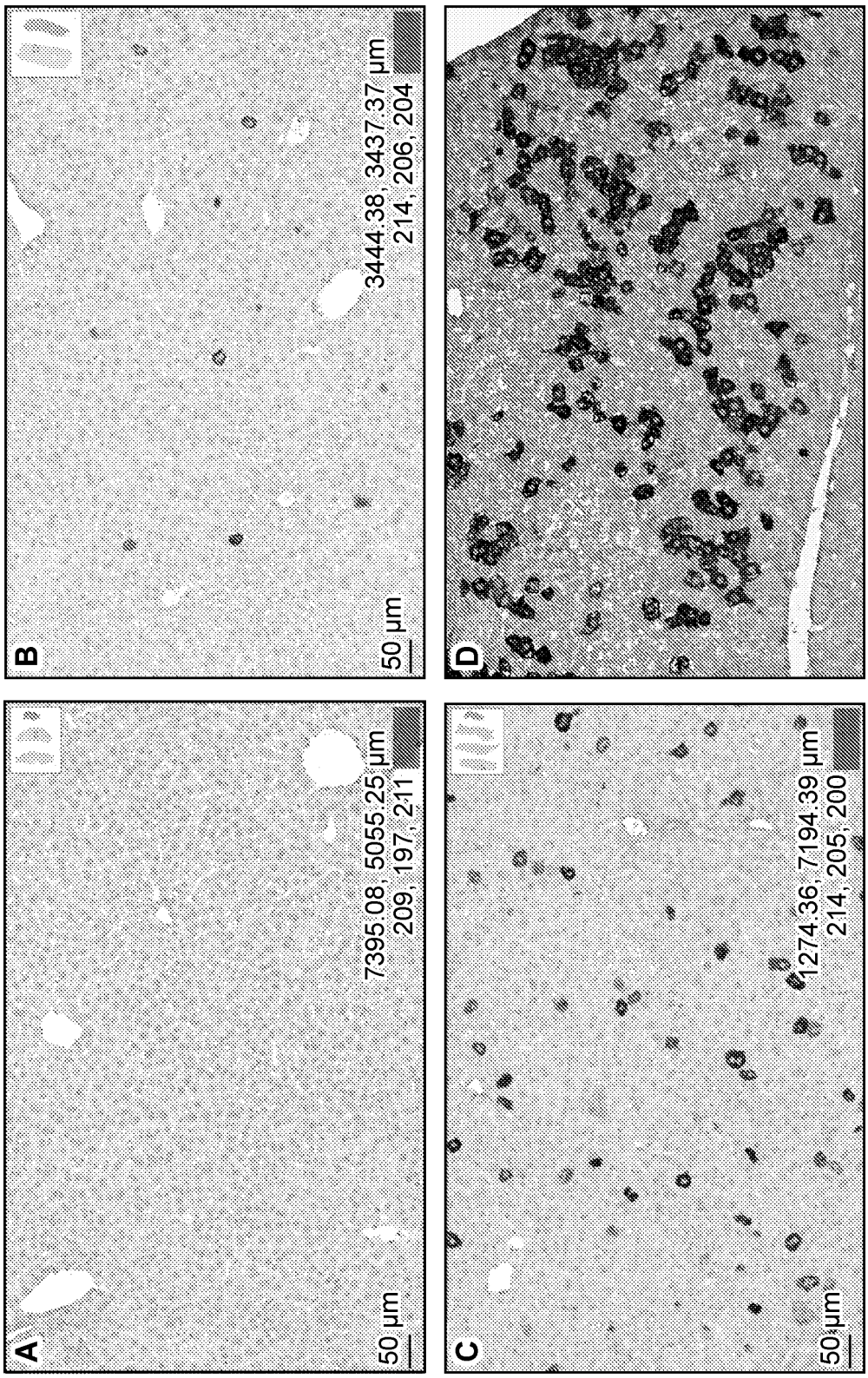
A
50 µm
7395.08, 5055.25 µm
209, 197, 211
B
50 µm
3444.38, 3437.37 µm
214, 206, 204
C
50µm
1274.36, 7194.39 µm
214, 205, 200
D

FIG. 9

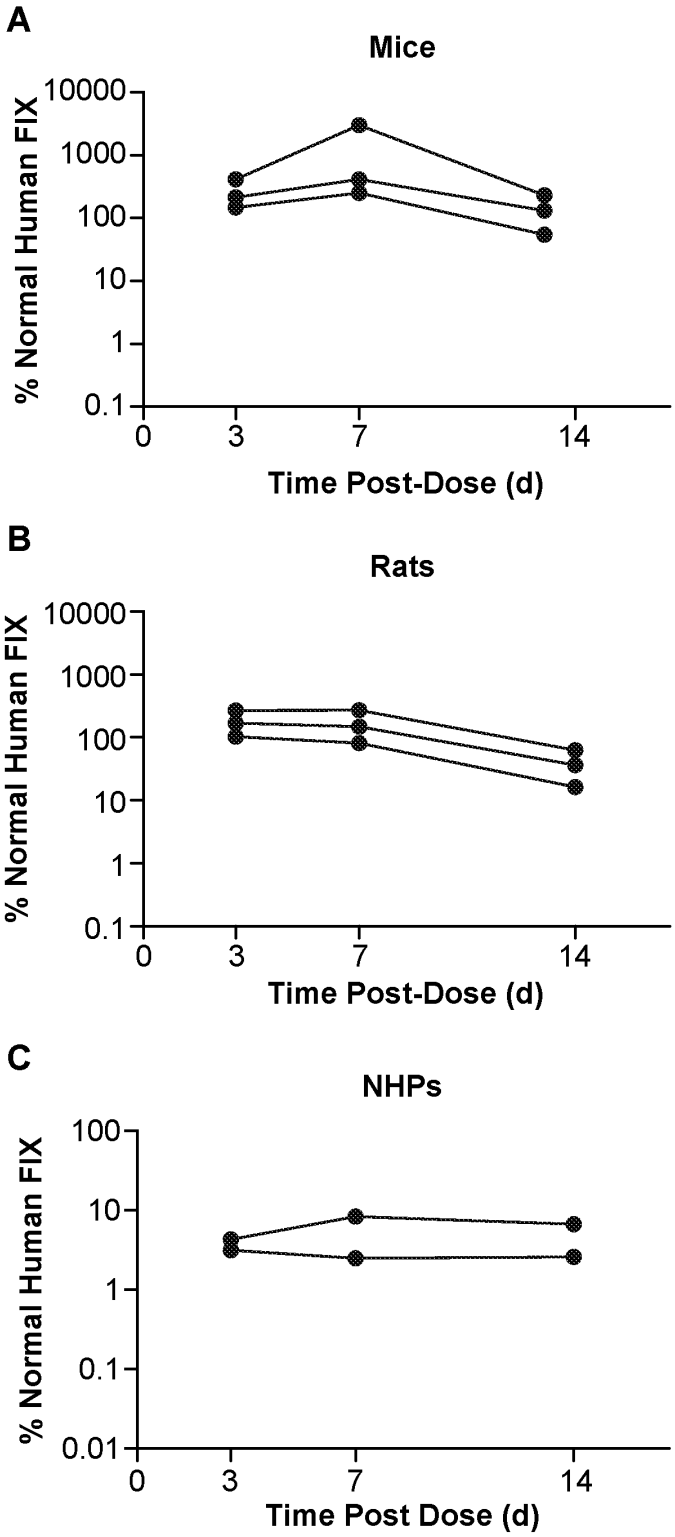
A
Mice
% Normal Human FIX
10000
1000
100
10
1
0.1
0
3
7
14
Time Post-Dose (d)
B
Rats
% Normal Human FIX
10000
1000
100
10
1
0.1
0
3
7
14
Time Post-Dose (d)
C
NHPs
% Normal Human FIX
100
10
1
0.1
0.01
0
3
7
14
Time Post Dose (d)

FIG. 10

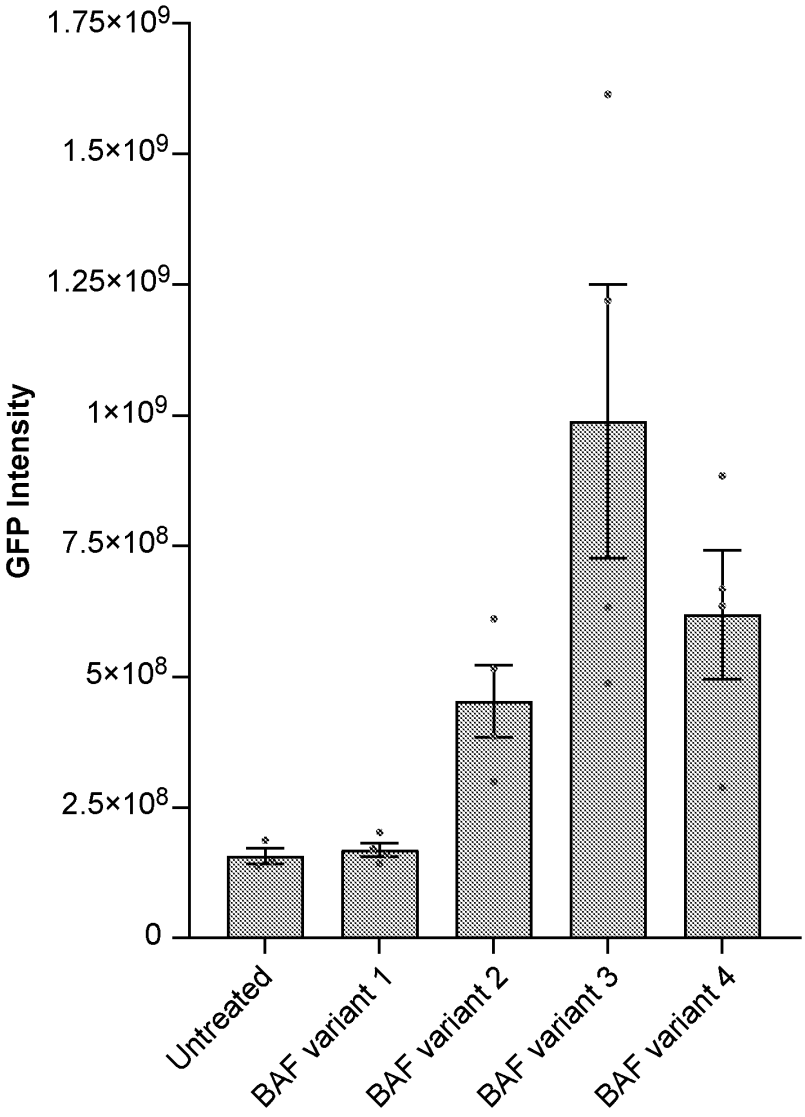
1.75×10^9
1.5×10^9
1.25×10^9
1×10^9
7.5×10^8
5×10^8
2.5×10^8
0
GFP Intensity
Untreated
BAF variant 1
BAF variant 2
BAF variant 3
BAF variant 4

FIG. 11