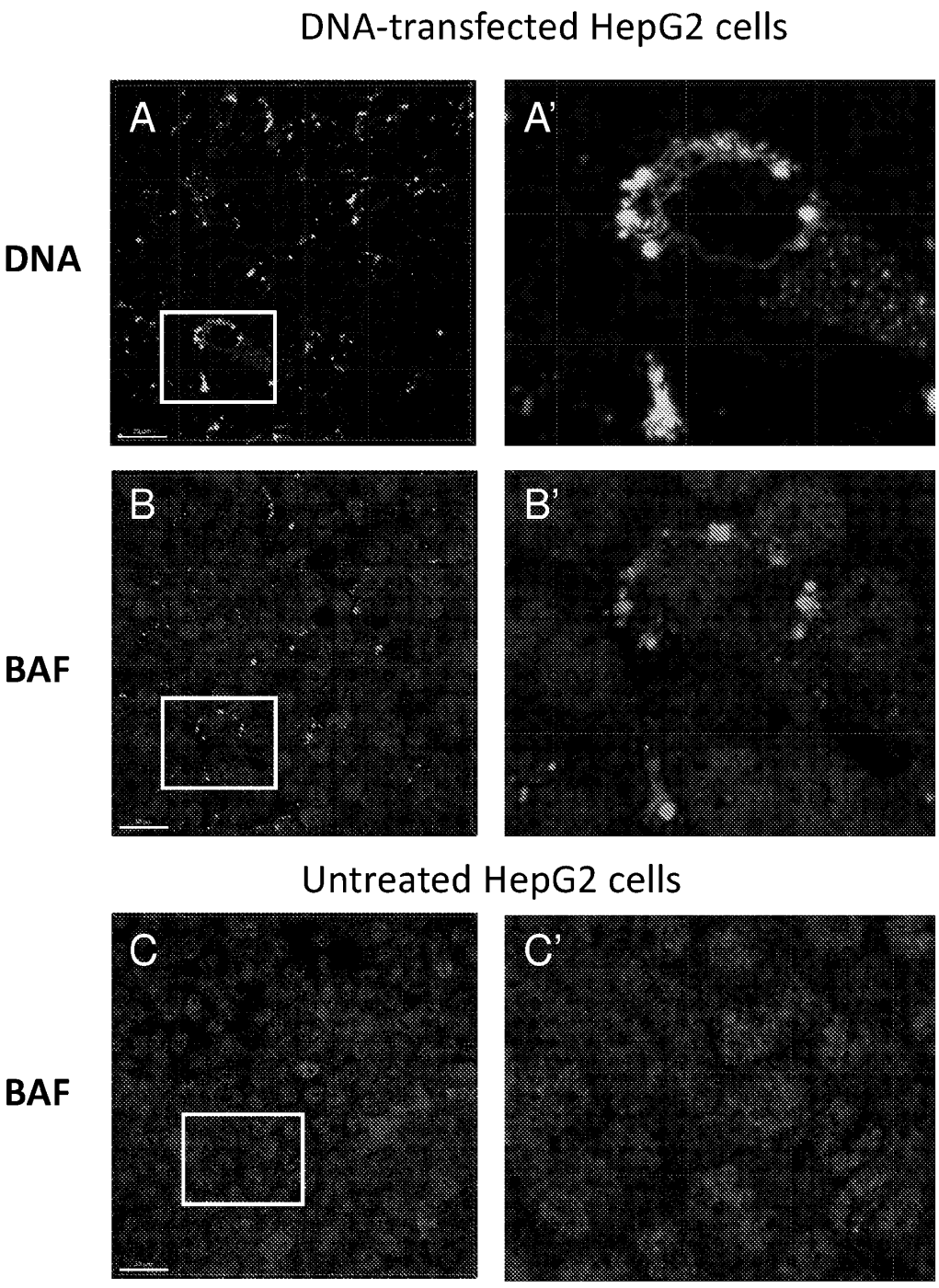

DNA-transfected HepG2 cells
DNA
BAF
A
A'
B
B'
Untreated HepG2 cells
BAF
C
C'

Figure 1

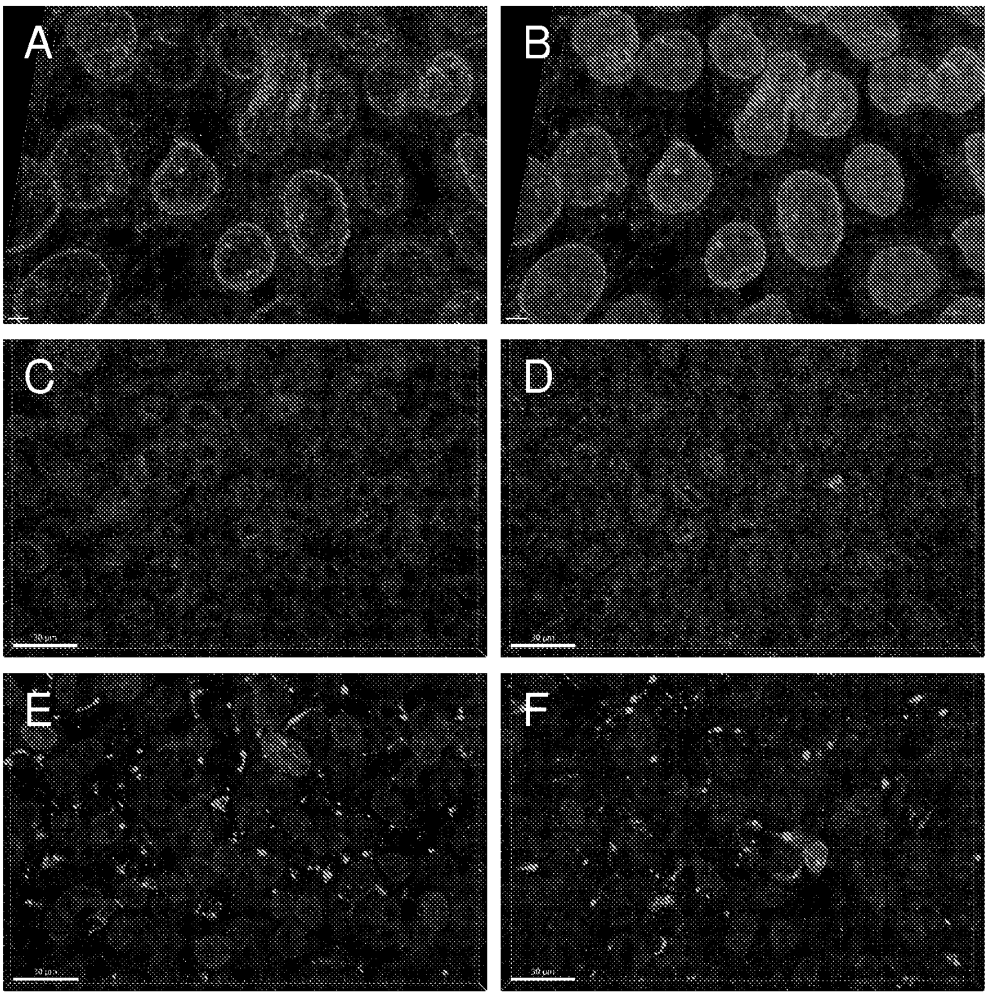


Figure 2

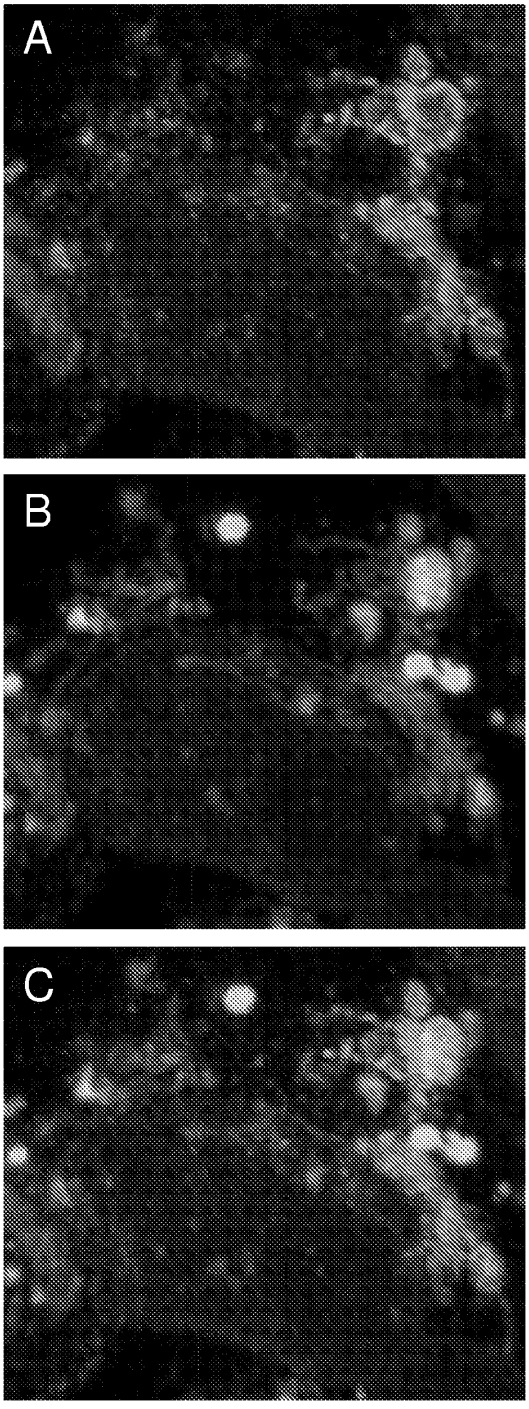


Figure 3

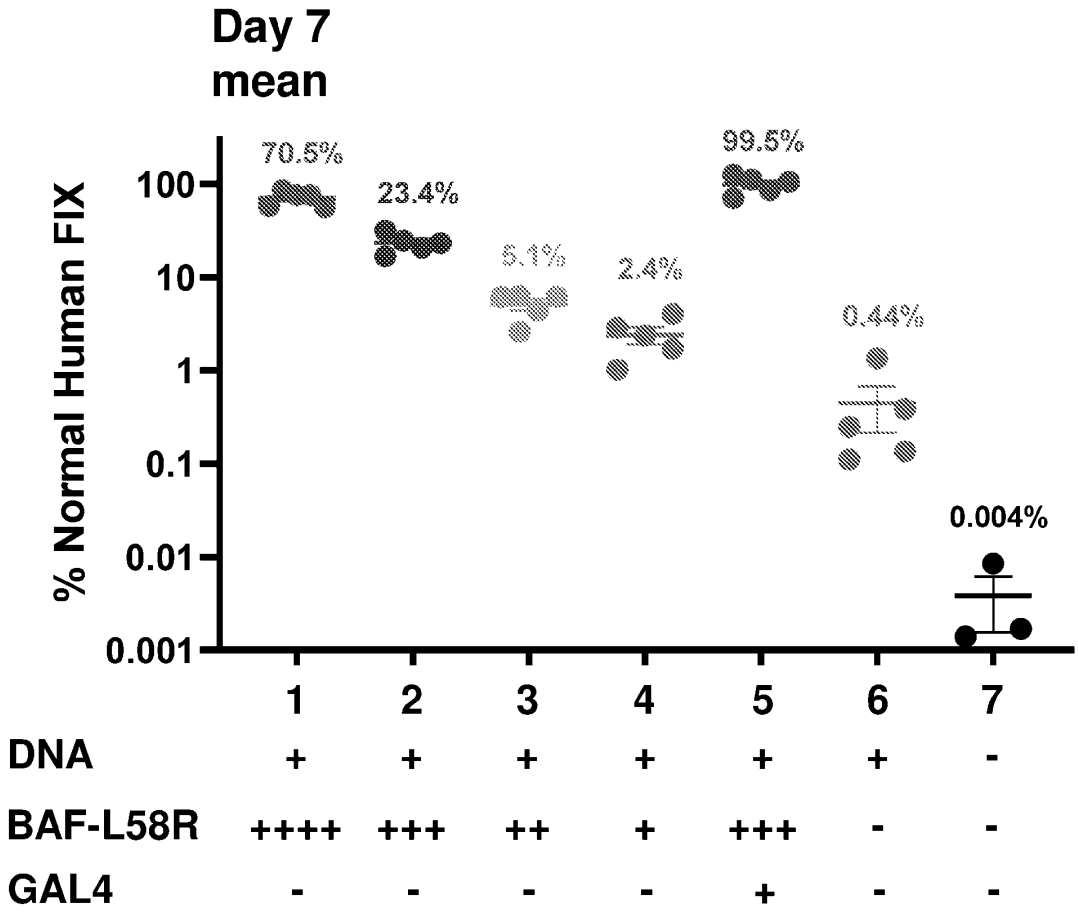

Day 7
mean
% Normal Human FIX
100
10
1
0.1
0.01
0.001
70.5%
23.4%
5.1%
2.4%
99.5%
0.44%
0.004%
1
2
3
4
5
6
7
DNA + + + + + + -
BAF-L58R ++++ +++ ++ + +++ - -
GAL4 - - - - + - -

Figure 4

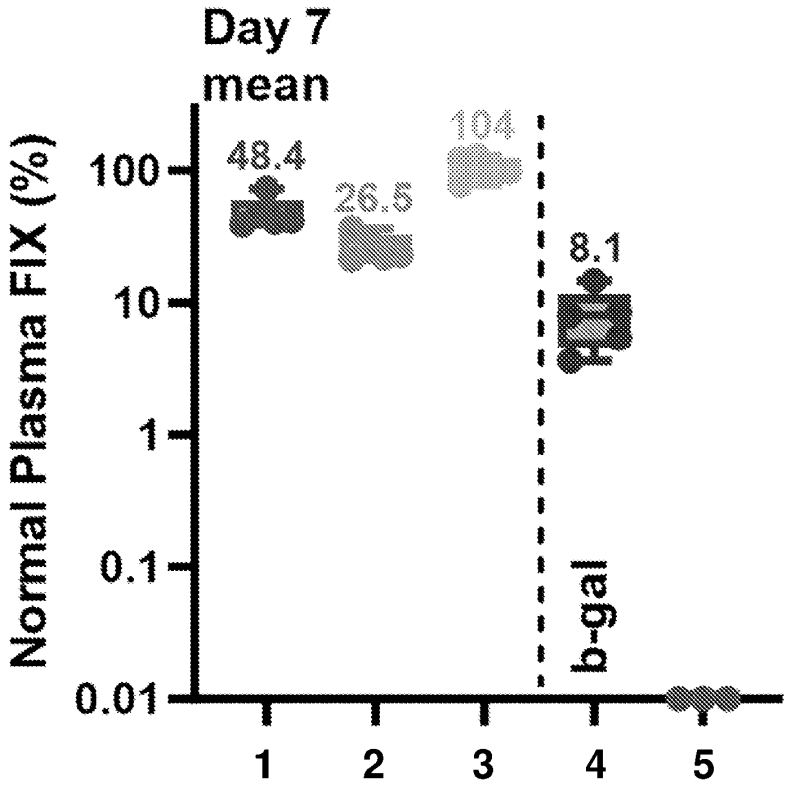

Day 7
mean
Normal Plasma FIX (%)
100
10
1
0.1
0.01
48.4
26.5
104
8.1
b-gal
1
2
3
4
5

Figure 5

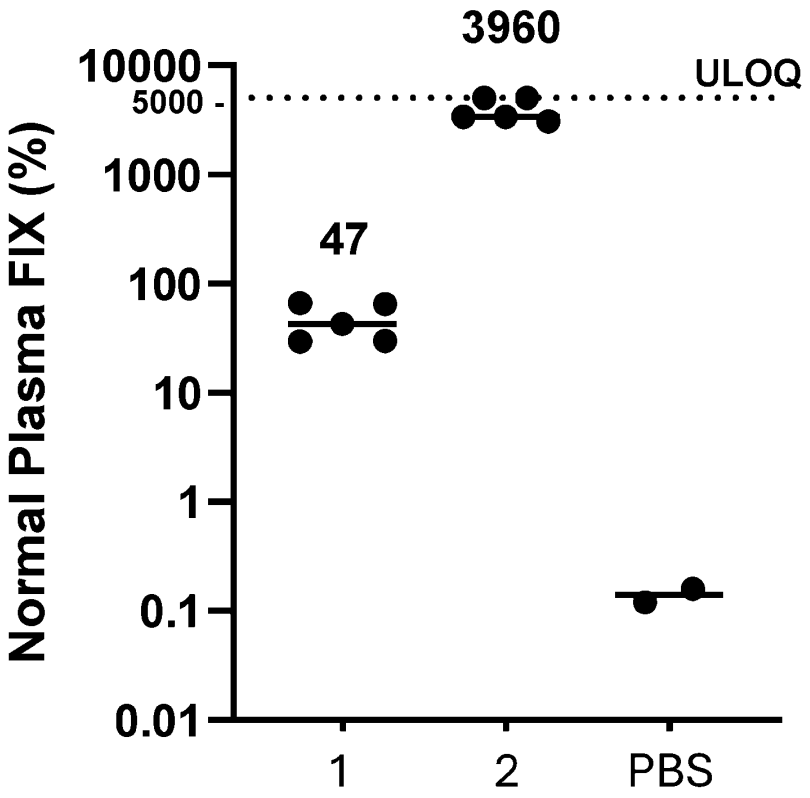

3960
ULOQ
10000
5000
1000
47
100
10
1
0.1
0.01
Normal Plasma FIX (%)
1
2
PBS

Figure 6

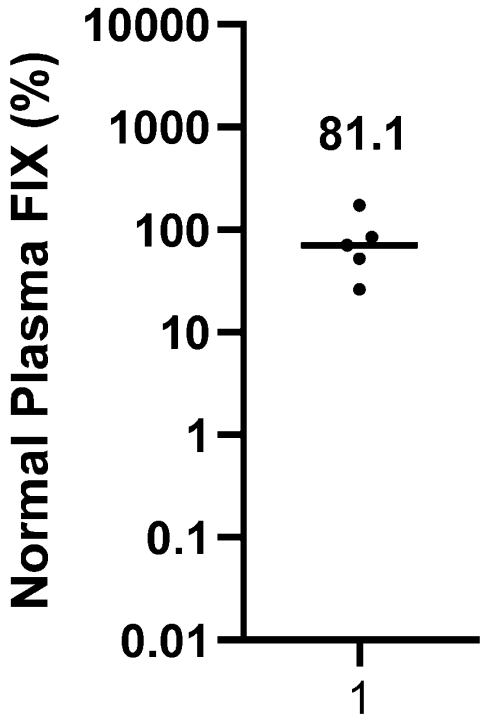


Figure 7

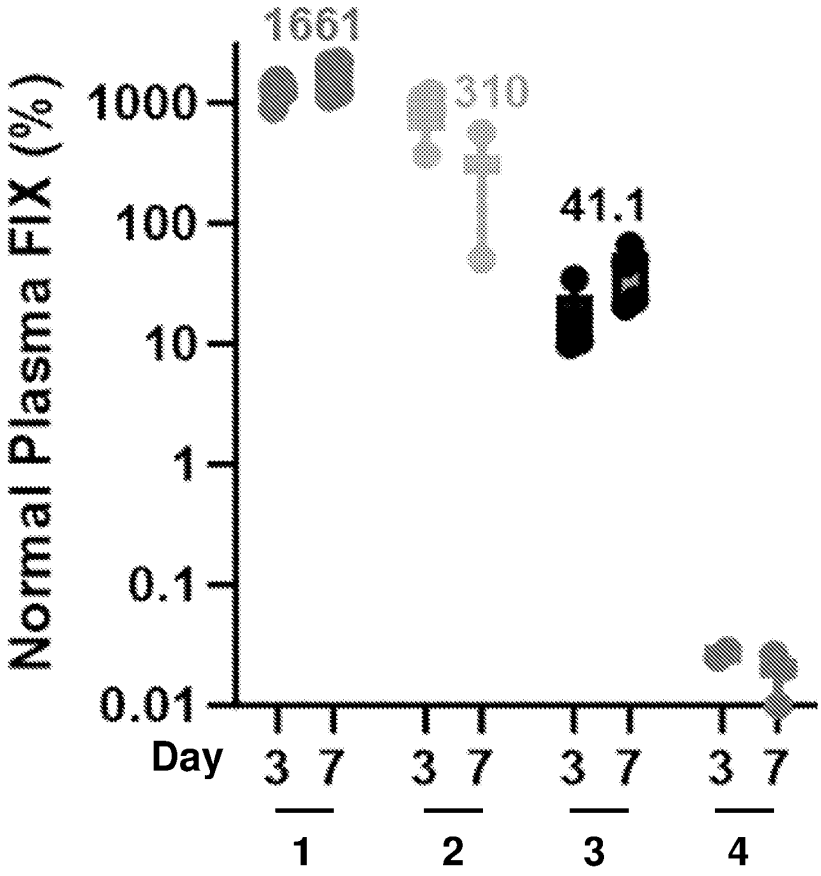

1661
310
41.1
Normal Plasma FIX (%)
1000
100
10
1
0.1
0.01
Day
3 7 3 7 3 7 3 7
1 2 3 4

Figure 8

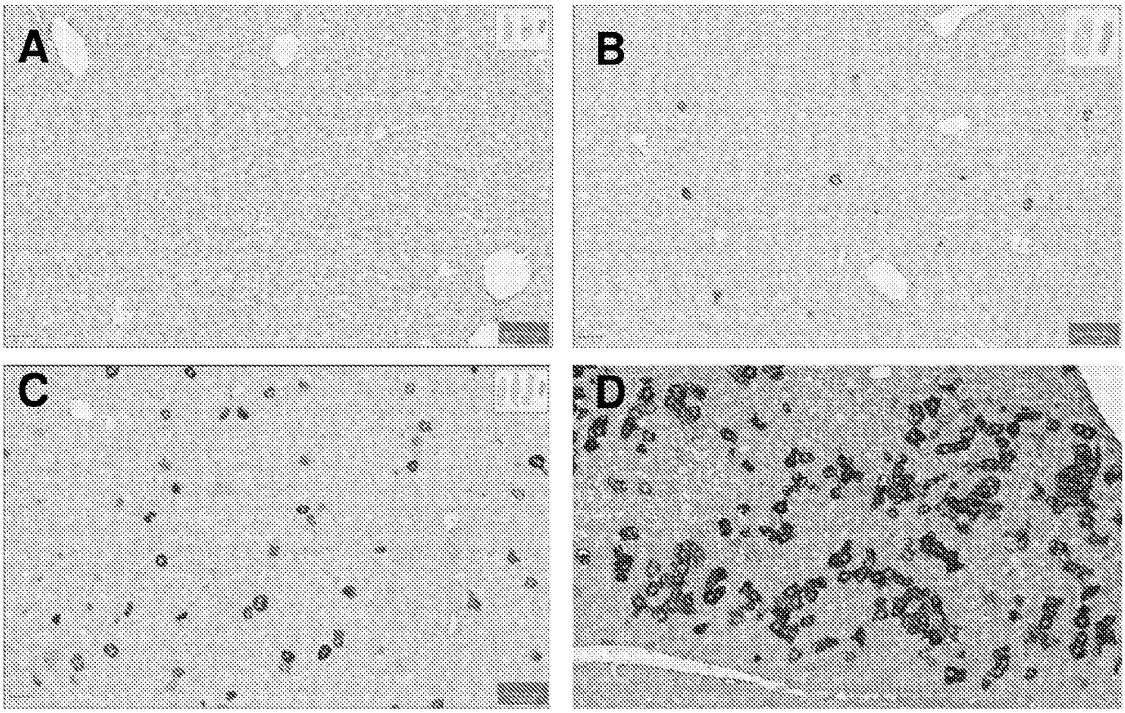


Figure 9

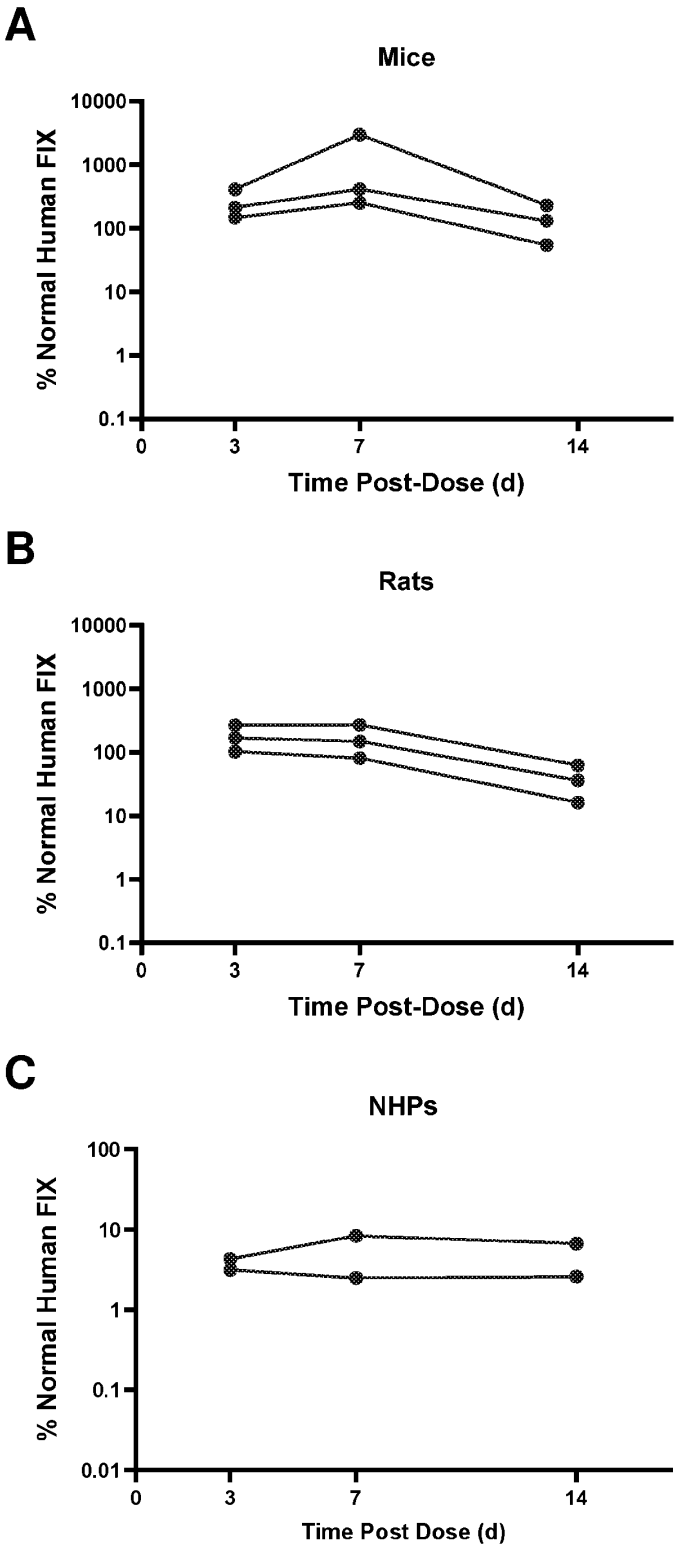

A
Mice
% Normal Human FIX
10000
1000
100
10
1
0.1
0
3
7
14
Time Post-Dose (d)
B
Rats
% Normal Human FIX
10000
1000
100
10
1
0.1
0
3
7
14
Time Post-Dose (d)
C
NHPs
% Normal Human FIX
100
10
1
0.1
0.01
0
3
7
14
Time Post Dose (d)

Figure 10

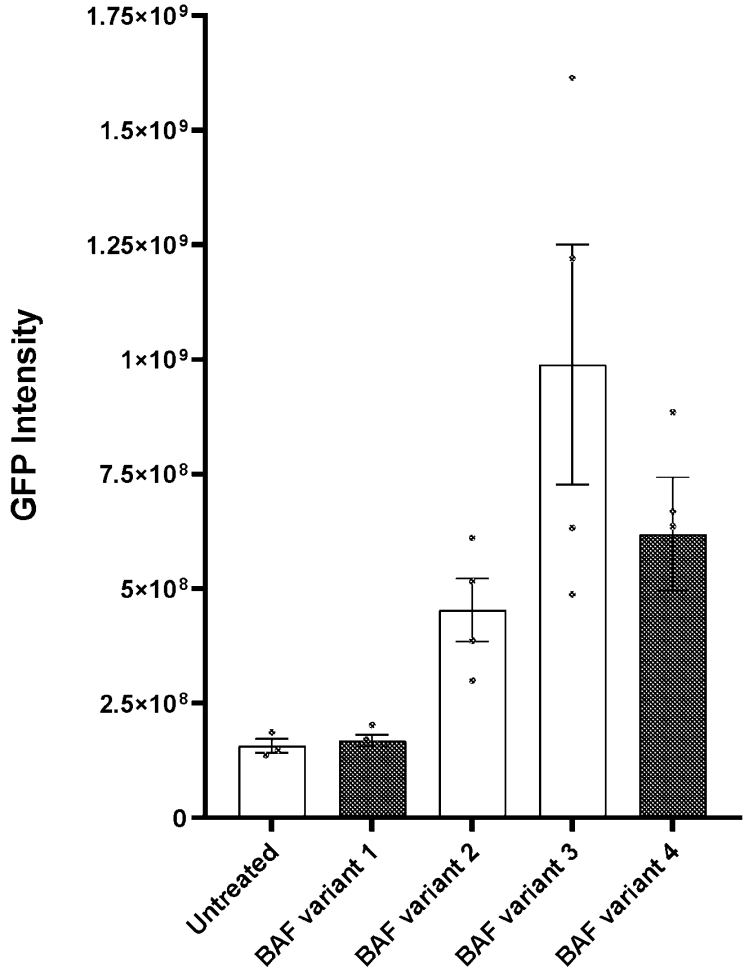

GFP Intensity
1.75×10⁹
1.5×10⁹
1.25×10⁹
1×10⁹
7.5×10⁸
5×10⁸
2.5×10⁸
0
Untreated
BAF variant 1
BAF variant 2
BAF variant 3
BAF variant 4

Figure 11