

Exon 1

5' cgagATGTCTCGCTCCGT**A**GGCTTAA**A**GCTGTGCTCGCGTACTCTCTCTTCTGGCCTGGAGGCTATCCAGC

Exon 2

5' GTACTCCAAAGATTCAGGTTTACTCACGTCA**TCCAGCAGAGAA**TGGAAAG**TCAAAT**TTCCCTGAATTGCTCAAATTTCTGG
GTTTCATCCGACATTGAAGTTGAC**TTACTGAAGA**ATGGAGAGAG**AAATTGAA**AAAGTGGAGCATTCAGACTTGTCT

TTCAGCAAGGACTGGTCTTTCATCTCTTGTACTACACTGAATTCACCCCCACTGAAAAAGATGAGTATGCCCTGCCGTG
TGAACCATGTGACTTTGTCA**CAGCCC**CAAGATAGTTAAGTGGG

FIG. 1

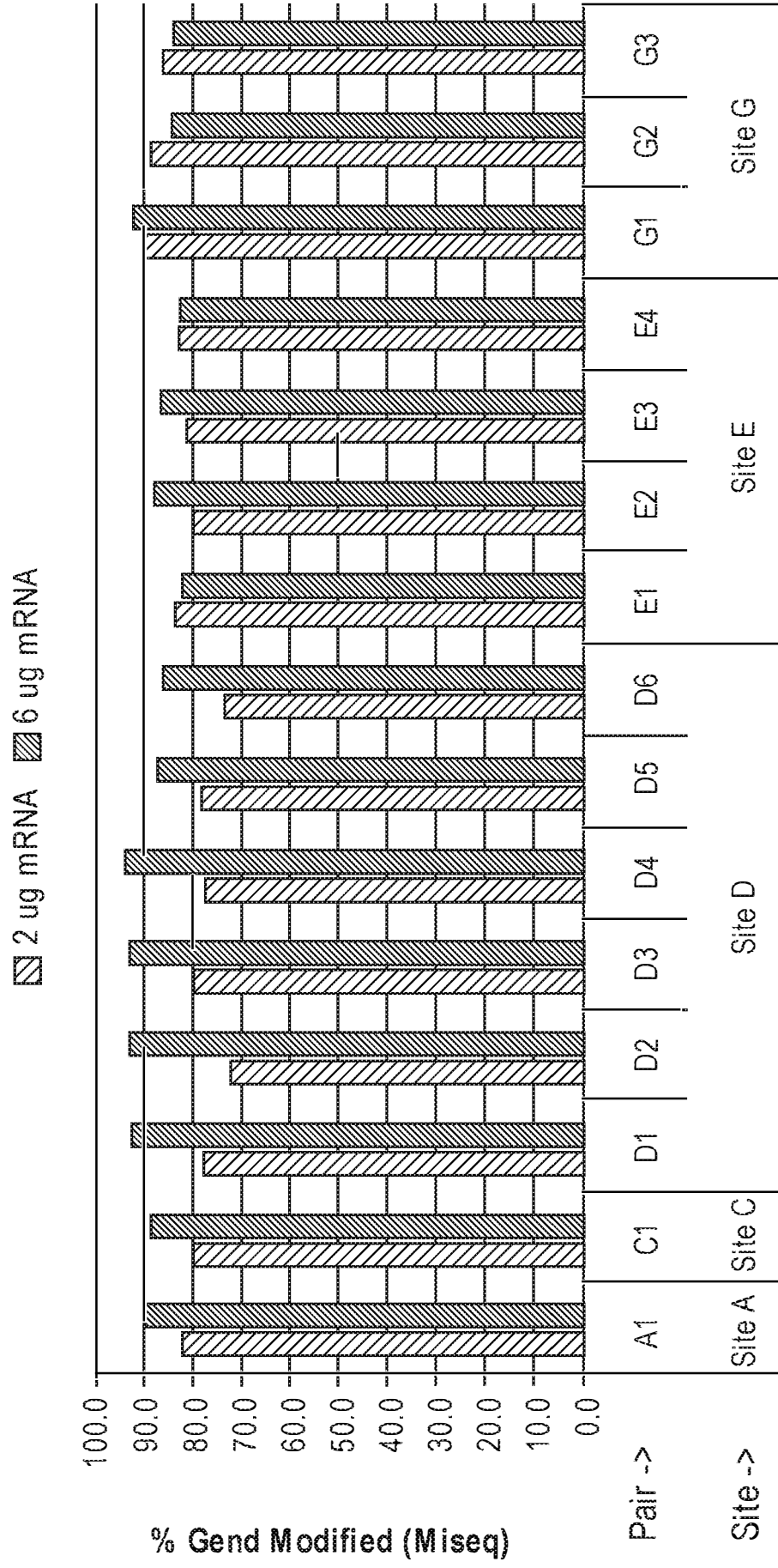


FIG. 2

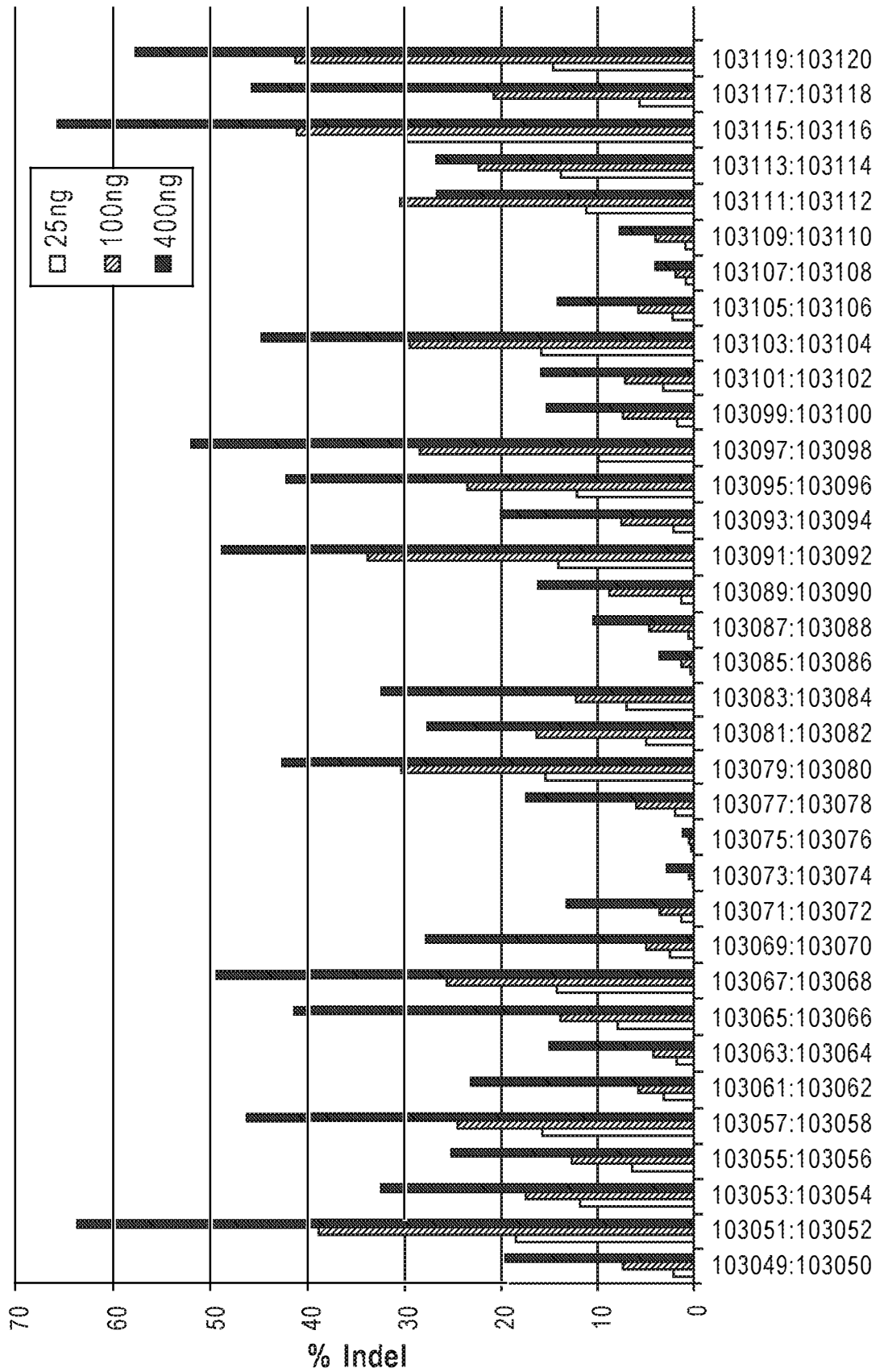


FIG. 2B

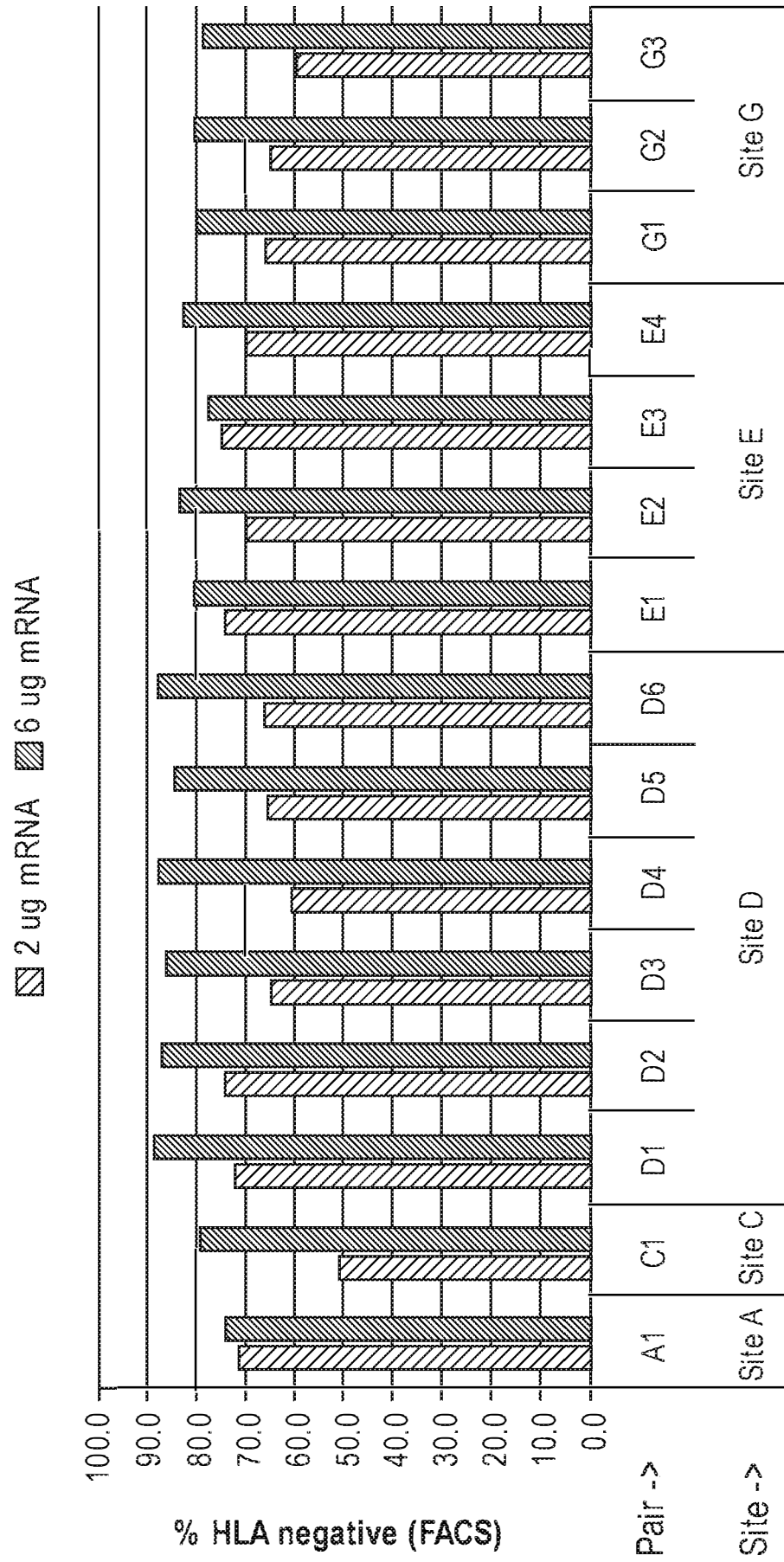
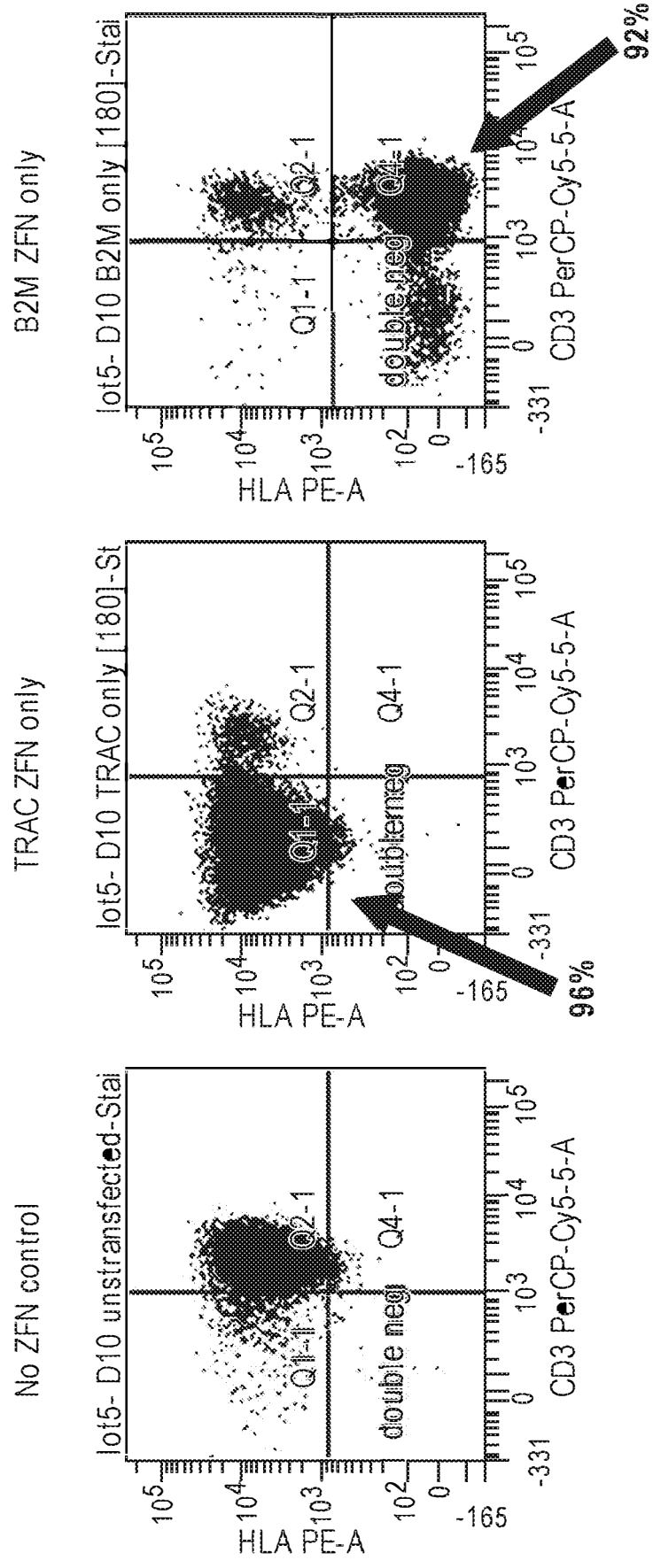


FIG. 3



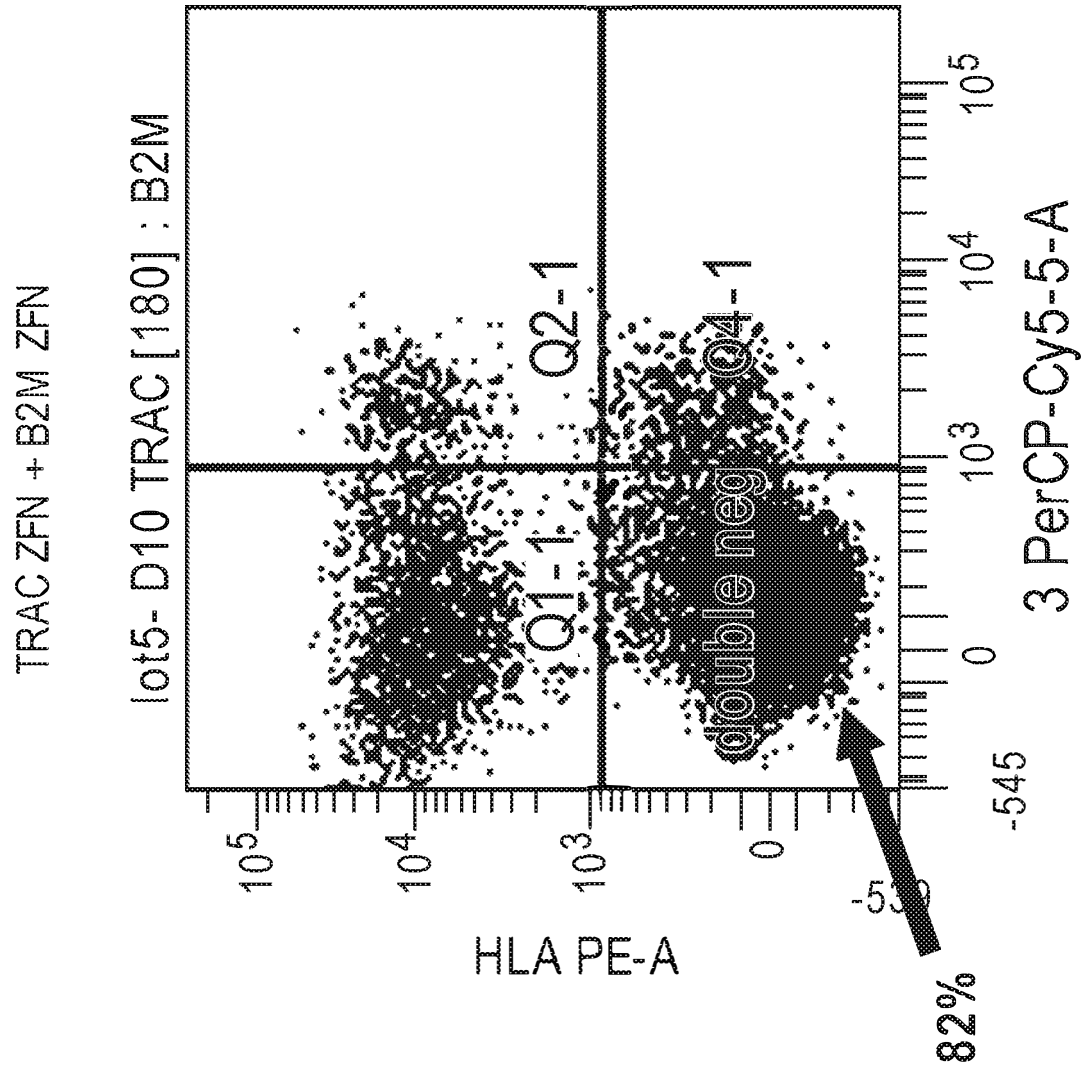


FIG. 4E

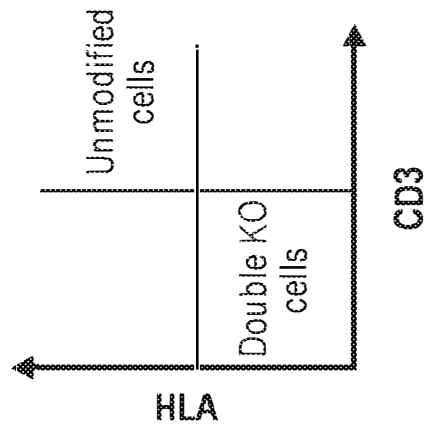


FIG. 4D

	FACS				
	D10				
ug/mL	TRAC-	B2M-	DOUBLE-	Total GFP+	DOUBLE-GFP+
sham	0.5	0.1	0.0	0.0	0.0
TRAC/B2M KO only	85.0	83.6	80.0	0.0	0.0
TRAC/B2M KO + 1E5vg/cell TRAC locus AAV donor	91.9	92.7	89.3	80.8	83.0
TRAC/B2M KO + 3E4vg/cell TRAC locus AAV donor	91.2	93.4	89.1	71.9	74.3
TRAC/B2M KO + 1E5vg/cell B2M locus AAV donor	88.2	90.5	86.4	54.9	59.6
TRAC/B2M KO + 3E4vg/cell B2M locus AAV donor	89.8	92.2	87.9	43.2	46.7

FIG. 5