Figure 1A

5'-TCAAACAGACACCA**TG**GTGCATCTGACTCCTG**A**GGAGAAGTCTGCCGTTACTGCCCTGTGGGGC

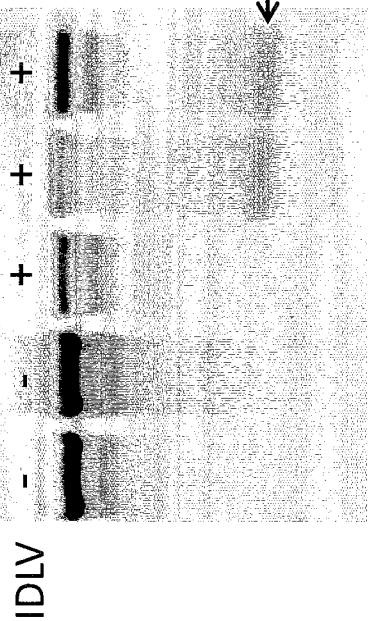


Figure 1D

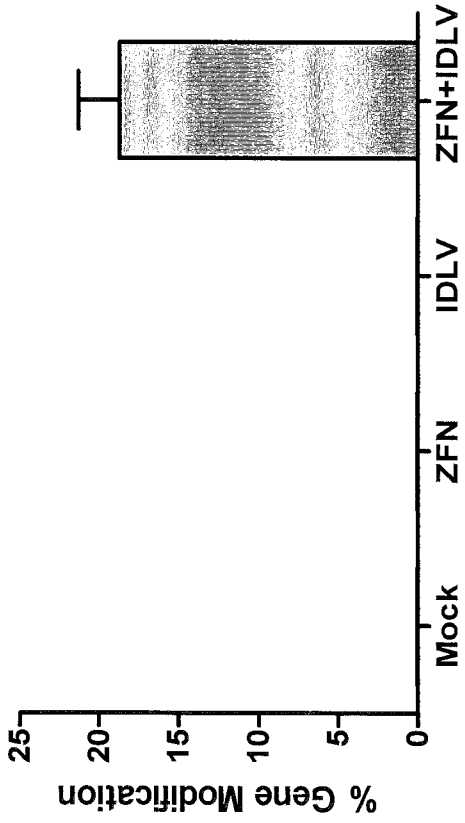


Figure 1E

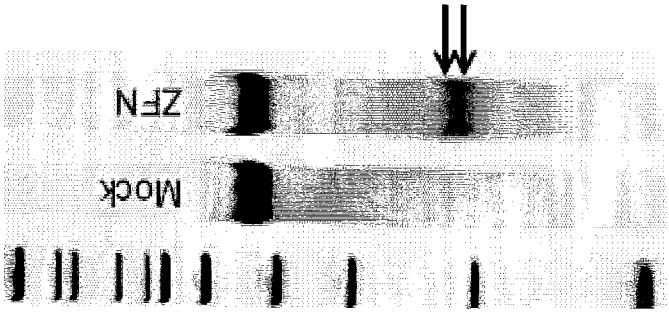


Figure 1B

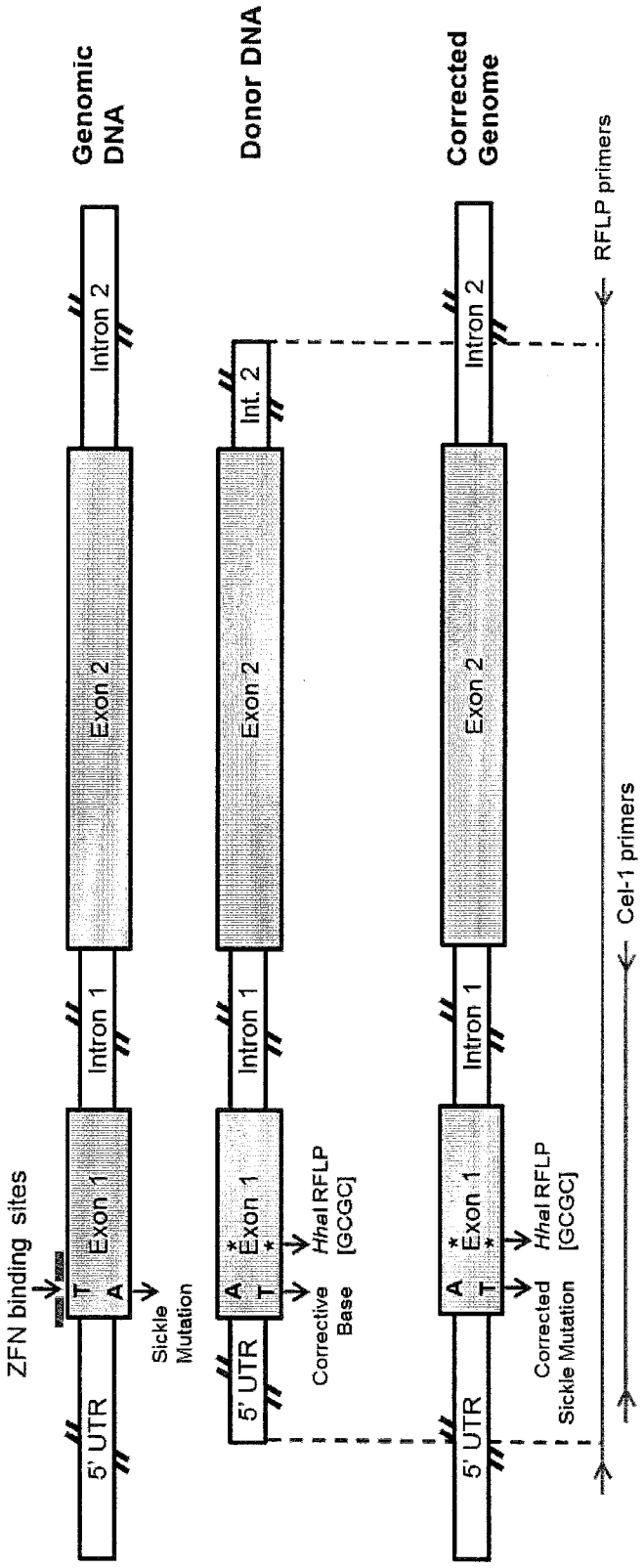

Genomic DNA
Donor DNA
Corrected Genome
ZFN binding sites
5' UTR
Exon 1
Intron 1
Exon 2
Intron 2
Int. 2
T
A
Sickle Mutation
Corrective Base
HhaI RFLP [GCGC]
Corrected Sickle Mutation
RFLP primers
Cel-1 primers

Figure 1C

Figure 2A

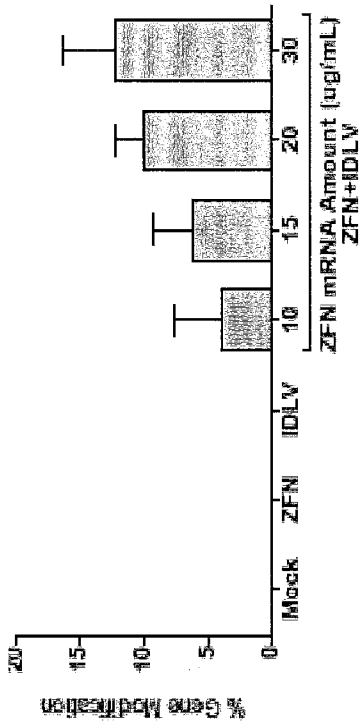


Figure 2B

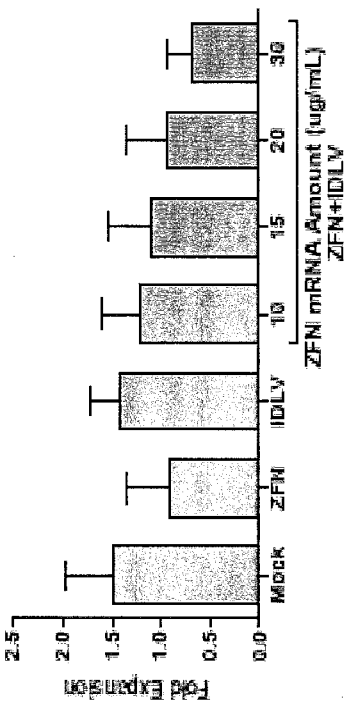
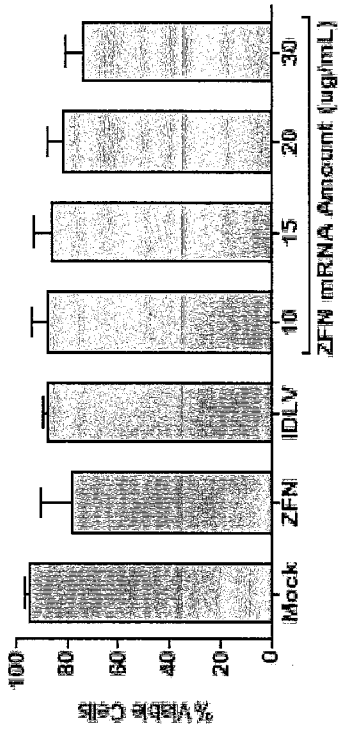


Figure 2C



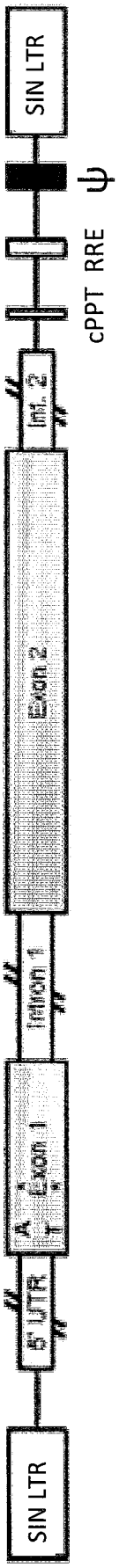


Figure 2D

Figure 3A

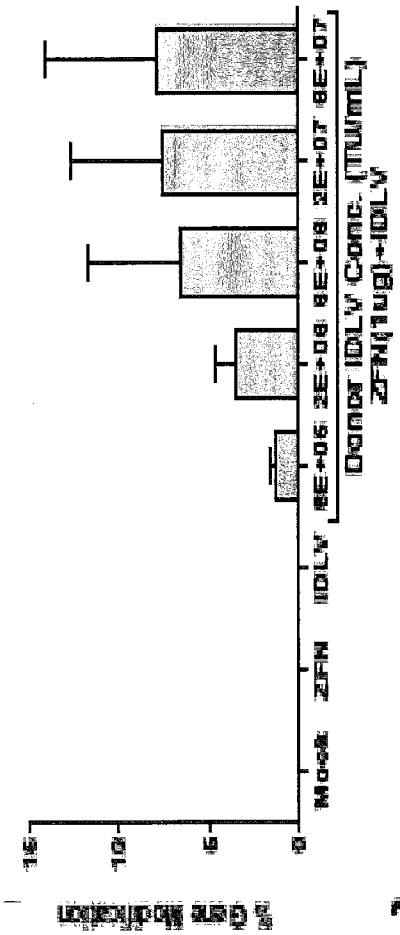


Figure 3B

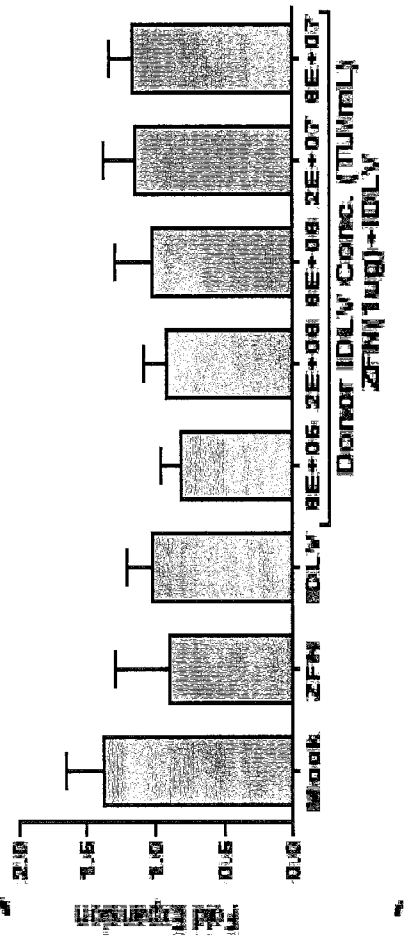


Figure 3C

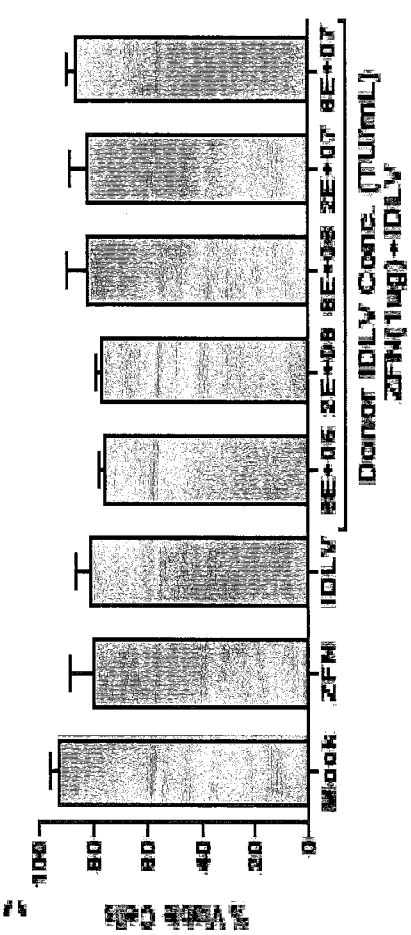


Figure 4A

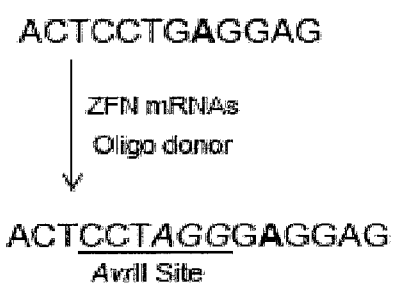


Figure 4B

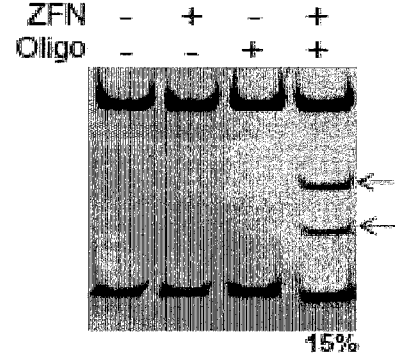


Figure 4C

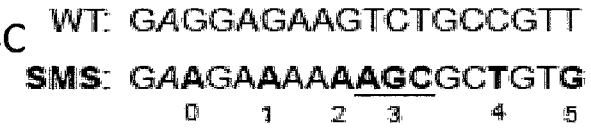


Figure 4D

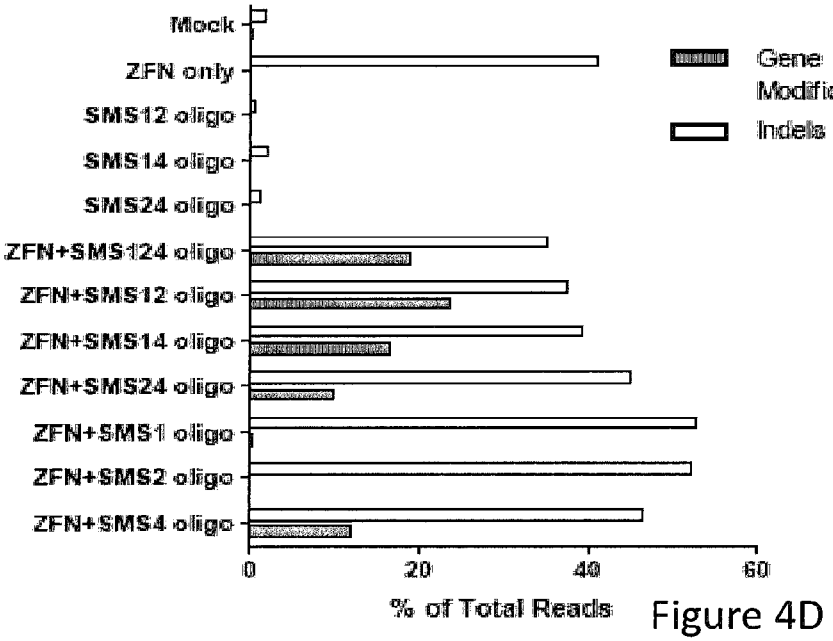


Figure 4E

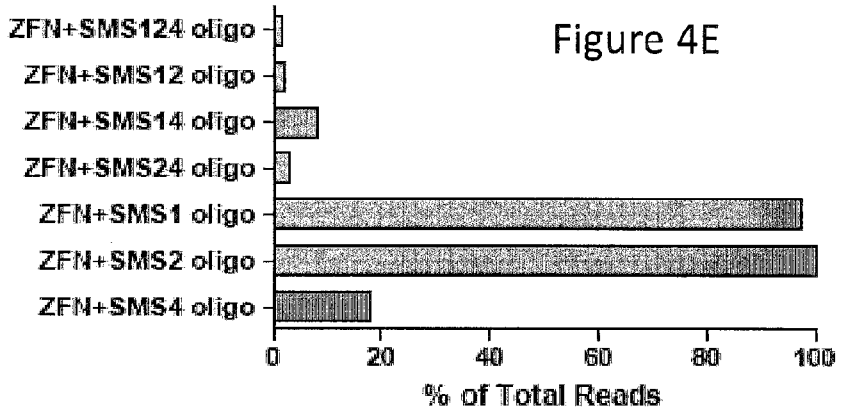
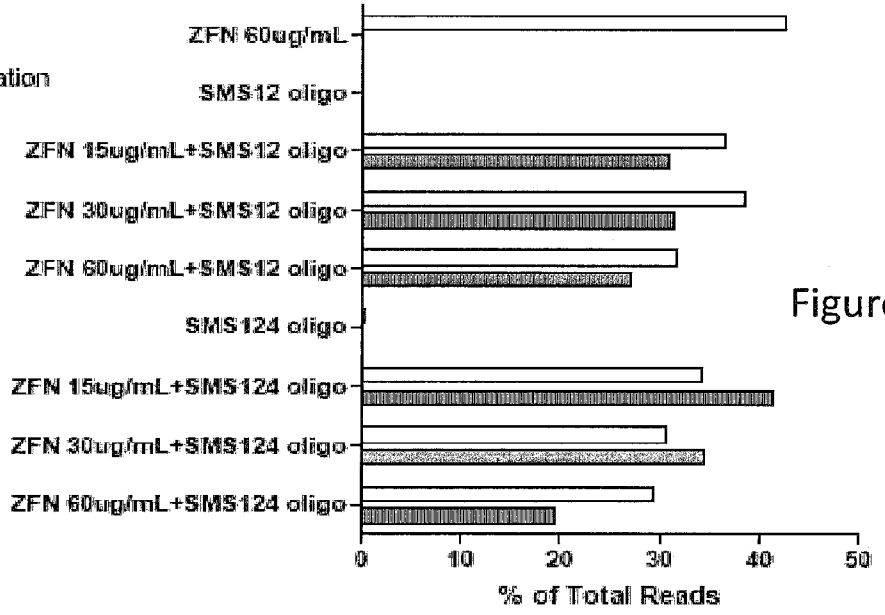


Figure 4F



SUBSTITUTE SHEET (RULE 26)

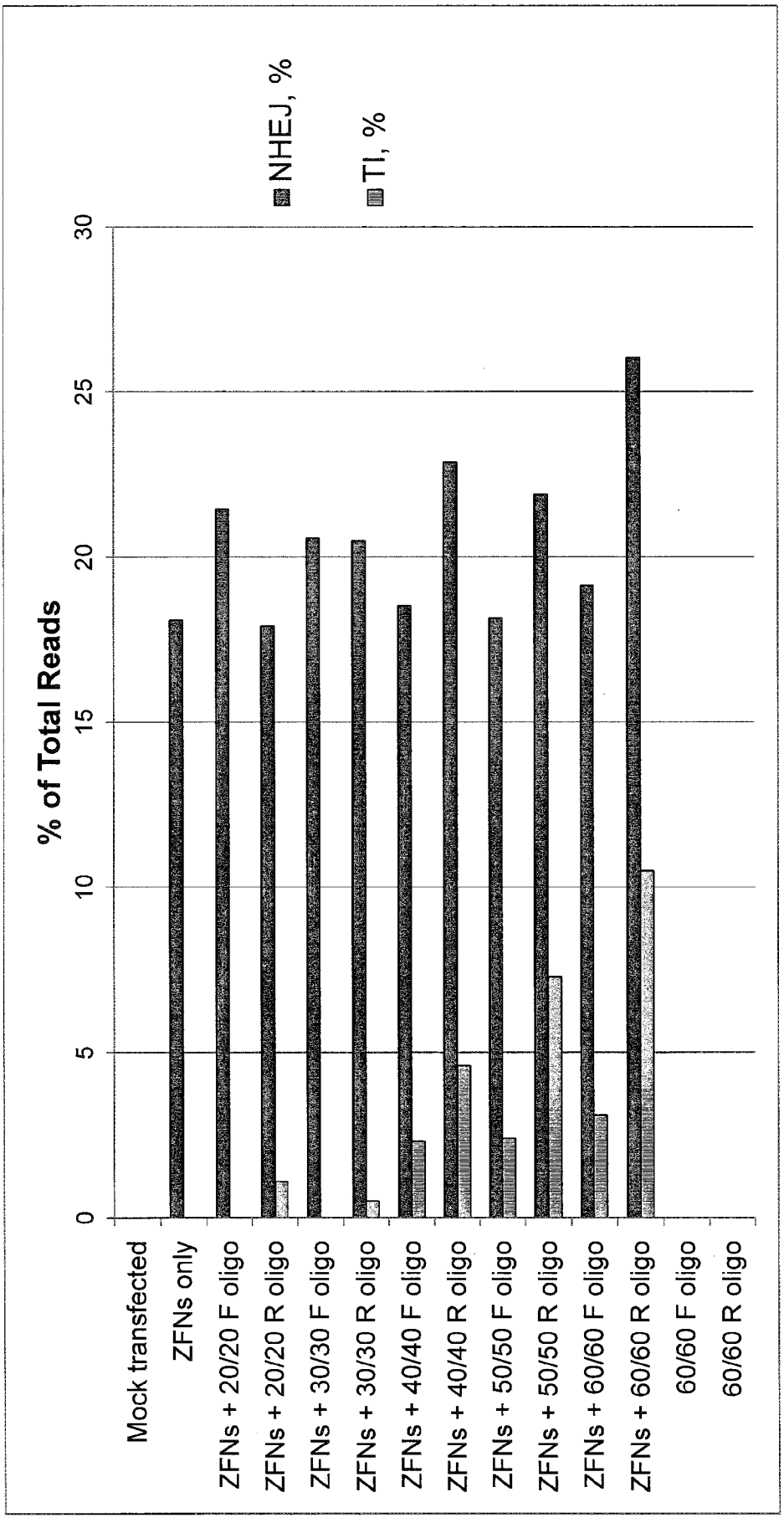

% of Total Reads
0
5
10
15
20
25
30
NHEJ, %
TI, %
Mock transfected
ZFNs only
ZFNs + 20/20 F oligo
ZFNs + 20/20 R oligo
ZFNs + 30/30 F oligo
ZFNs + 30/30 R oligo
ZFNs + 40/40 F oligo
ZFNs + 40/40 R oligo
ZFNs + 50/50 F oligo
ZFNs + 50/50 R oligo
ZFNs + 60/60 F oligo
ZFNs + 60/60 R oligo
60/60 F oligo
60/60 R oligo

Figure 5

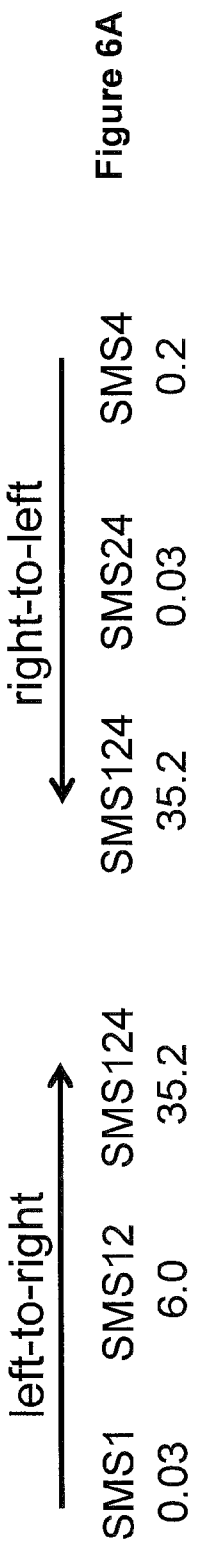


Figure 6A

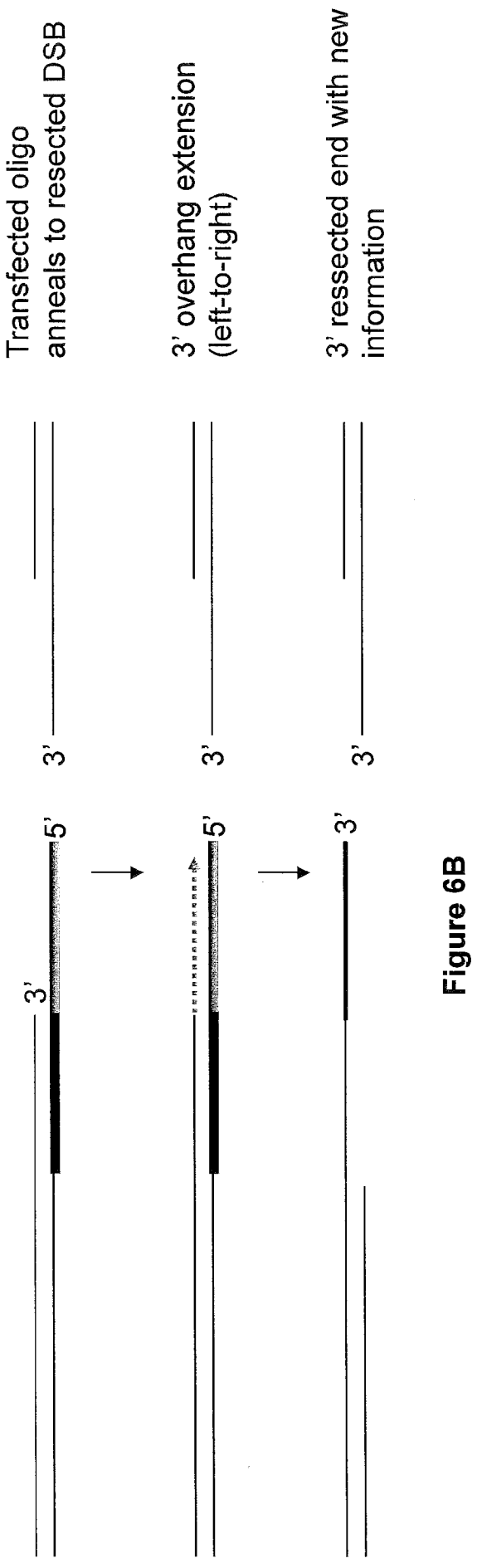


Figure 6B

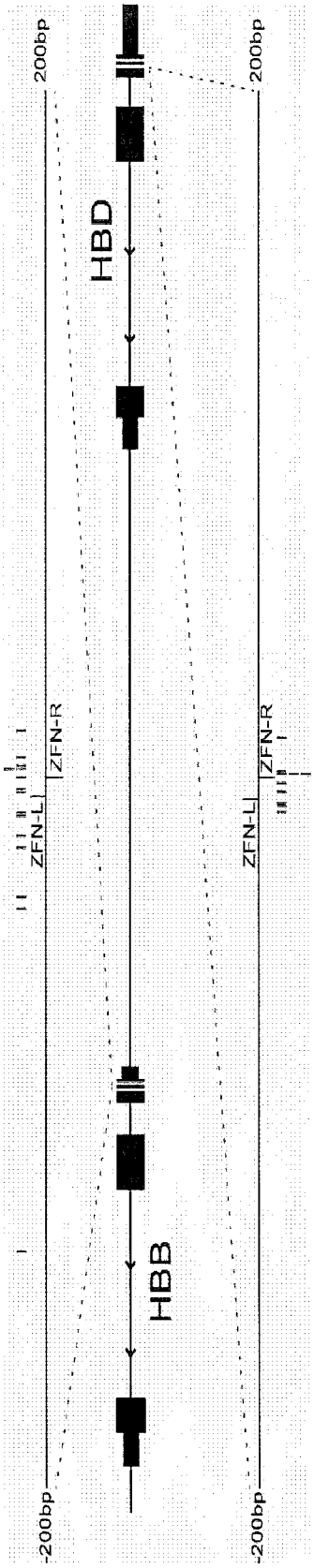


Figure 7A

Figure 7B

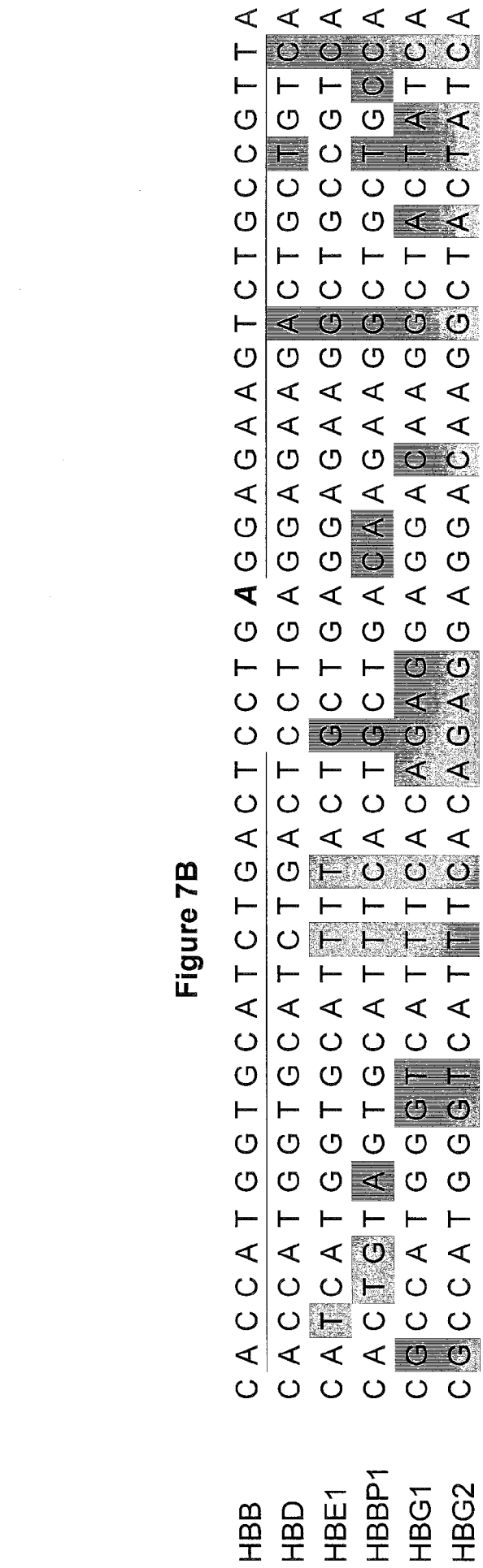


Figure 7C

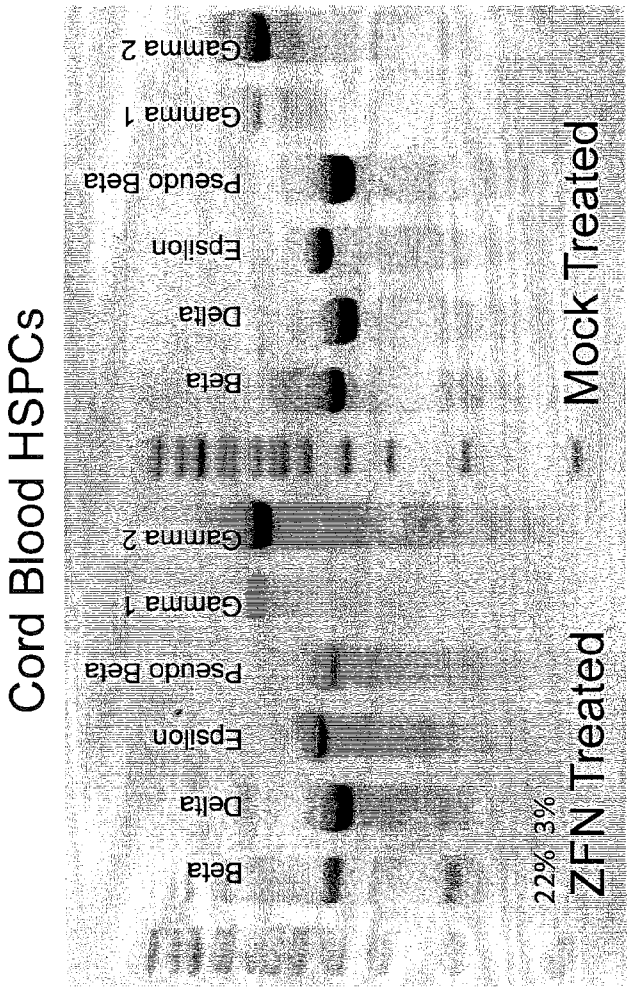
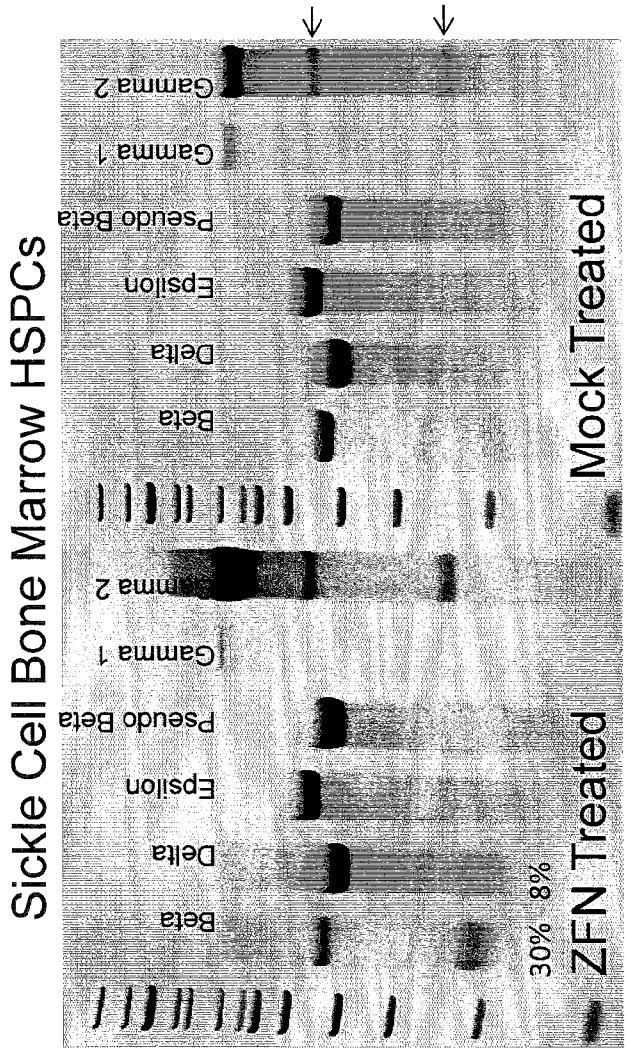


Figure 7D



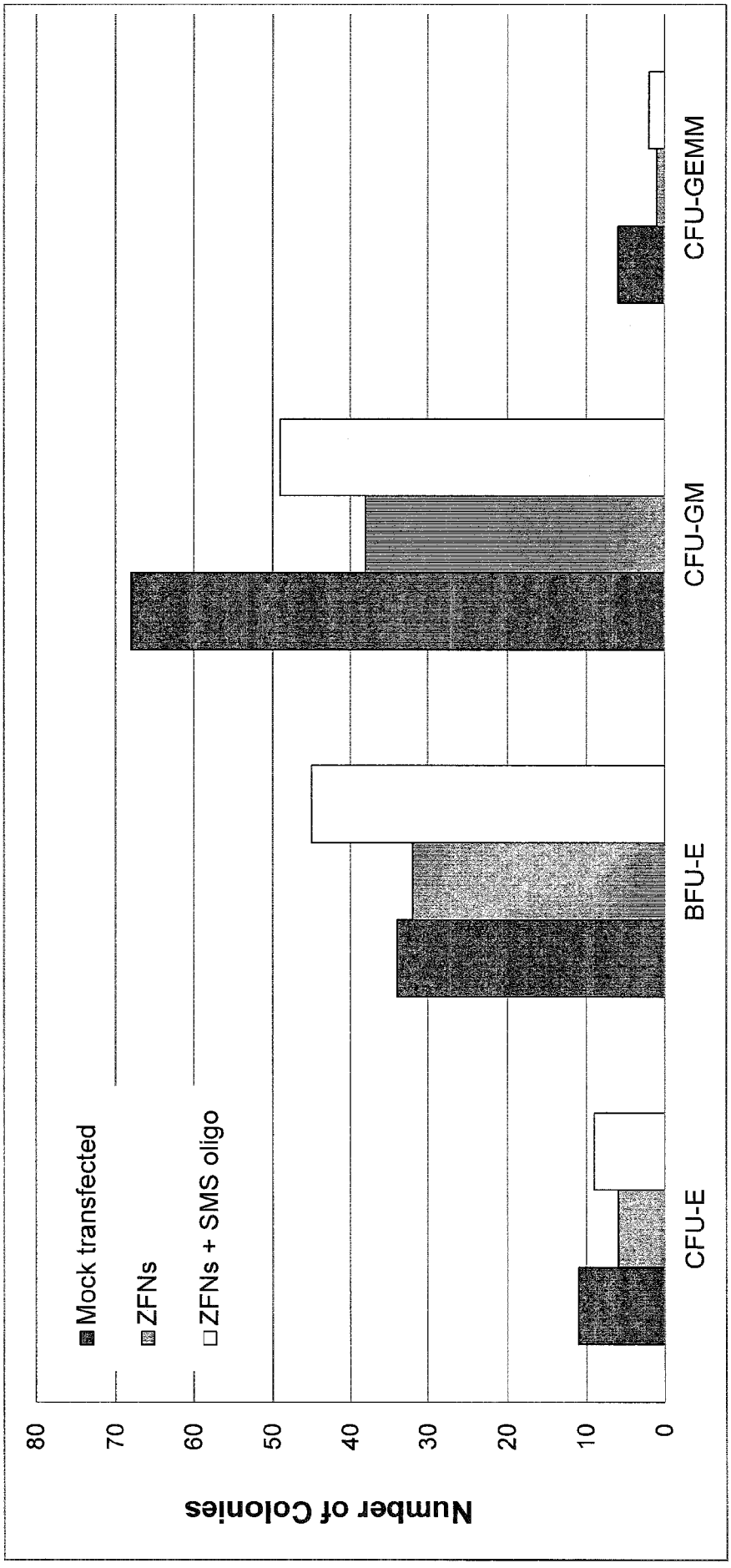

Number of Colonies
80
70
60
50
40
30
20
10
0
Mock transfected
ZFNs
ZFNs + SMS oligo
CFU-E
BFU-E
CFU-GM
CFU-GEMM

Figure 8

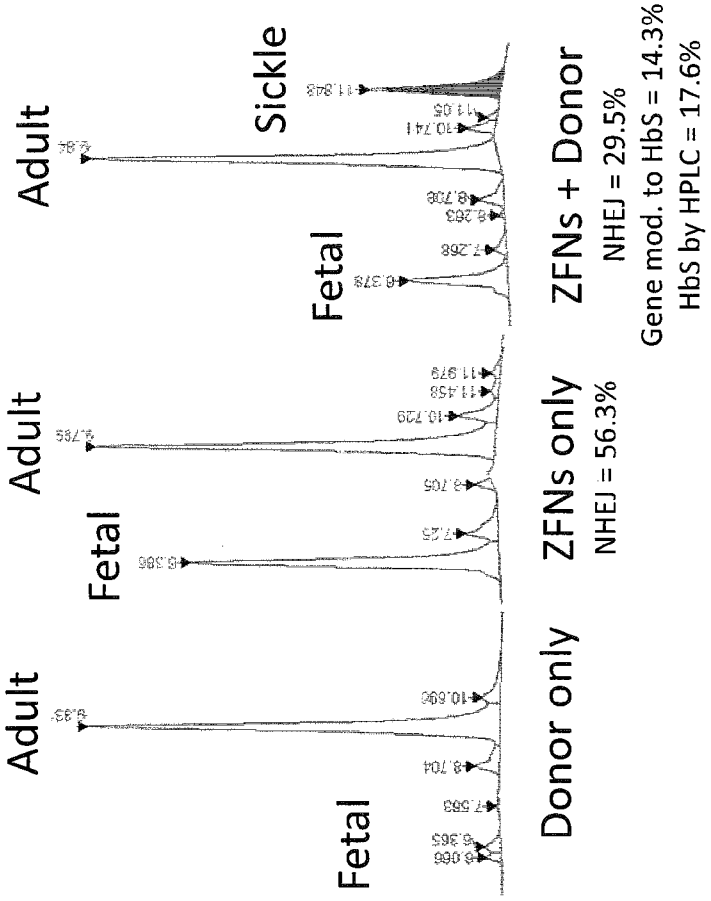


Figure 9B

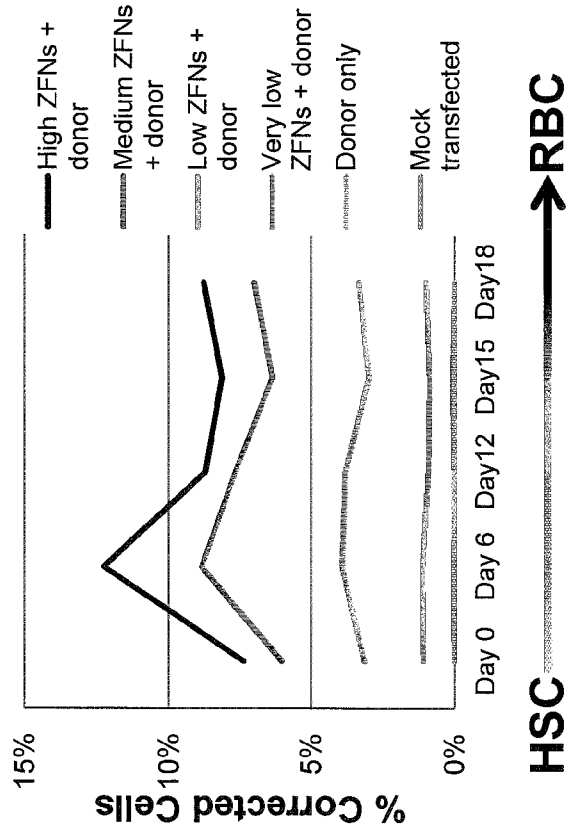


Figure 9A

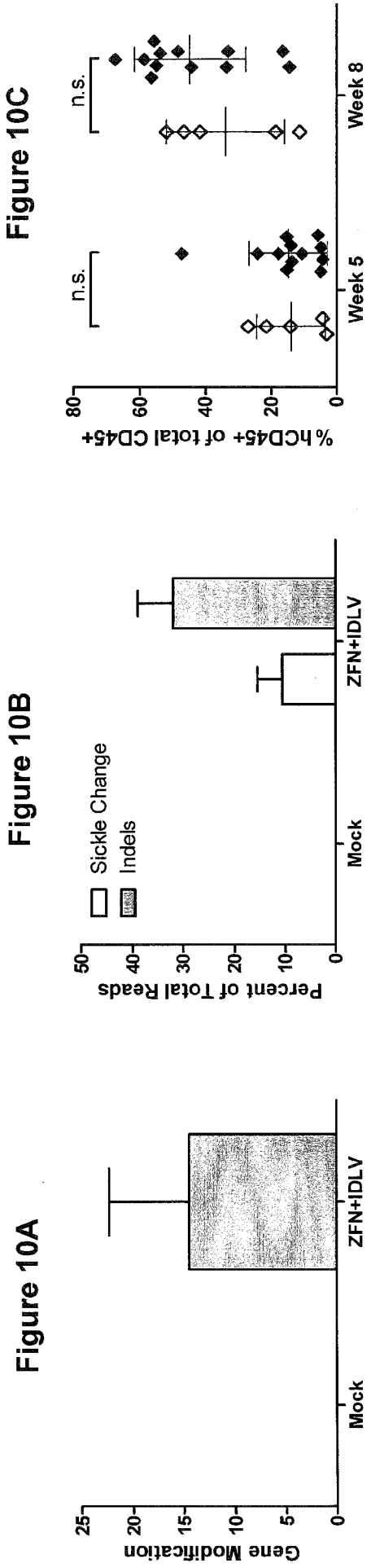


Figure 10A

Figure 10B

Figure 10C

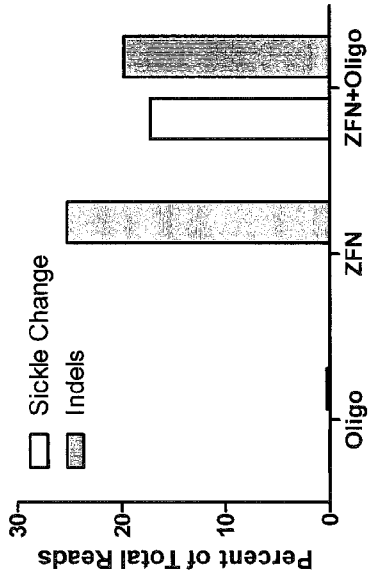


Figure 10D

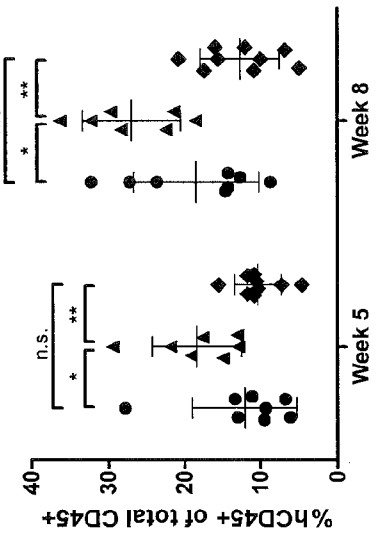


Figure 10E

Figure 11A

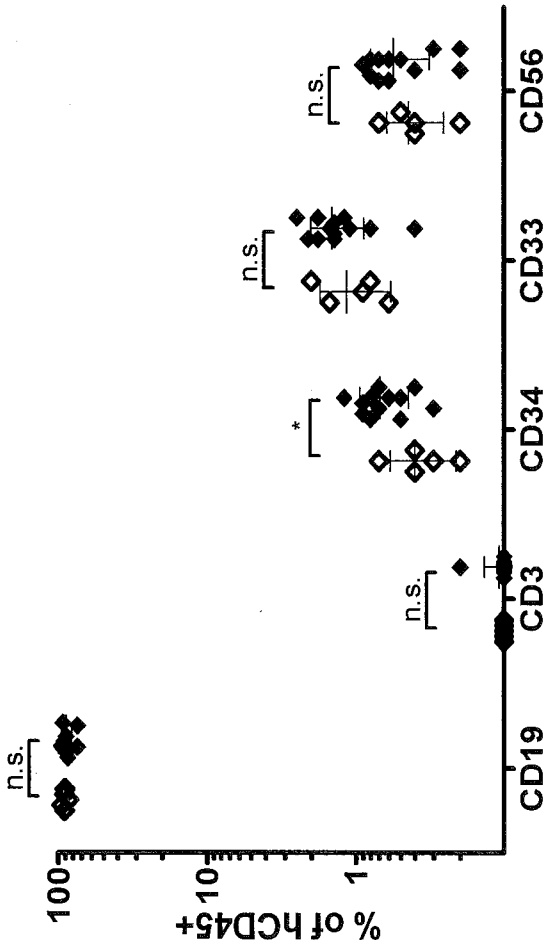
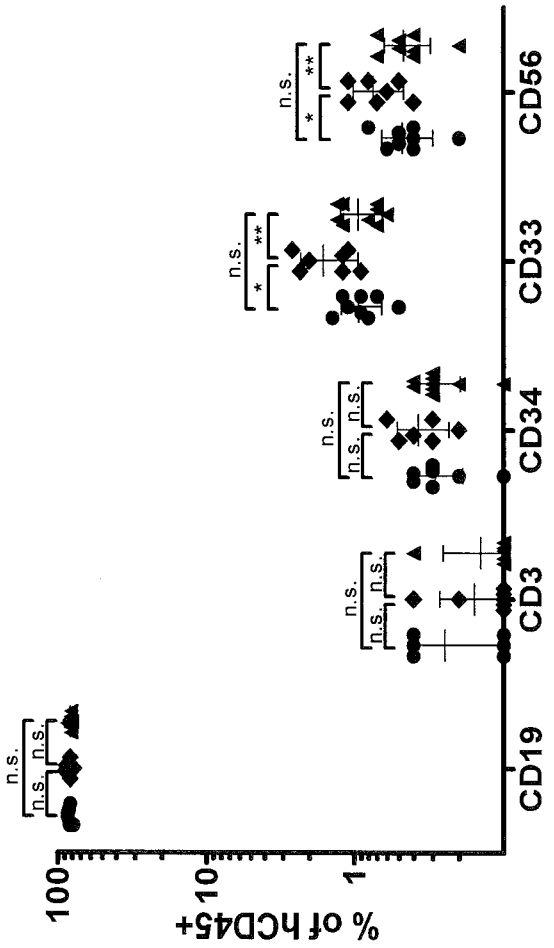
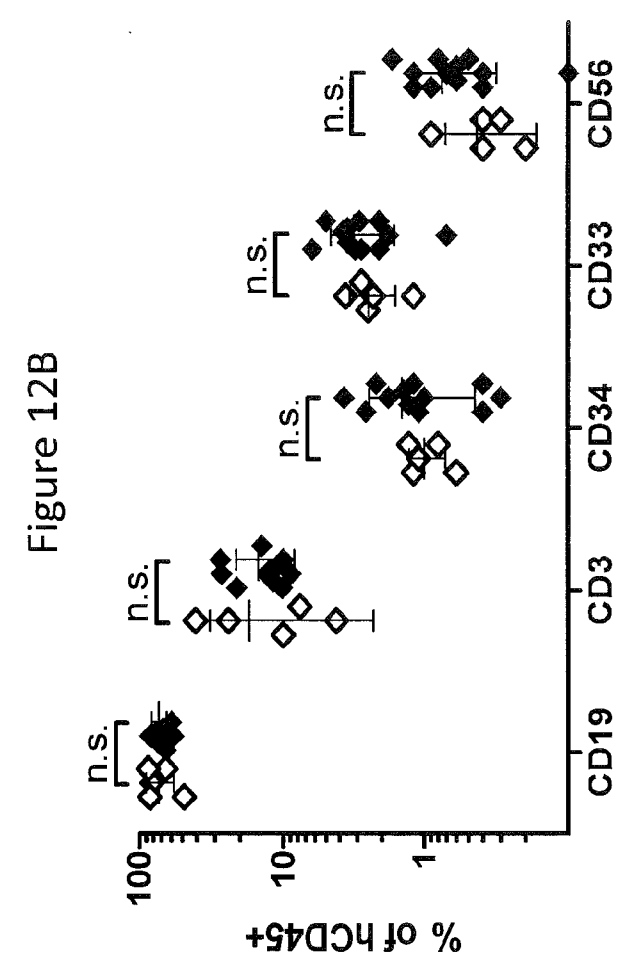
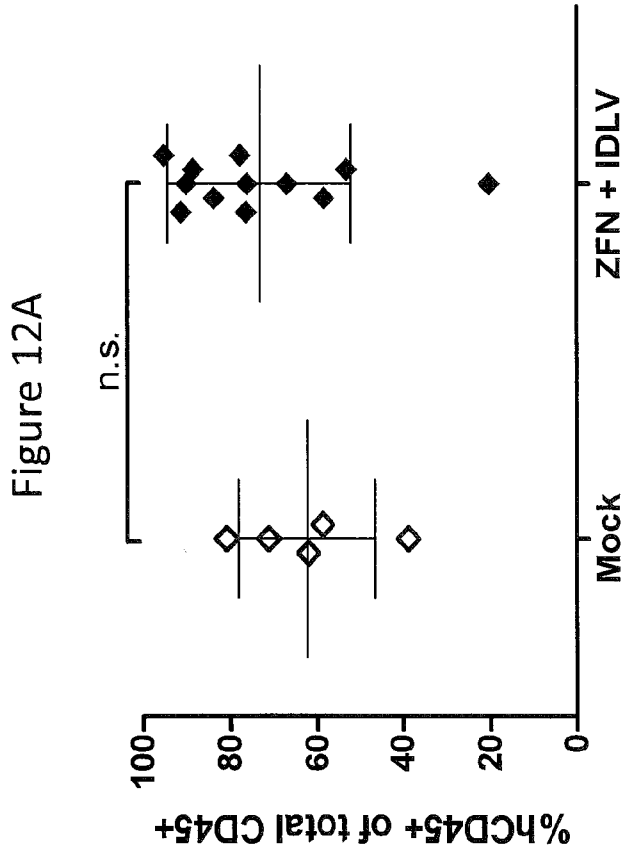
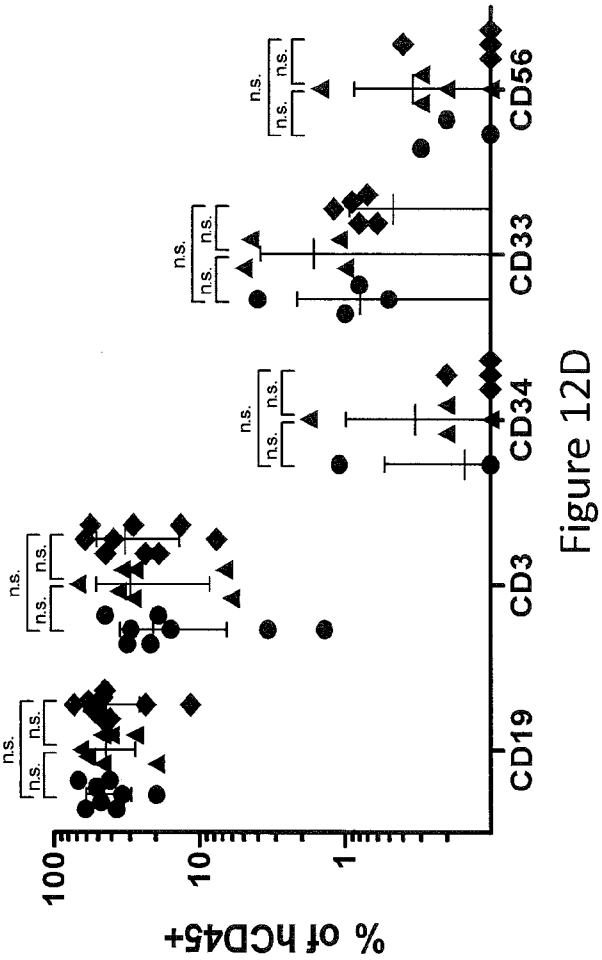


Figure 11B




Figure 12B
% of hCD45+
100
10
1
n.s.
CD19
CD3
CD34
CD33
CD56


Figure 12A
%hCD45+ of total CD45+
100
80
60
40
20
0
n.s.
Mock
ZFN + IDLV


Figure 12D
% of hCD45+
100
10
1
n.s.
CD19
CD3
CD34
CD33
CD56

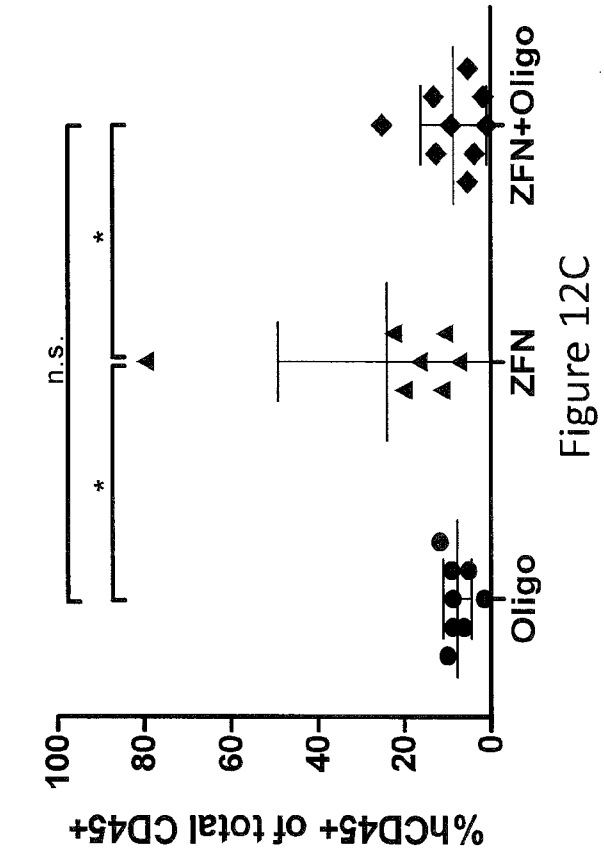

Figure 12C
%hCD45+ of total CD45+
100
80
60
40
20
0
n.s.
*
*
Oligo
ZFN
ZFN+Oligo

Figure 13A

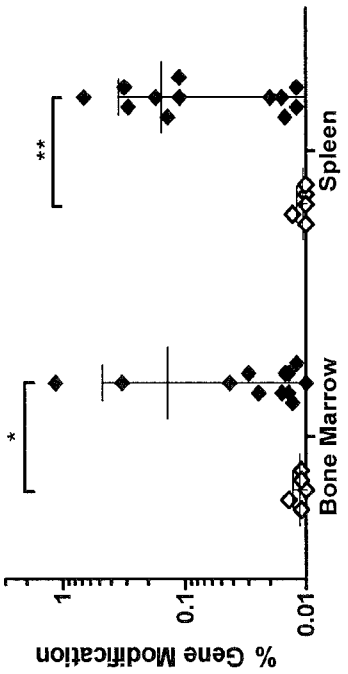


Figure 13B

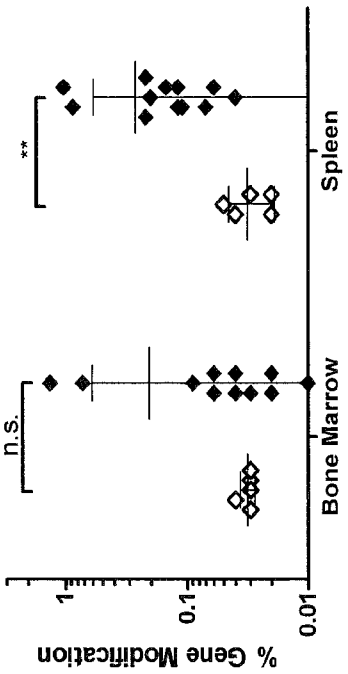
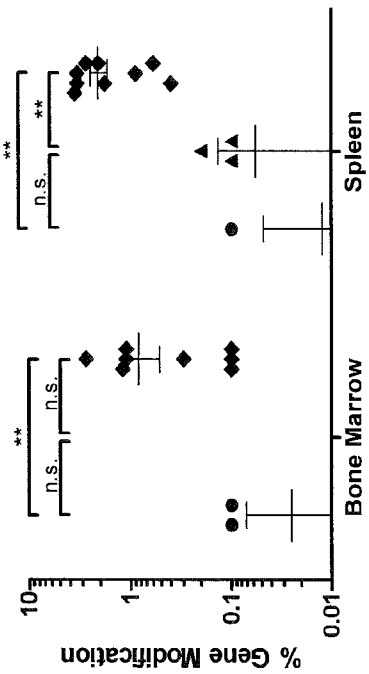


Figure 13C



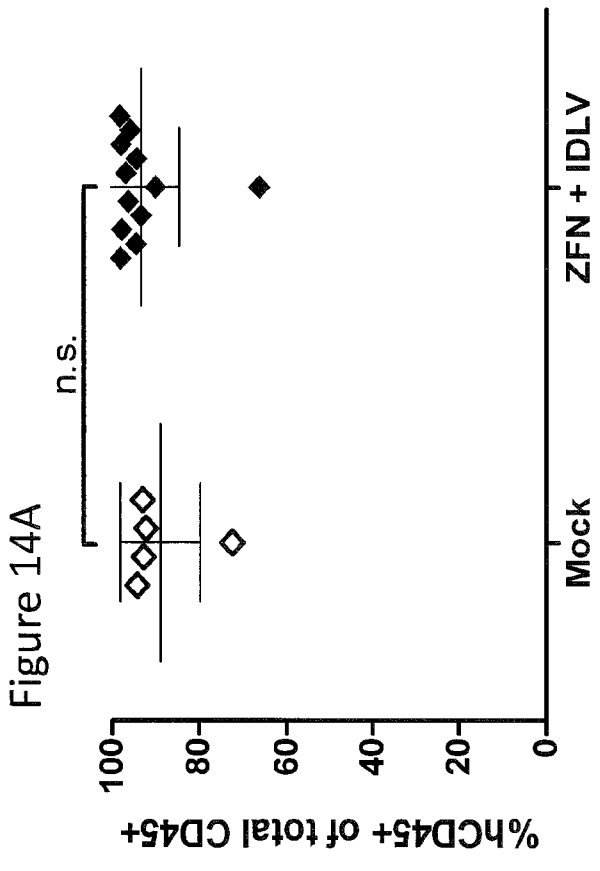


Figure 14A

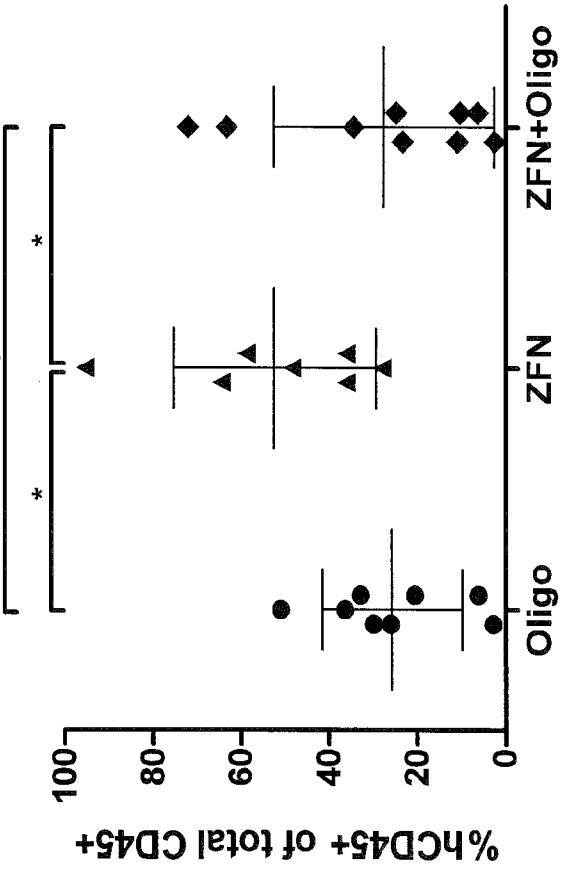


Figure 14C

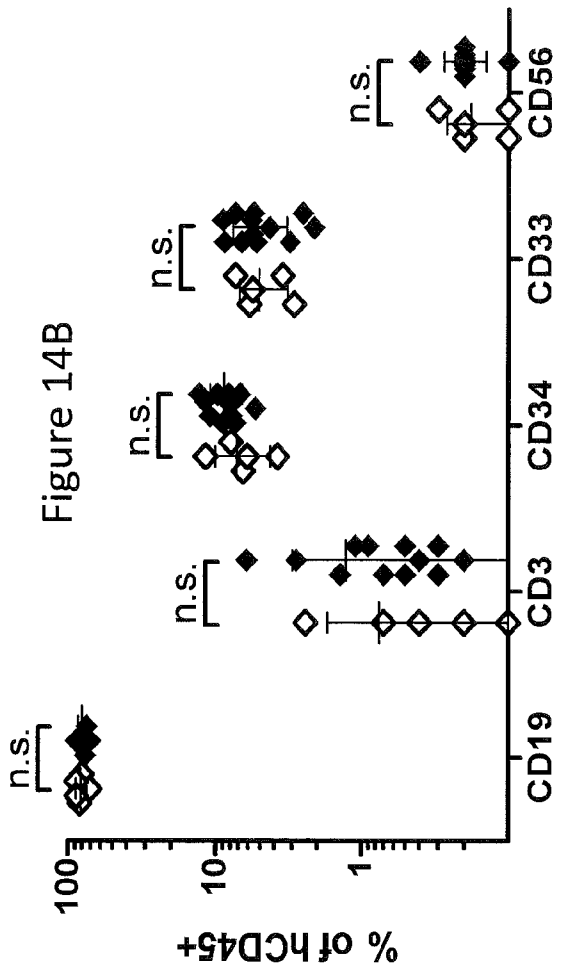


Figure 14B

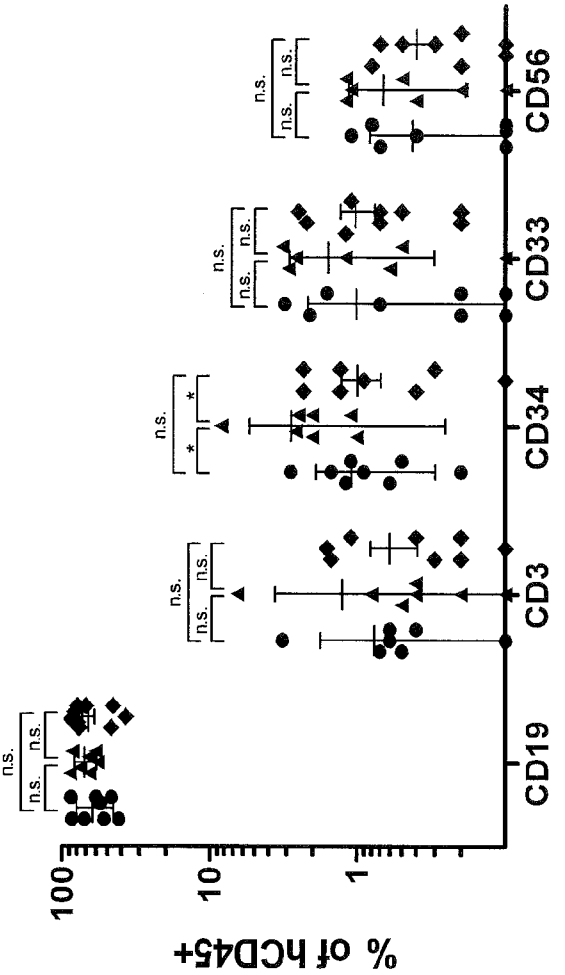


Figure 14D

Figure 15A

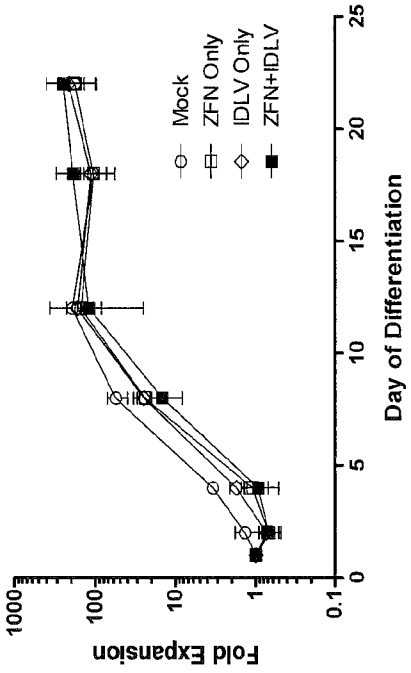


Figure 15B

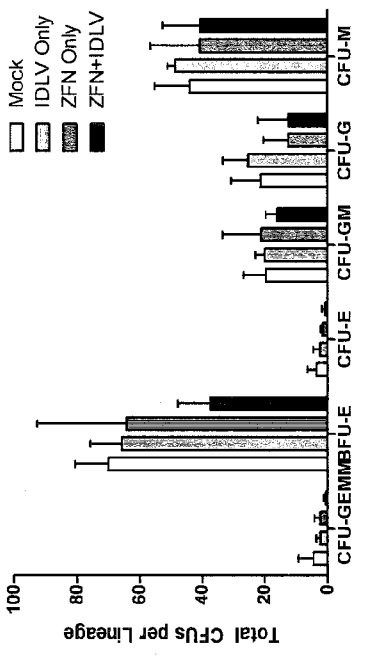
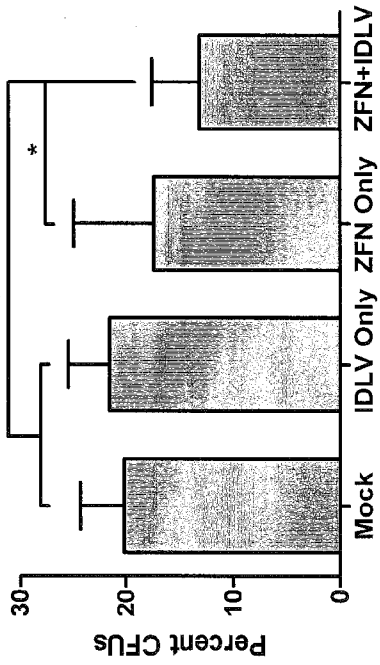


Figure 15C



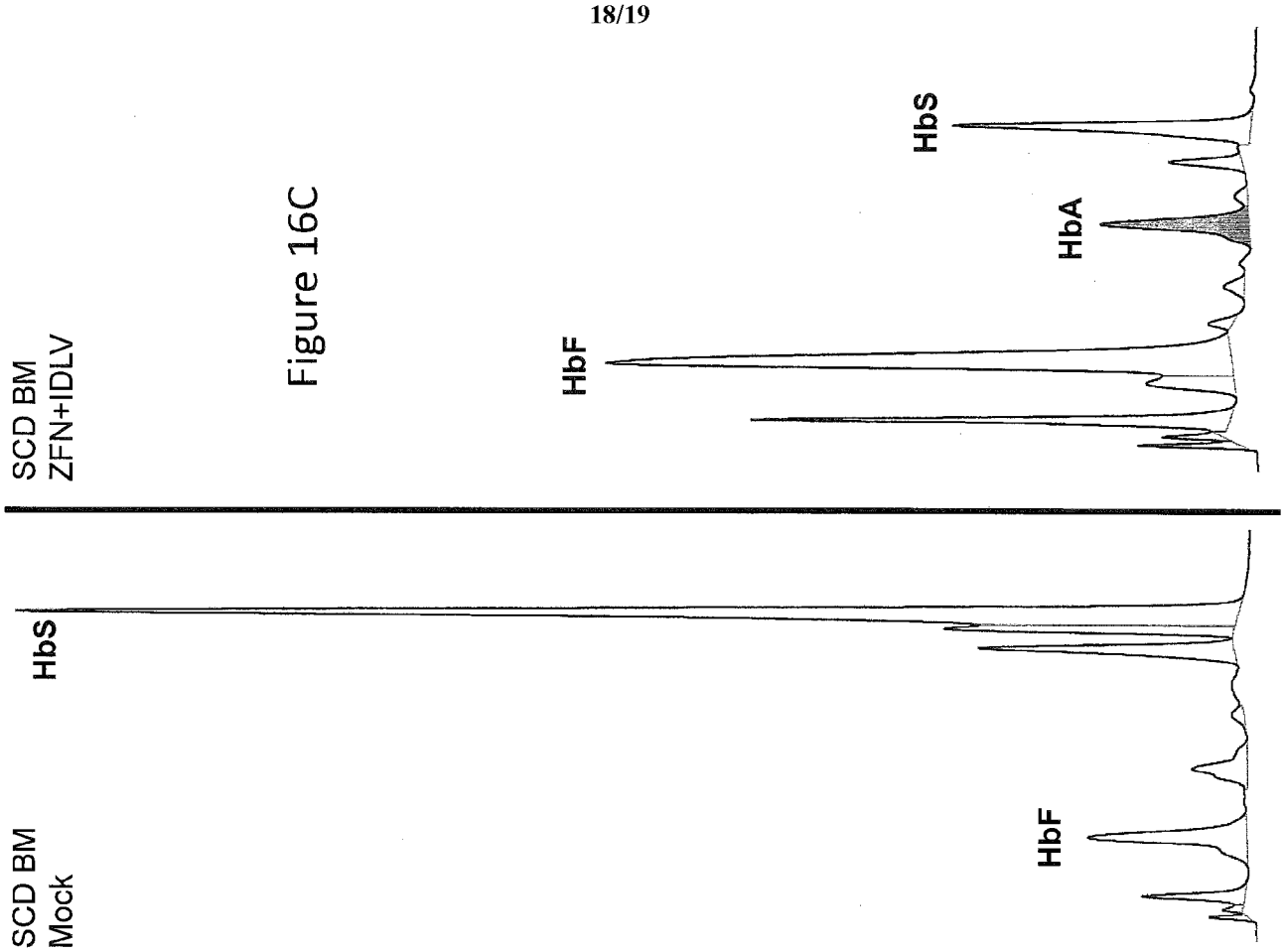


Figure 16C

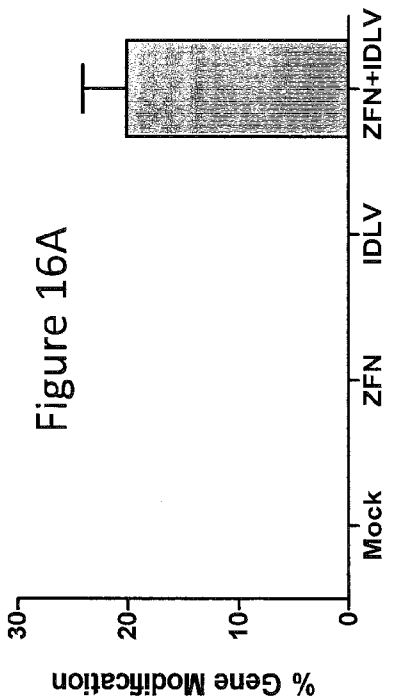


Figure 16A

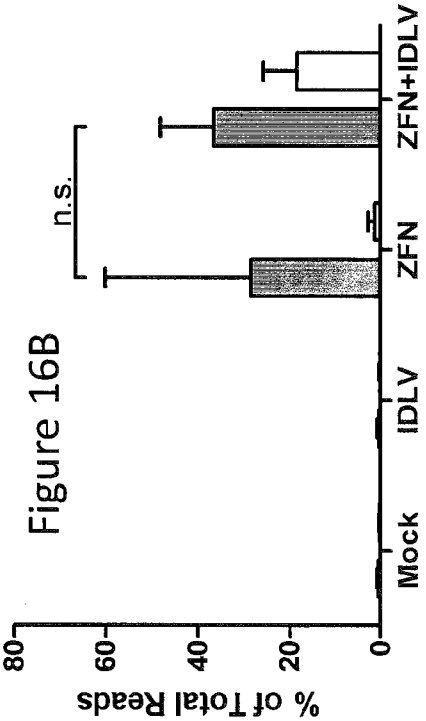


Figure 16B

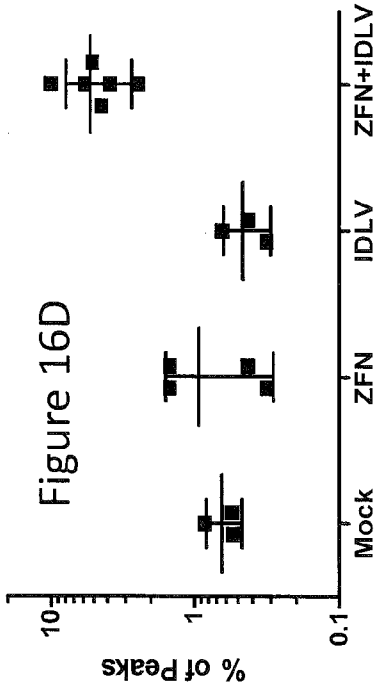


Figure 16D

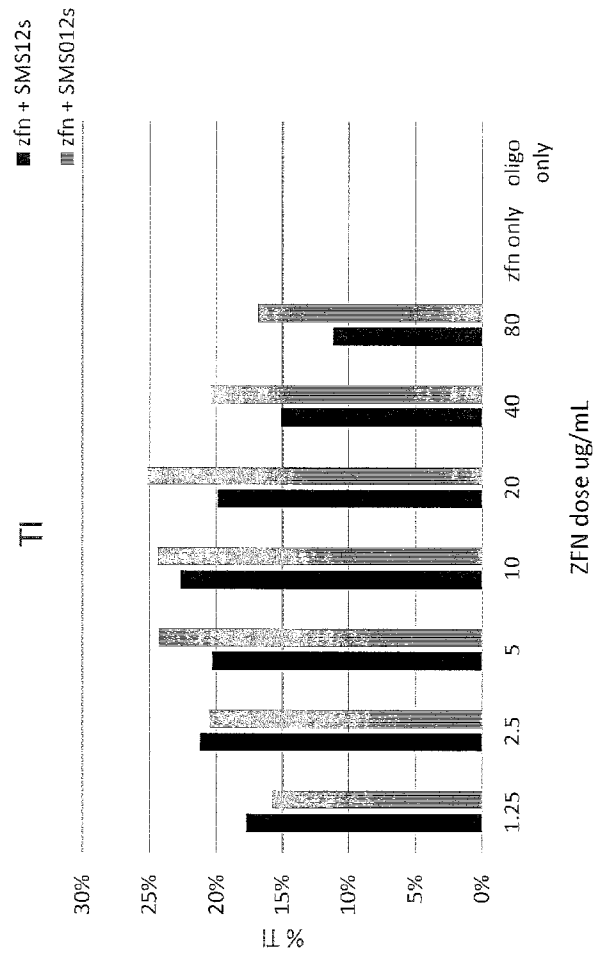


Figure 17B

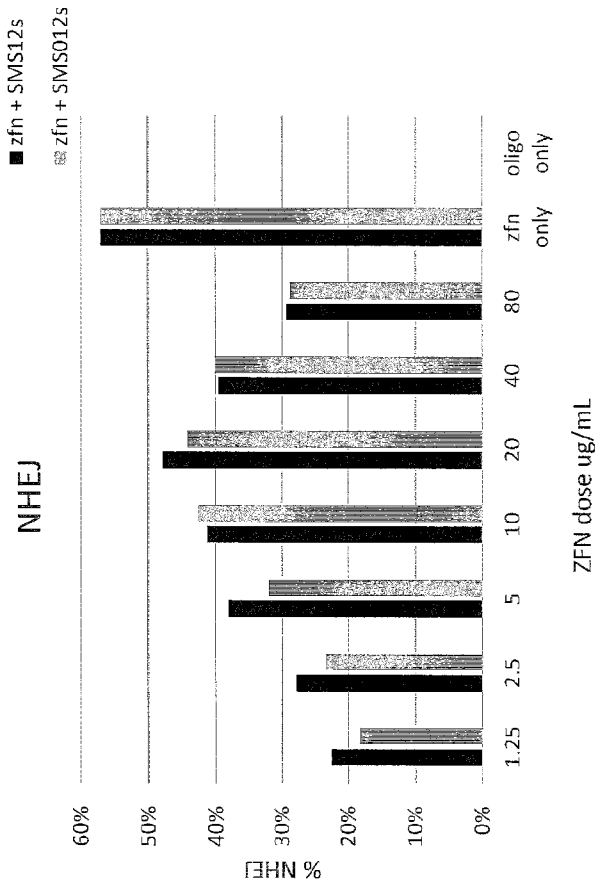


Figure 17A