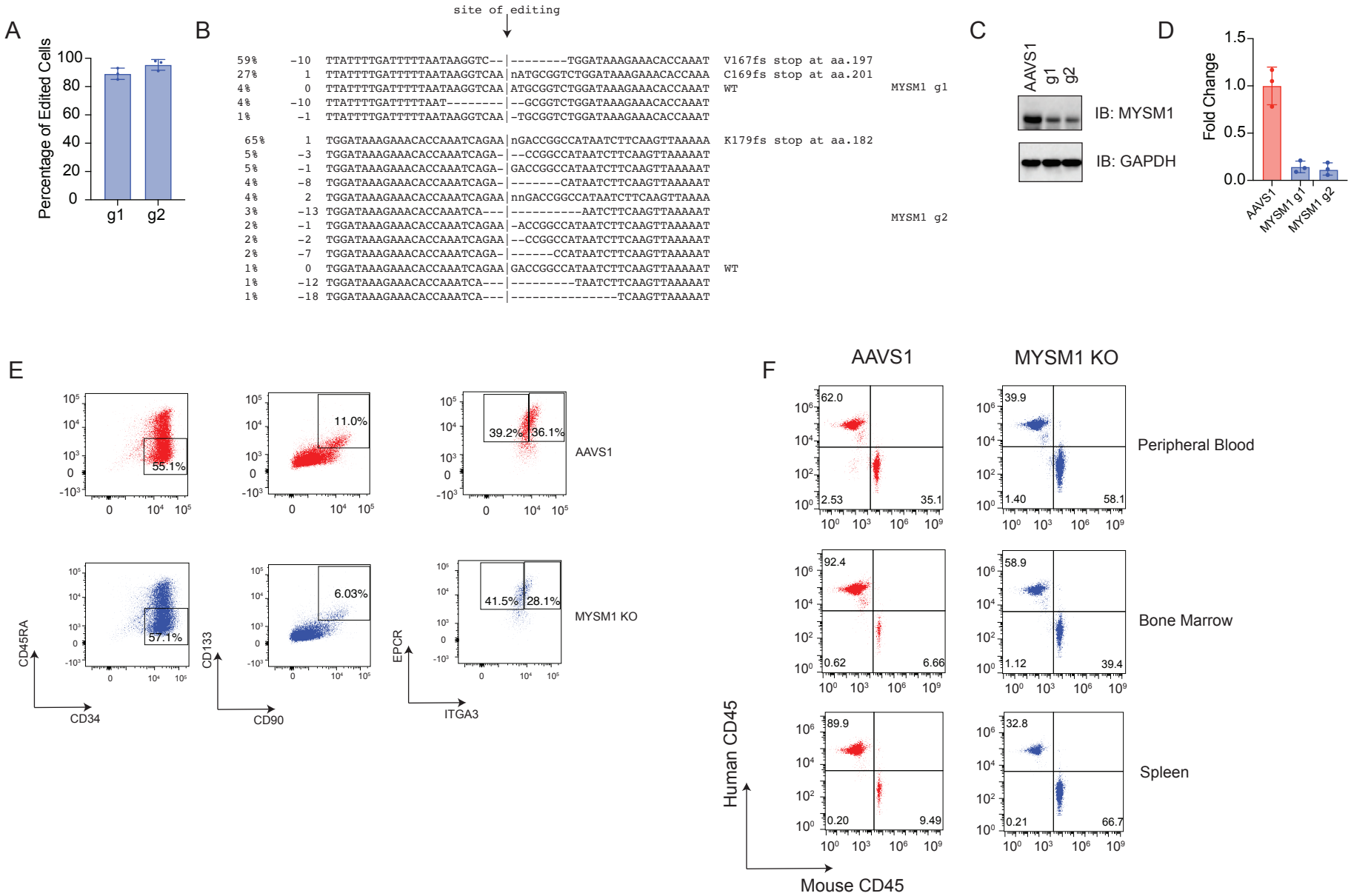
Figure S1


A
Percentage of Edited Cells
100
80
60
40
20
0
g1
g2
B
site of editing
59% -10 TTATTTTGATTTTTAATAAGGTC--|--------TGGATAAAGAAACACCAAAT V167fs stop at aa.197
27% 1 TTATTTTGATTTTTAATAAGGTCAA|nATGCGGTCTGGATAAAGAAACACCAAA C169fs stop at aa.201
4% 0 TTATTTTGATTTTTAATAAGGTCAA|ATGCGGTCTGGATAAAGAAACACCAAAT WT MYSM1 g1
4% -10 TTATTTTGATTTTTAAT--------|--GCGGTCTGGATAAAGAAACACCAAAT
1% -1 TTATTTTGATTTTTAATAAGGTCAA|-TGCGGTCTGGATAAAGAAACACCAAAT
65% 1 TGGATAAAGAAACACCAAATCAGAA|nGACCGGCCATAATCTTCAAGTTAAAAA K179fs stop at aa.182
5% -3 TGGATAAAGAAACACCAAATCAGA-|--CCGGCCATAATCTTCAAGTTAAAAAT
5% -1 TGGATAAAGAAACACCAAATCAGA-|GACCGGCCATAATCTTCAAGTTAAAAAT
4% -8 TGGATAAAGAAACACCAAATCAGA-|-------CATAATCTTCAAGTTAAAAAT
4% 2 TGGATAAAGAAACACCAAATCAGAA|nnGACCGGCCATAATCTTCAAGTTAAAA
3% -13 TGGATAAAGAAACACCAAATCA---|----------AATCTTCAAGTTAAAAAT MYSM1 g2
2% -1 TGGATAAAGAAACACCAAATCAGAA|-ACCGGCCATAATCTTCAAGTTAAAAAT
2% -2 TGGATAAAGAAACACCAAATCAGAA|--CCGGCCATAATCTTCAAGTTAAAAAT
2% -7 TGGATAAAGAAACACCAAATCAGA-|------CCATAATCTTCAAGTTAAAAAT
1% 0 TGGATAAAGAAACACCAAATCAGAA|GACCGGCCATAATCTTCAAGTTAAAAAT WT
1% -12 TGGATAAAGAAACACCAAATCA---|---------TAATCTTCAAGTTAAAAAT
1% -18 TGGATAAAGAAACACCAAATCA---|---------------TCAAGTTAAAAAT
C
AAVS1
g1
g2
IB: MYSM1
IB: GAPDH
D
Fold Change
1.5
1.0
0.5
0.0
AAVS1
MYSM1 g1
MYSM1 g2
E
55.1%
11.0%
39.2%
36.1%
AAVS1
57.1%
6.03%
41.5%
28.1%
MYSM1 KO
CD45RA
CD34
CD133
CD90
EPCR
ITGA3
F
AAVS1
MYSM1 KO
62.0
2.53
35.1
39.9
1.40
58.1
Peripheral Blood
92.4
0.62
6.66
58.9
1.12
39.4
Bone Marrow
89.9
0.20
9.49
32.8
0.21
66.7
Spleen
Human CD45
Mouse CD45