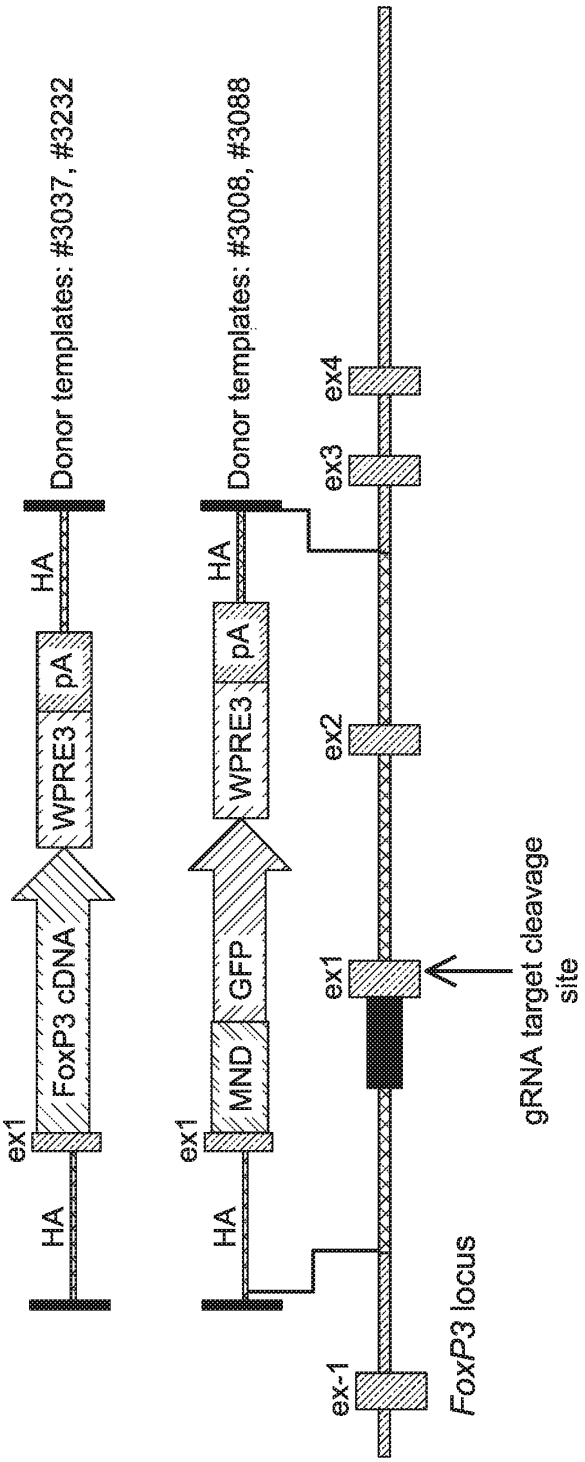

Donor templates: #3037, #3232
HA
pA
WPRE3
FoxP3 cDNA
ex1
HA
Donor templates: #3008, #3088
HA
pA
WPRE3
GFP
MND
ex1
HA
ex4
ex3
ex2
ex1
gRNA target cleavage site
ex-1
FoxP3 locus

FIG. 1

FIG. 2

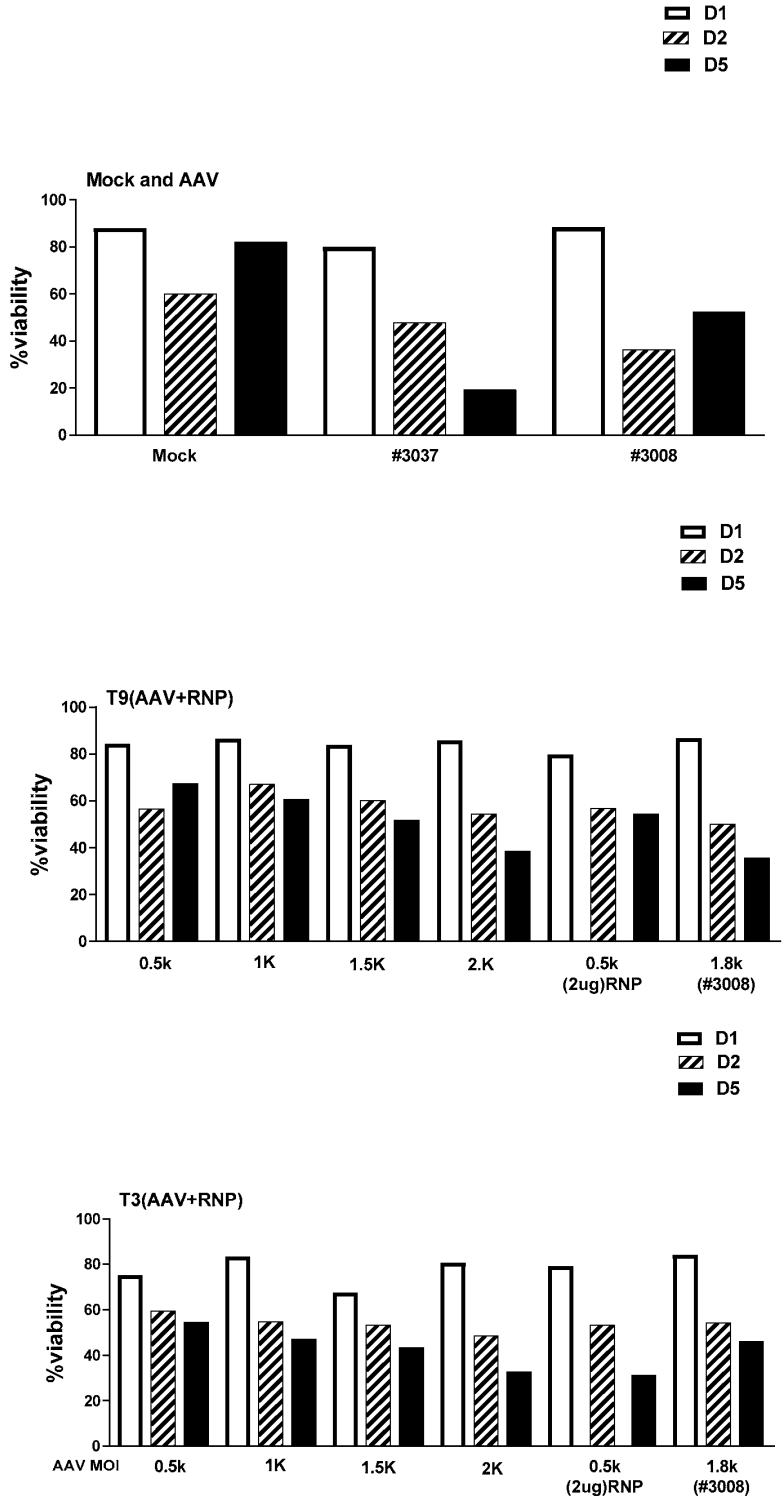

D1
D2
D5
Mock and AAV
%viability
100
80
60
40
20
0
Mock
#3037
#3008
D1
D2
D5
T9(AAV+RNP)
%viability
100
80
60
40
20
0
0.5k
1K
1.5K
2.K
0.5k
(2ug)RNP
1.8k
(#3008)
D1
D2
D5
T3(AAV+RNP)
%viability
100
80
60
40
20
0
AAV MOI
0.5k
1K
1.5K
2K
0.5k
(2ug)RNP
1.8k
(#3008)

FIG. 3

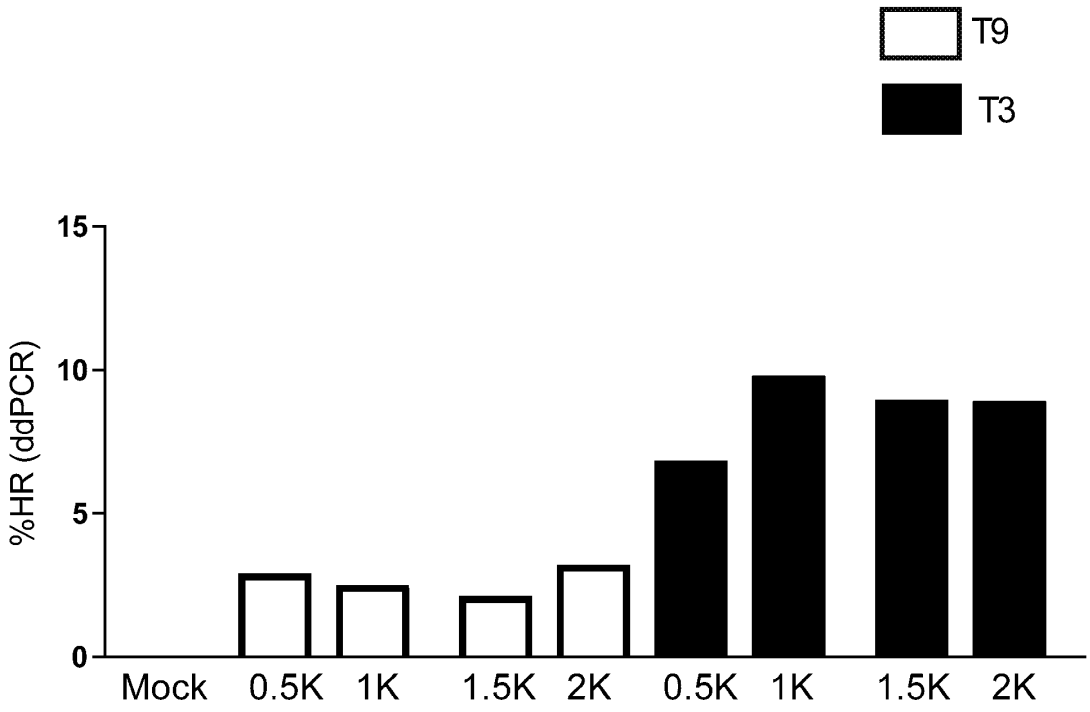


FIG. 4

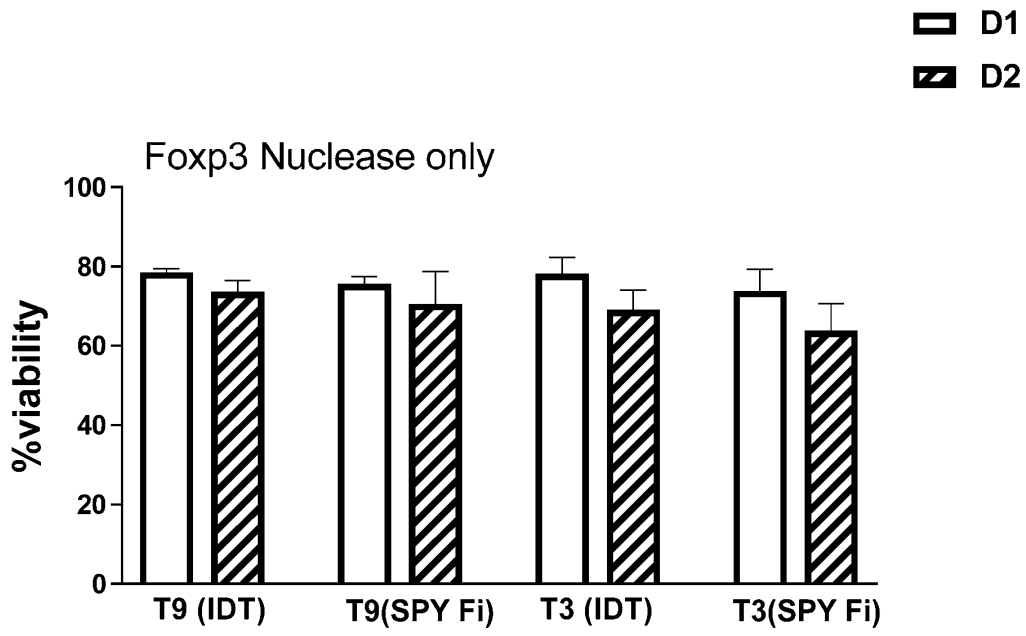

D1
D2
Foxp3 Nuclease only
%viability
100
80
60
40
20
0
T9 (IDT)
T9(SPY Fi)
T3 (IDT)
T3(SPY Fi)

FIG. 5

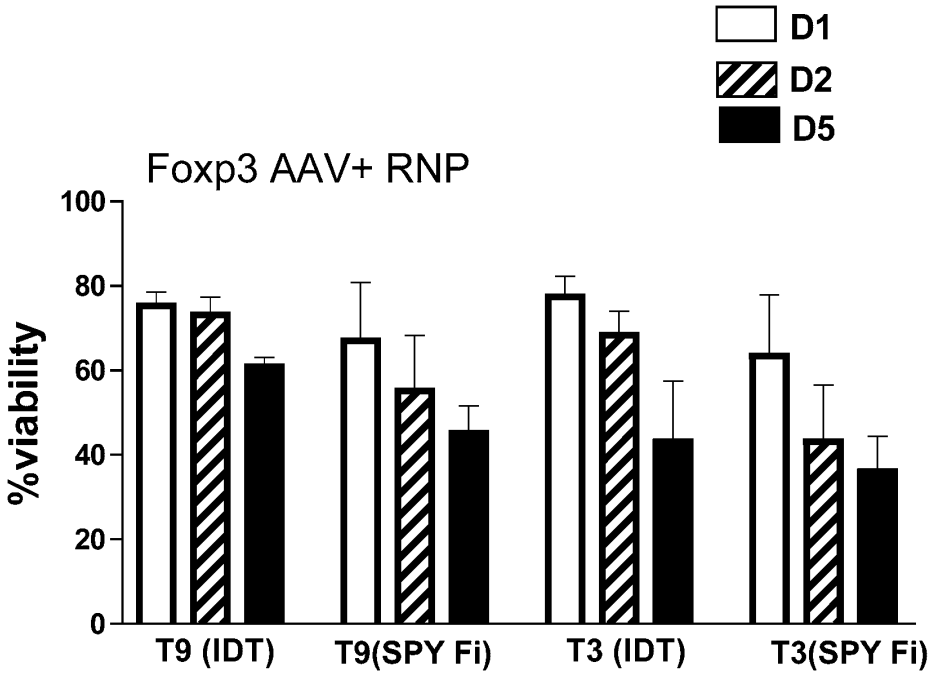

D1
D2
D5
Foxp3 AAV+ RNP
%viability
100
80
60
40
20
0
T9 (IDT)
T9(SPY Fi)
T3 (IDT)
T3(SPY Fi)

FIG. 6

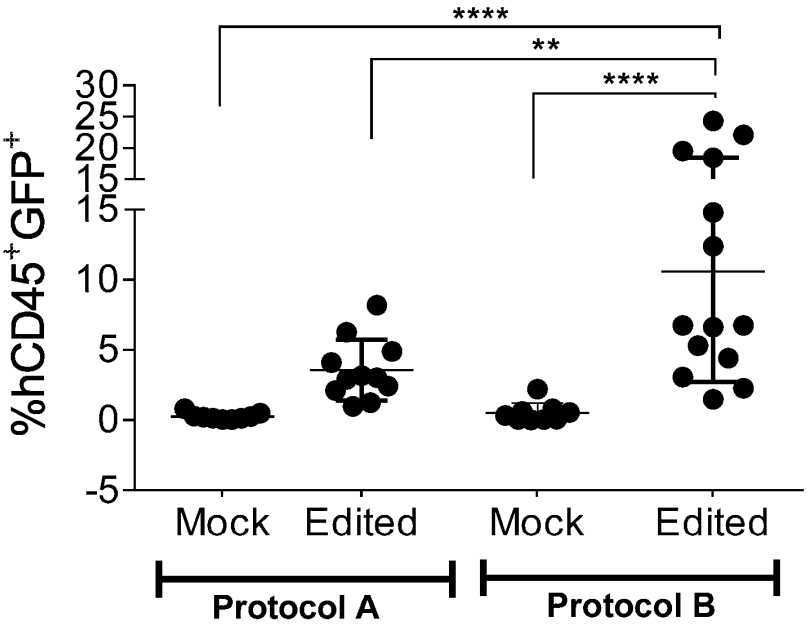

****
**
****
30
25
20
15
15
10
5
0
-5
%hCD45+GFP+
Mock
Edited
Mock
Edited
Protocol A
Protocol B

FIG. 7

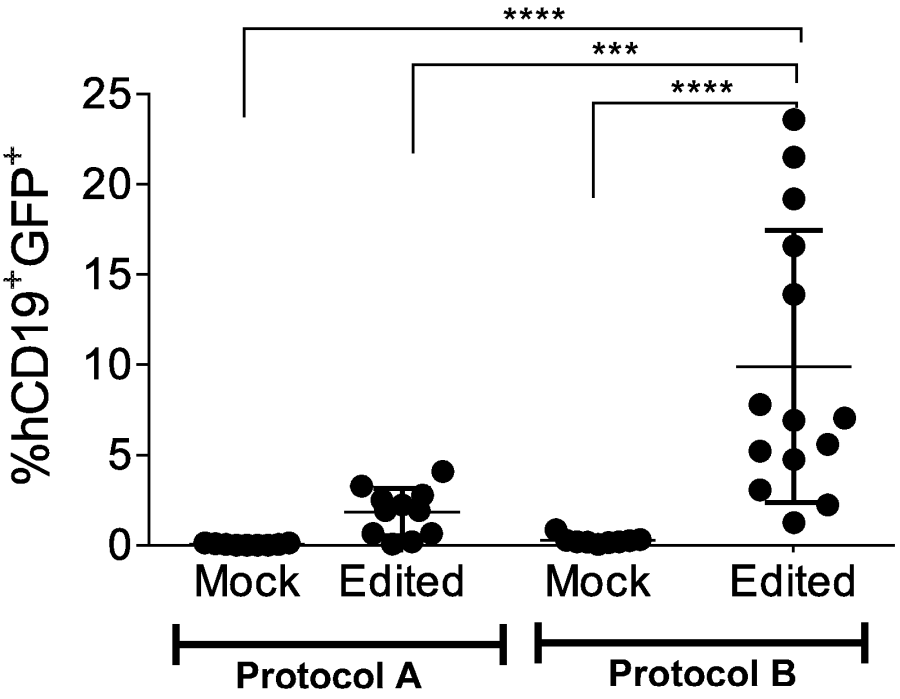
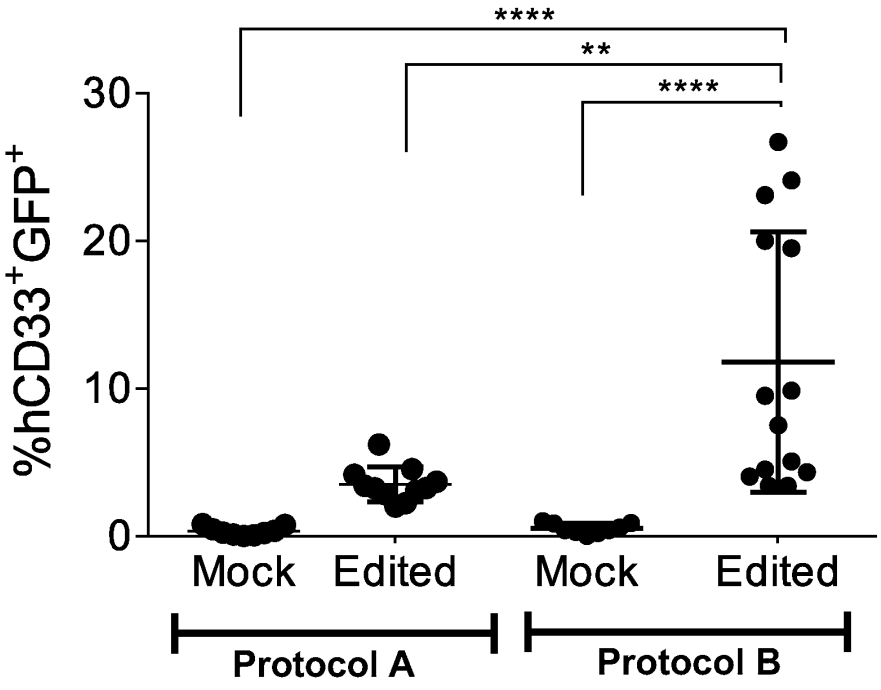

****
***
****
25
20
15
10
5
0
%hCD19+GFP+
Mock
Edited
Mock
Edited
Protocol A
Protocol B

FIG. 8


****
**
****
30
20
10
0
%hCD33+GFP+
Mock
Edited
Mock
Edited
Protocol A
Protocol B