

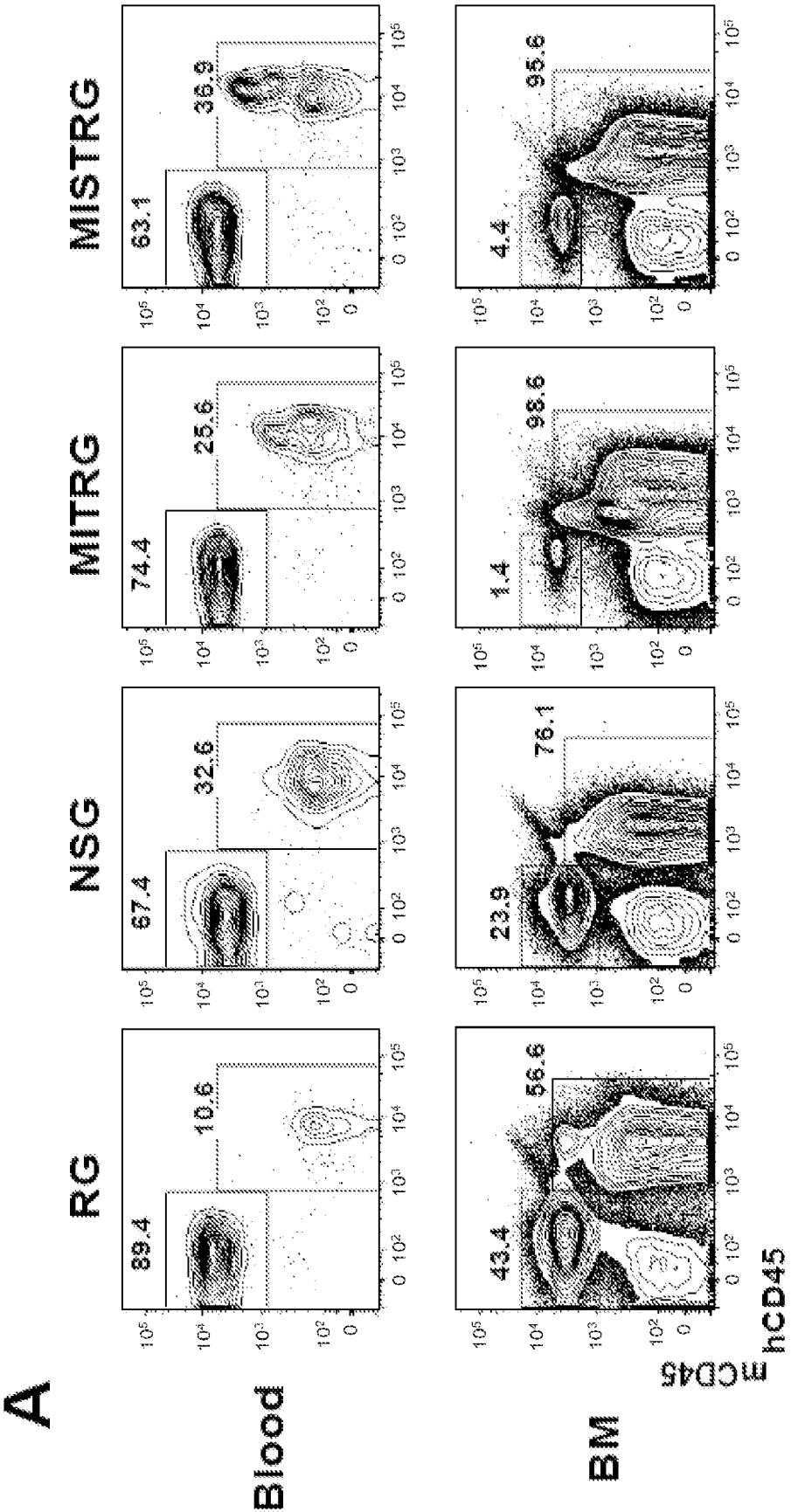


Figure 1A

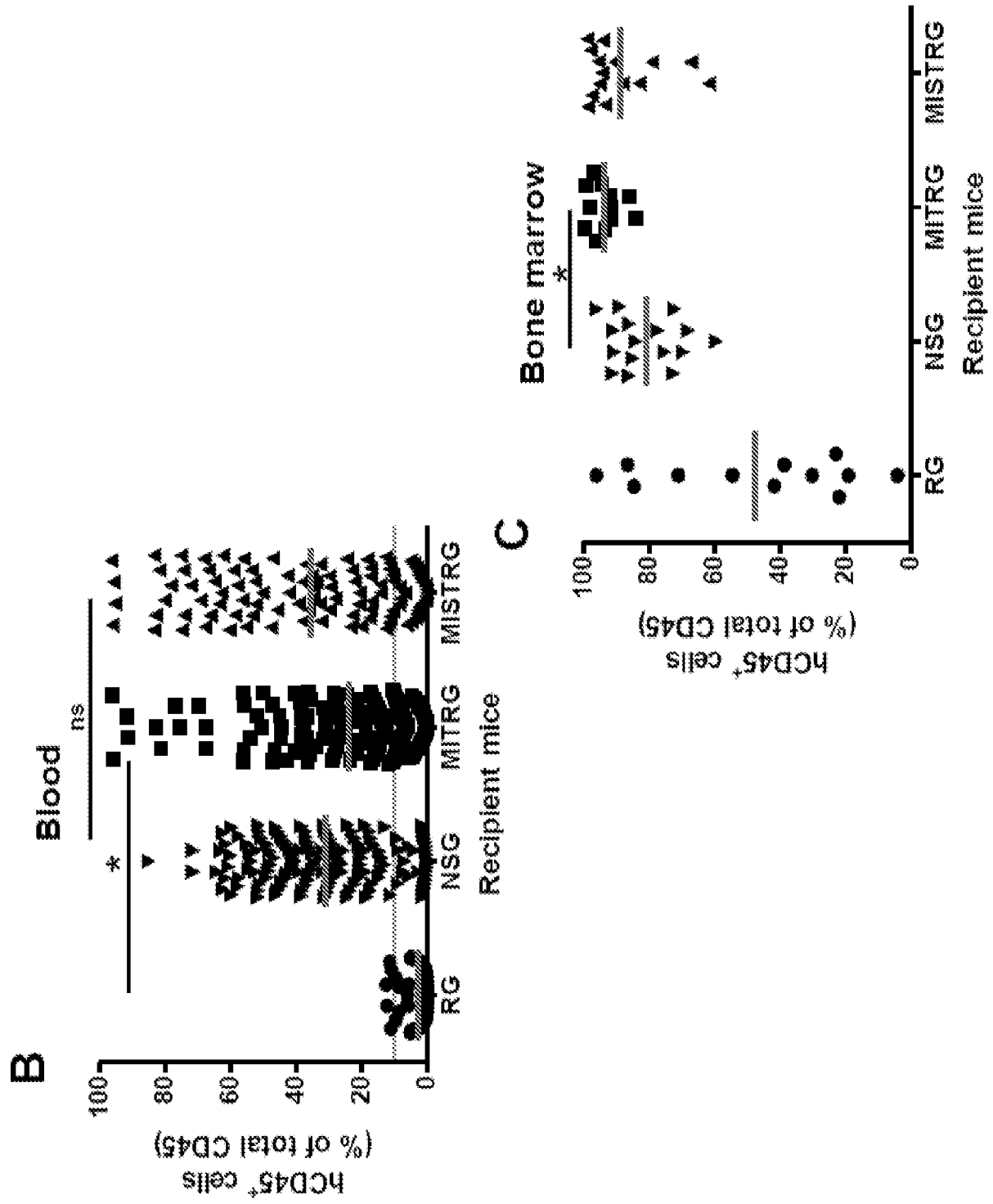


Figure 1B-1C

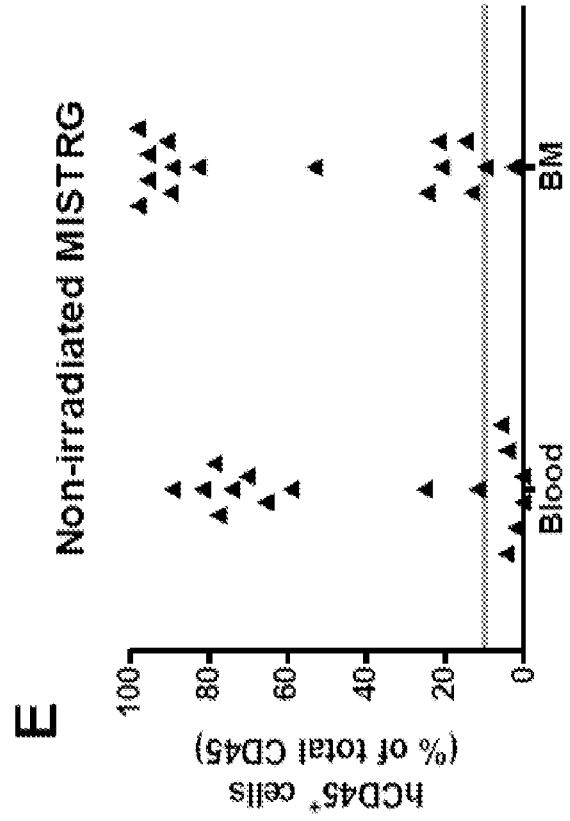
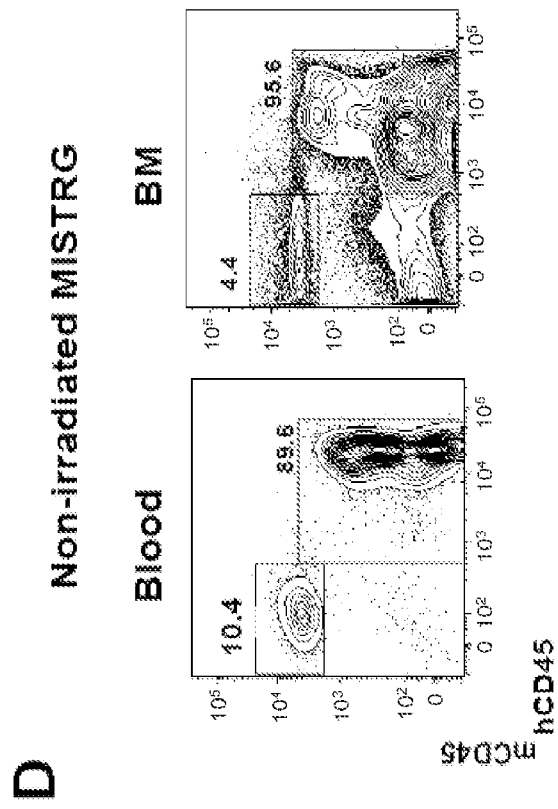


Figure 1D-1E

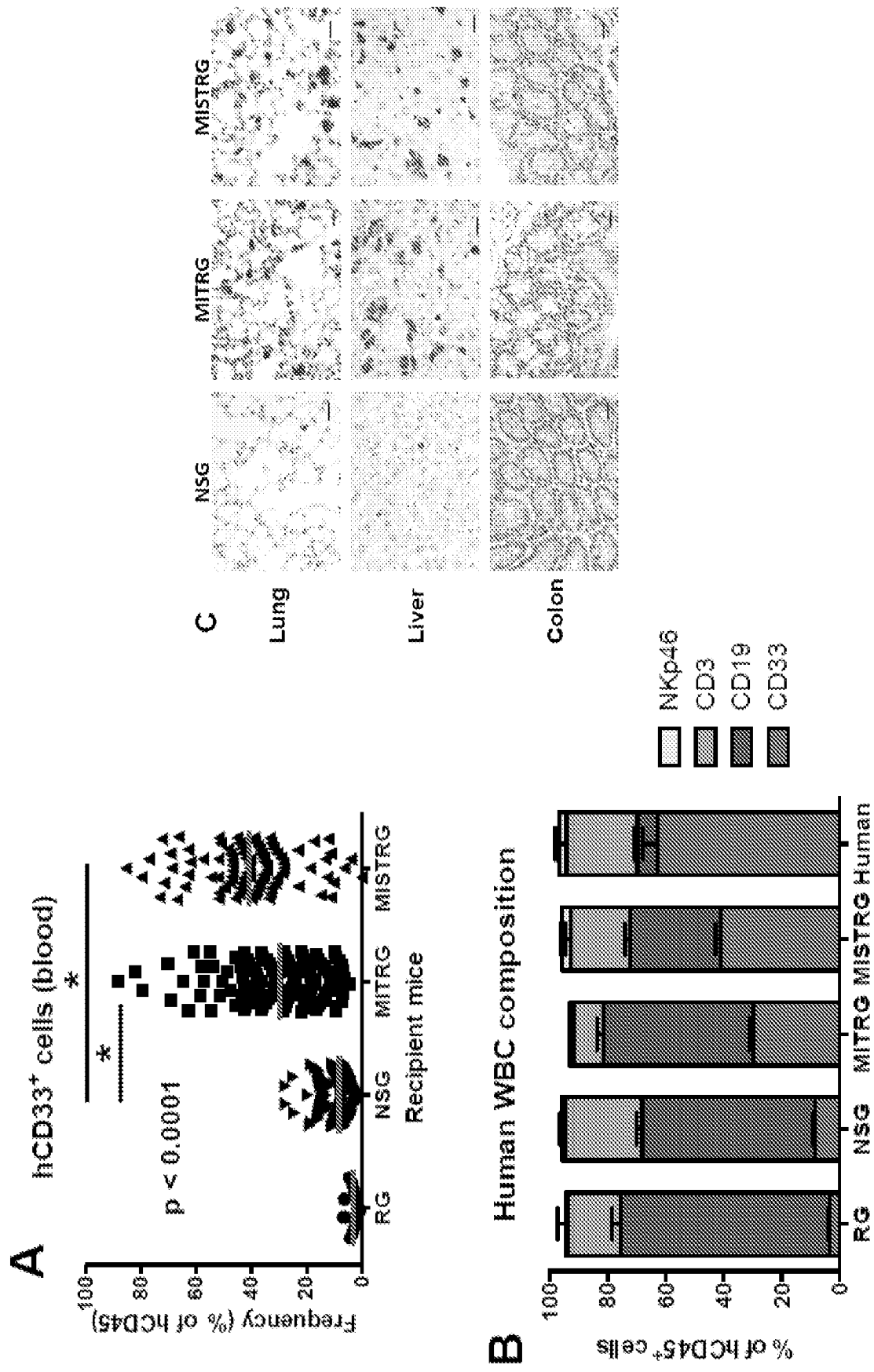


Figure 2A-2C

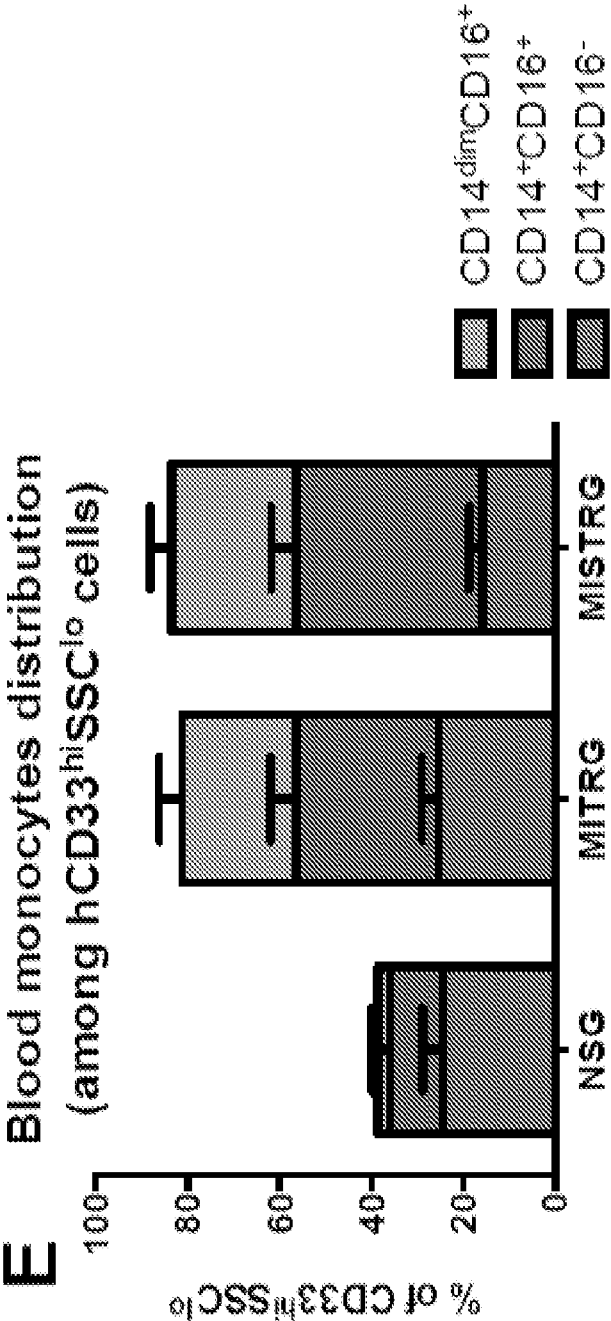
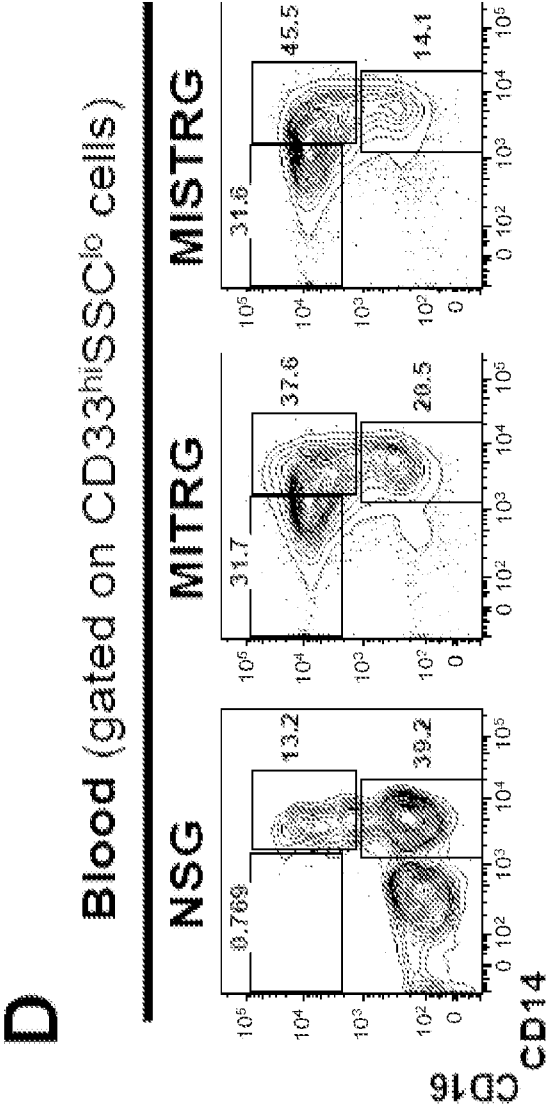
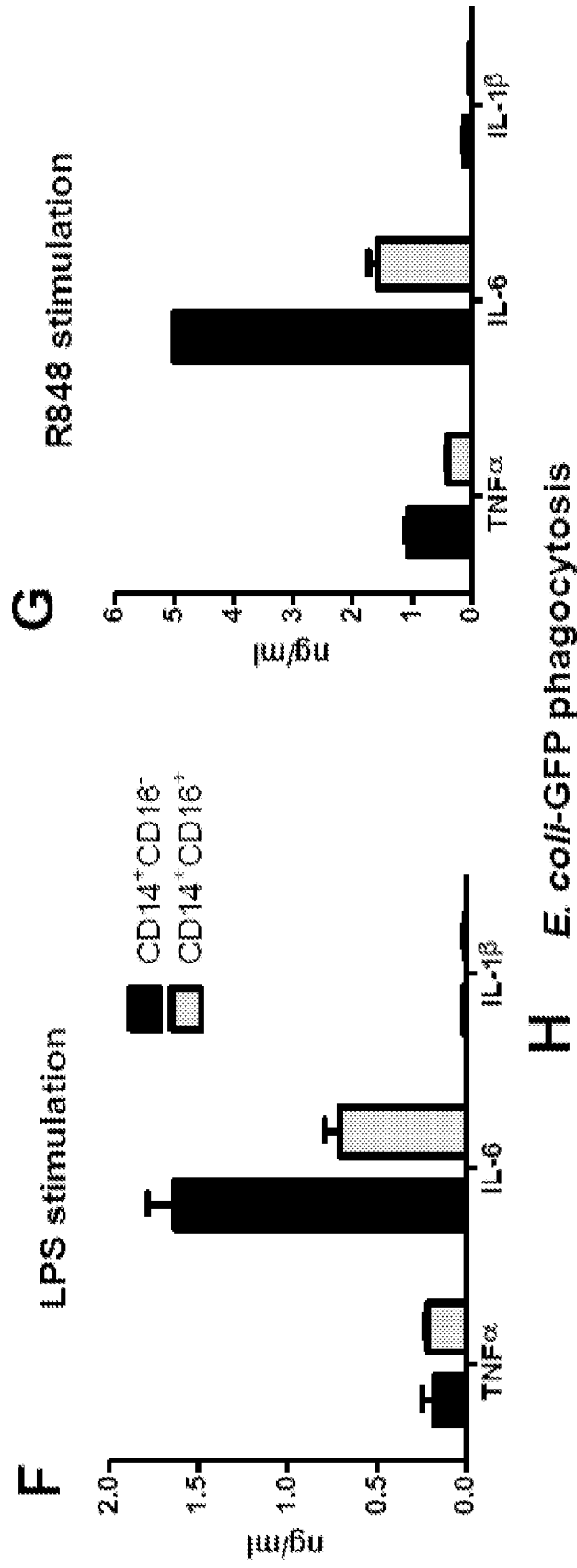


Figure 2D-2E



H *E. coli*-GFP phagocytosis

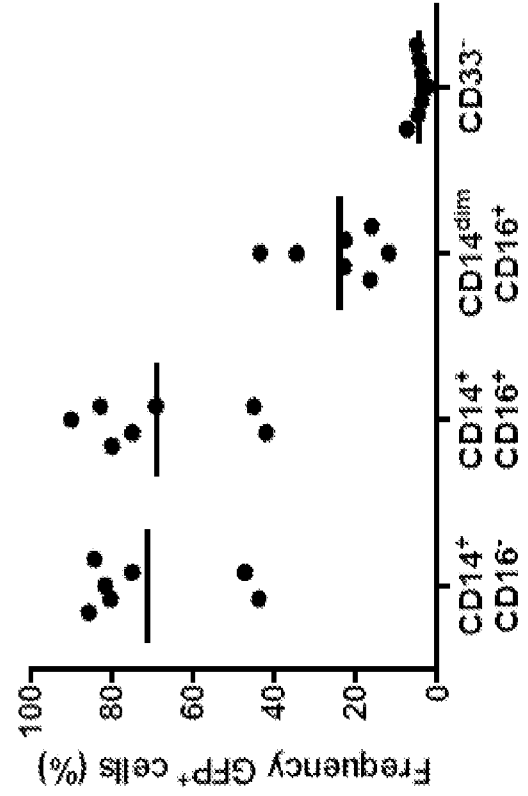
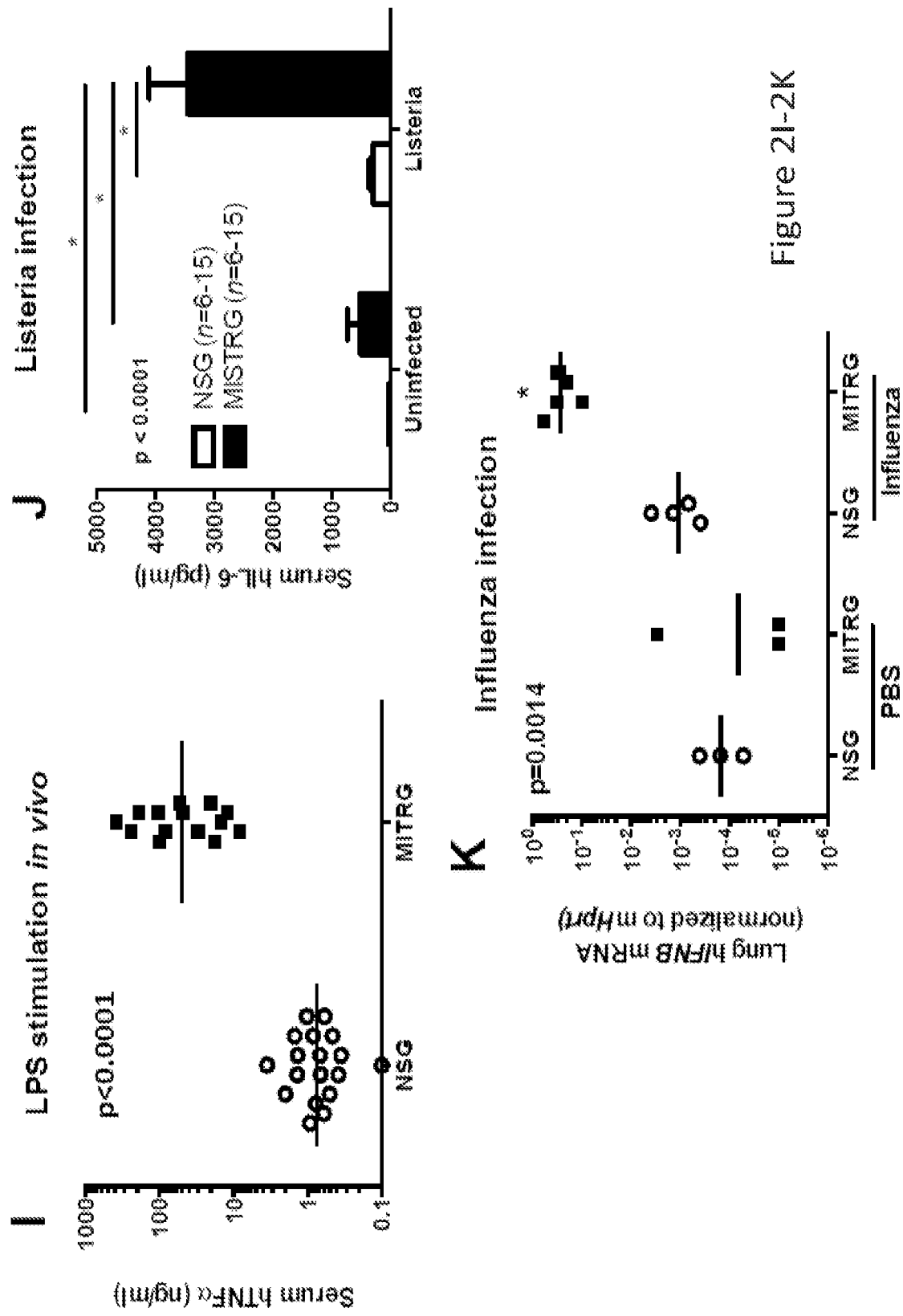


Figure 2F-2H



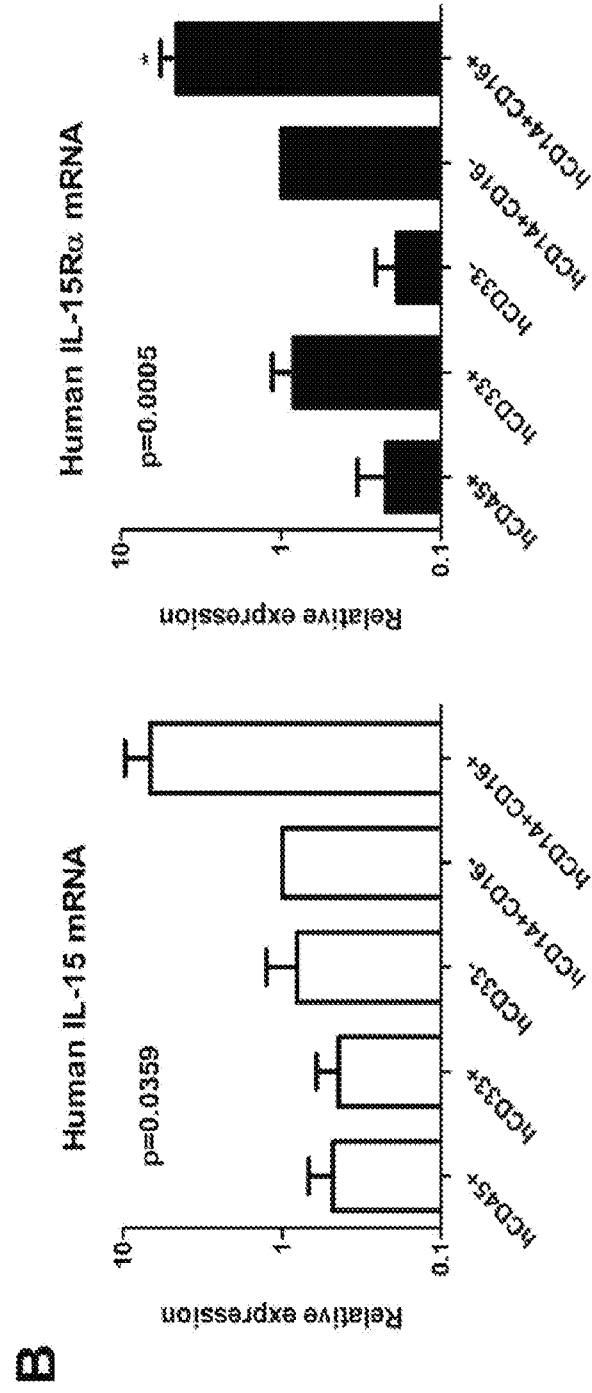
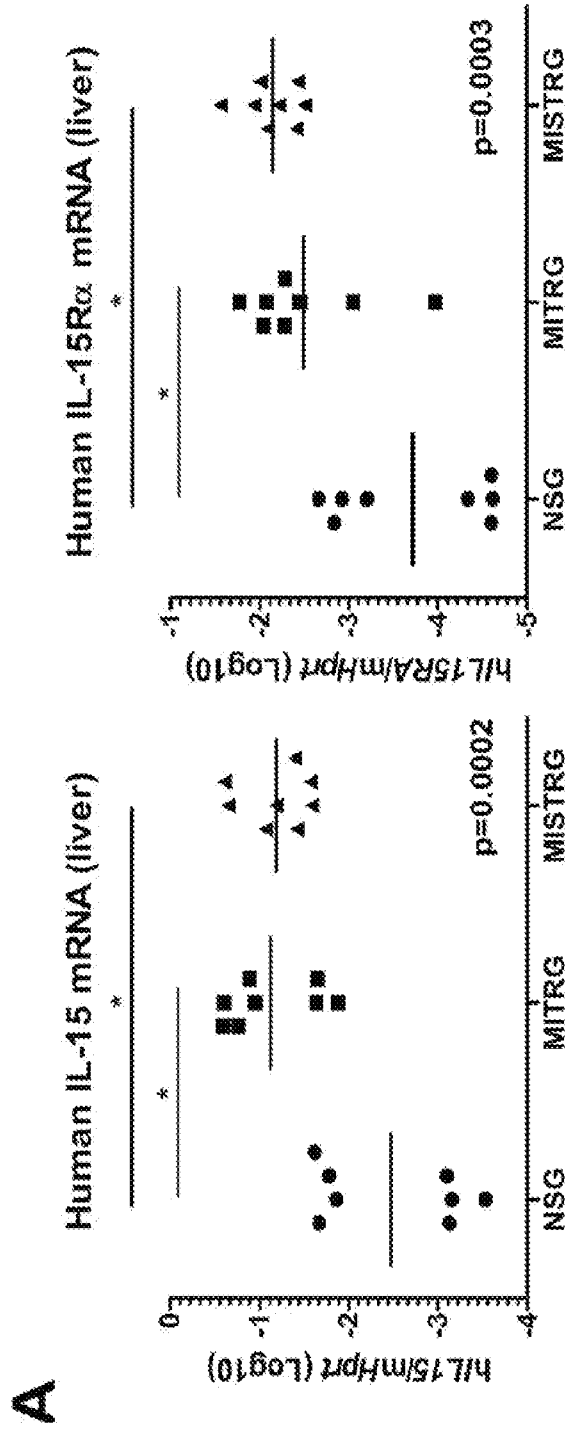


Figure 3A-3B

C

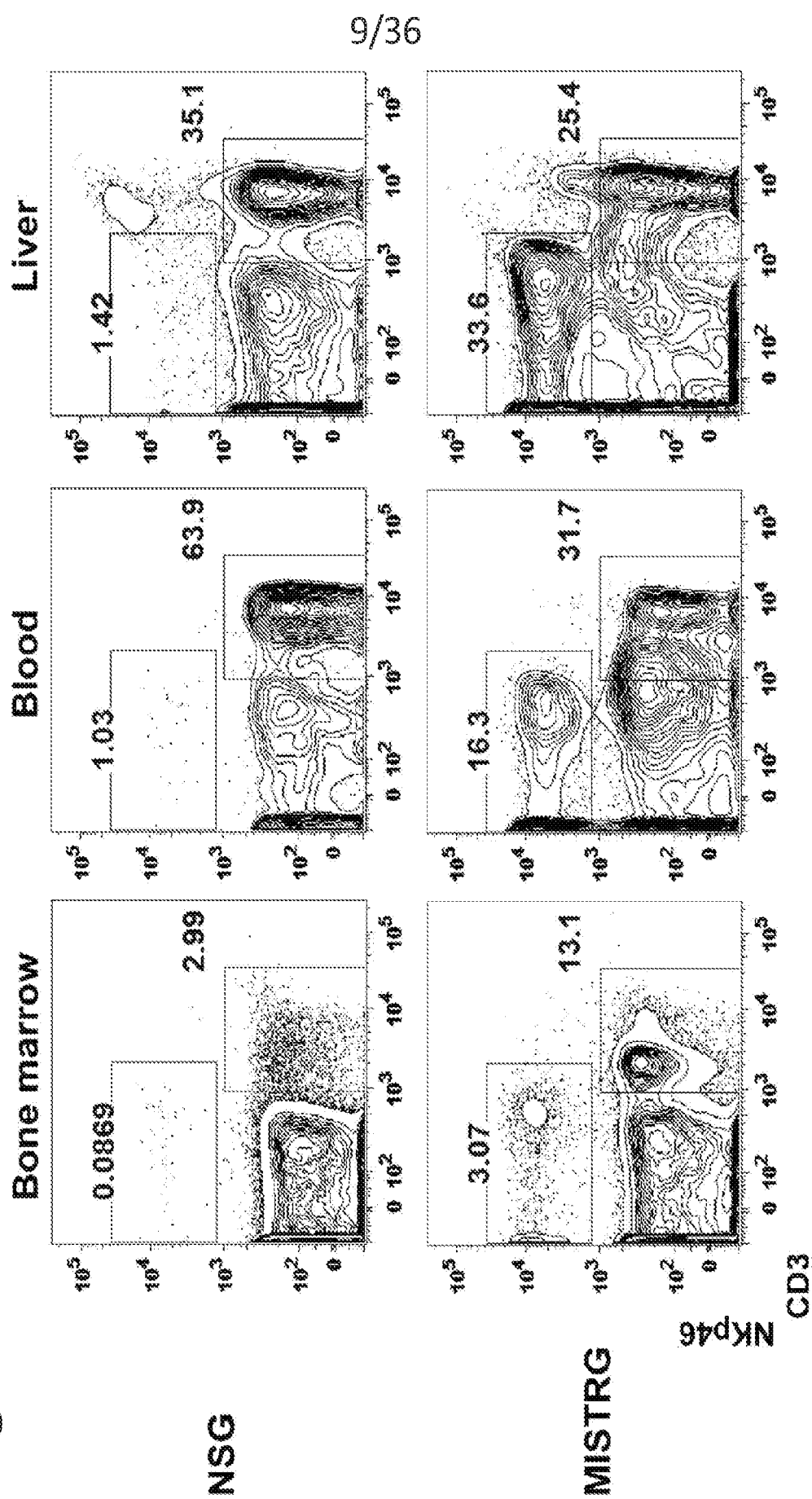
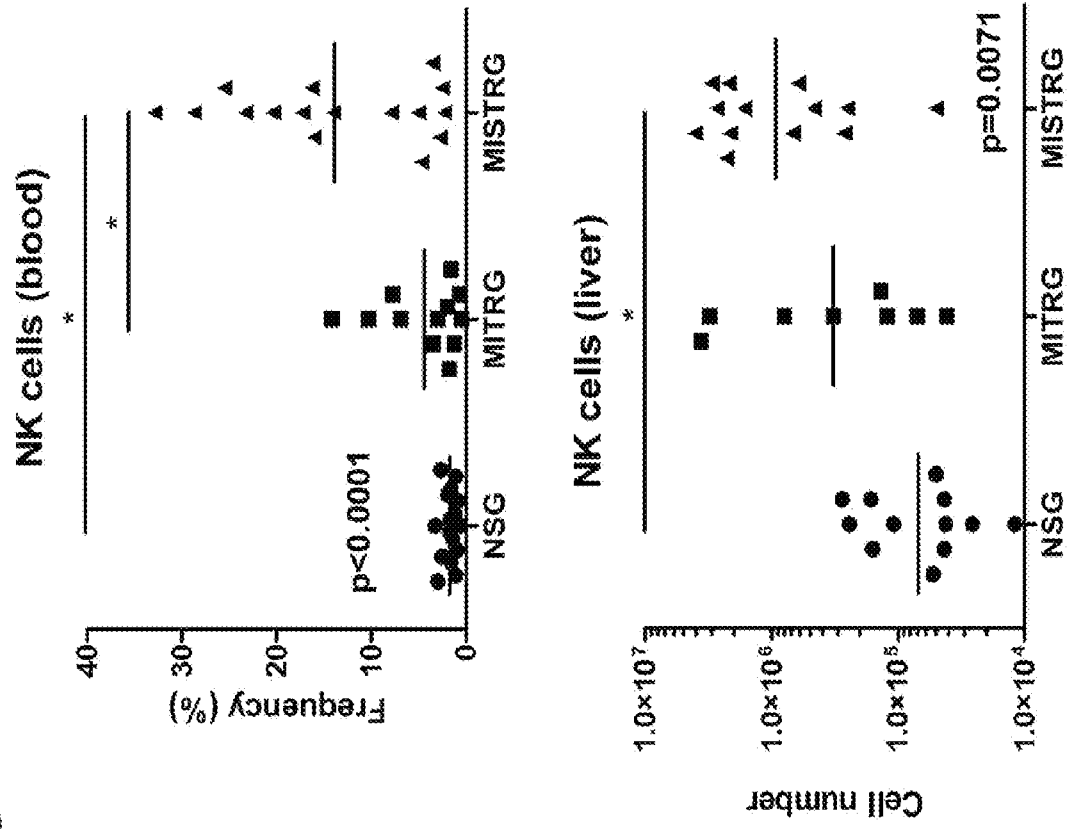


Figure 3C

D



E

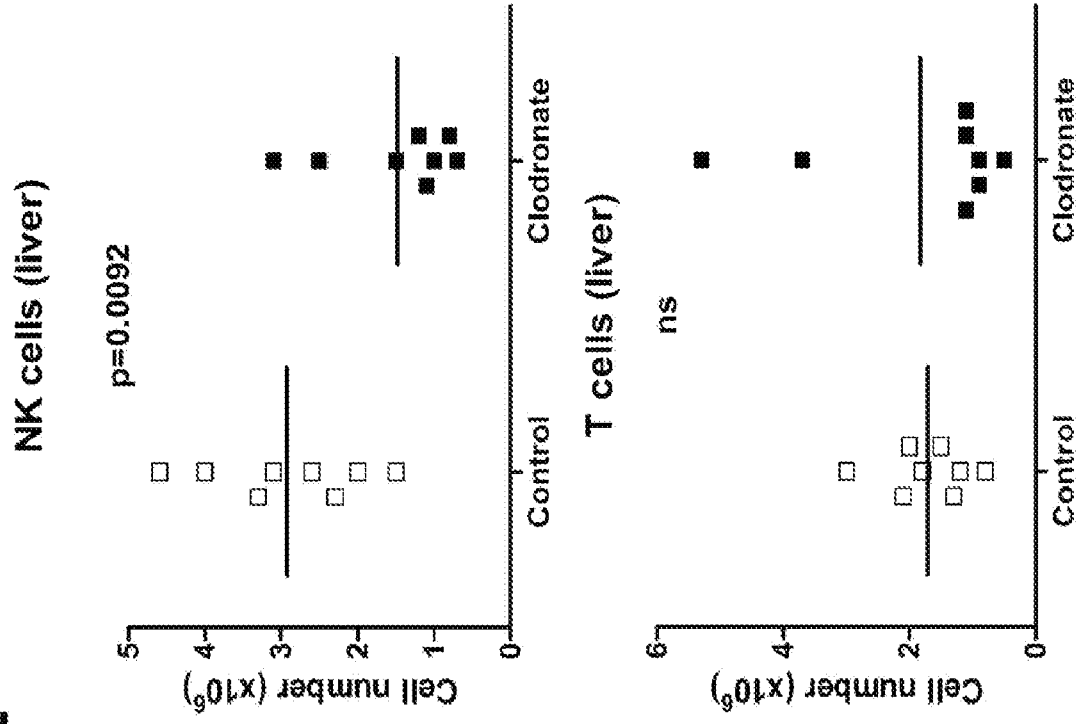


Figure 3D-3E

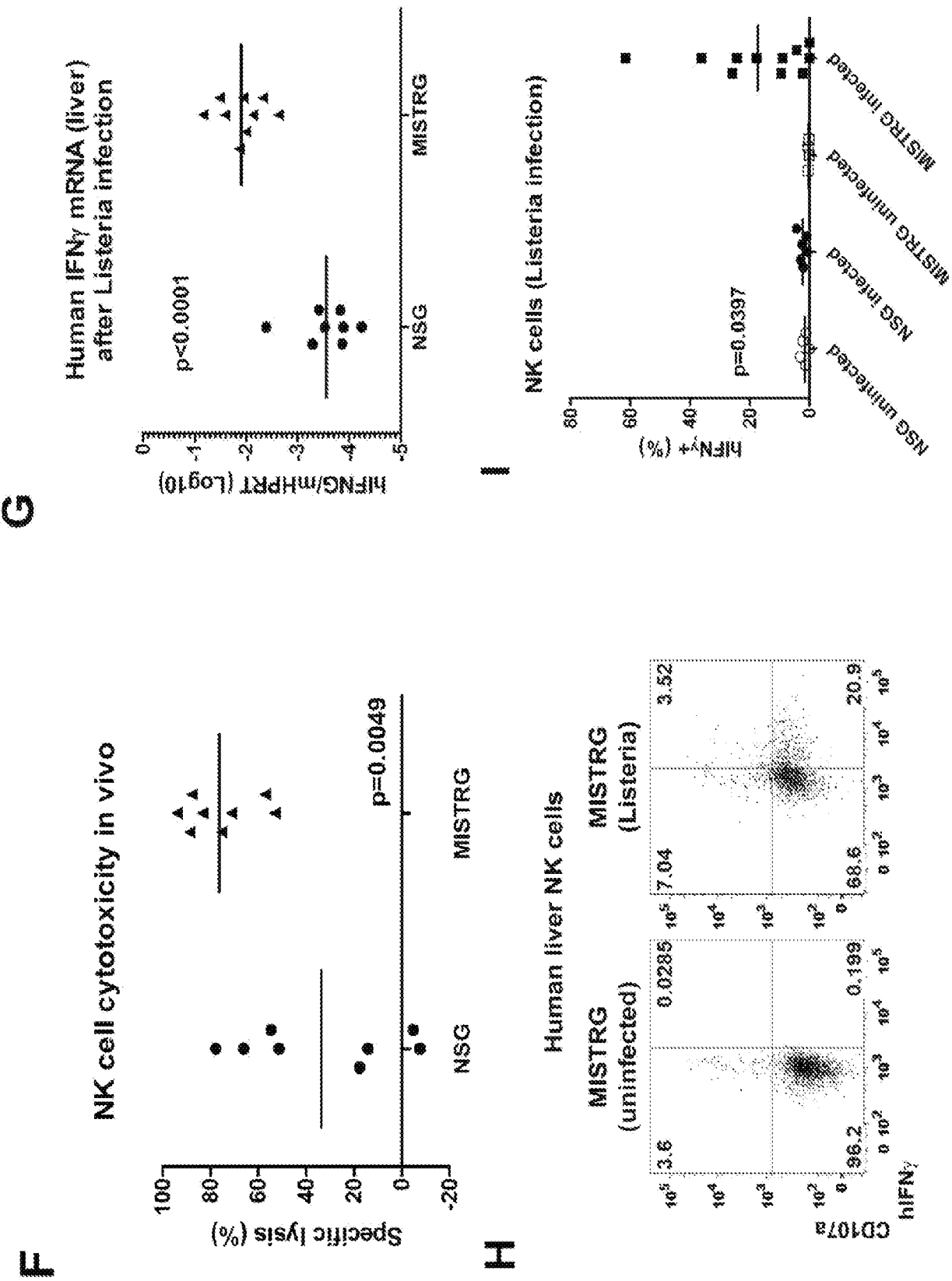


Figure 3F-3I

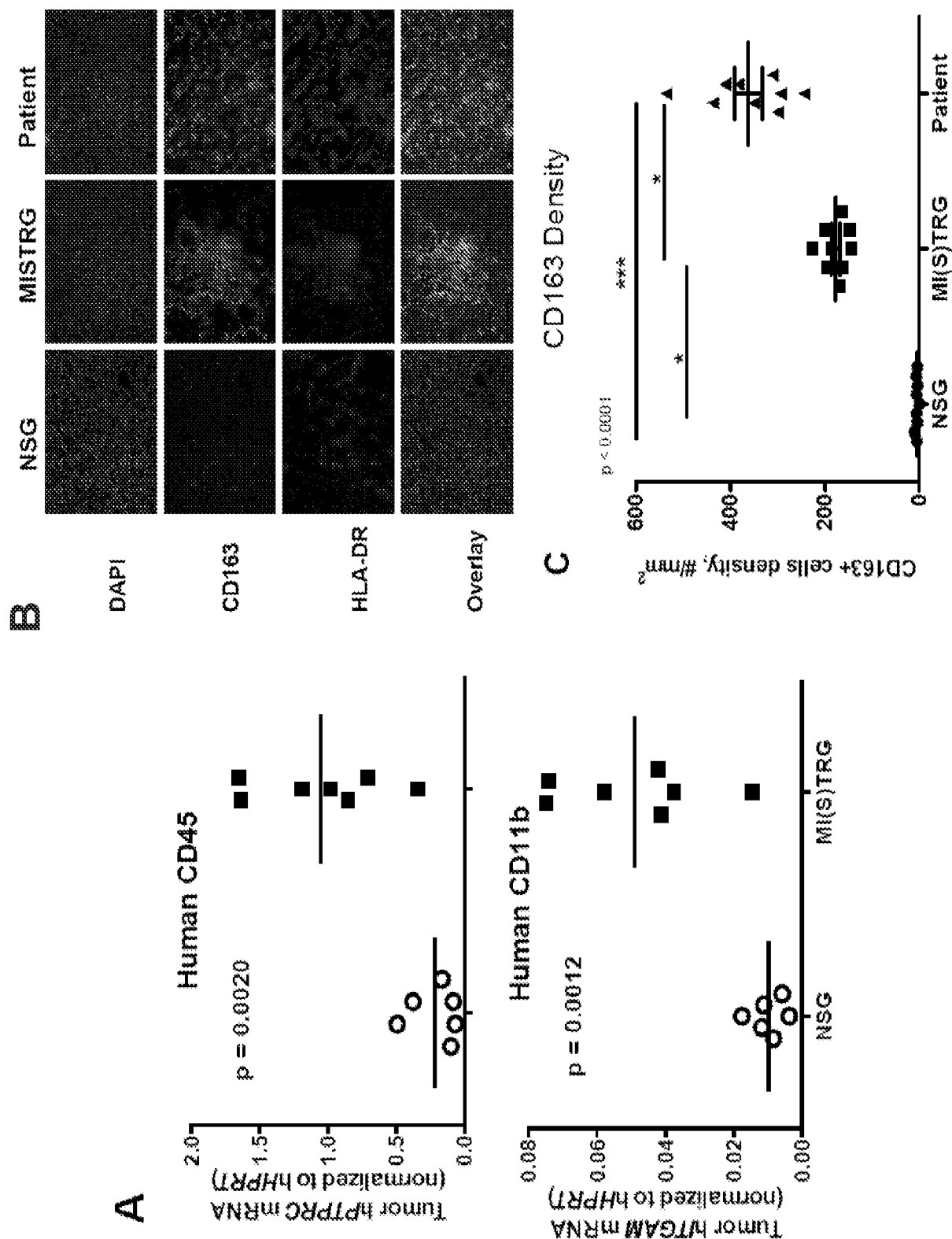


Figure 4A-4C

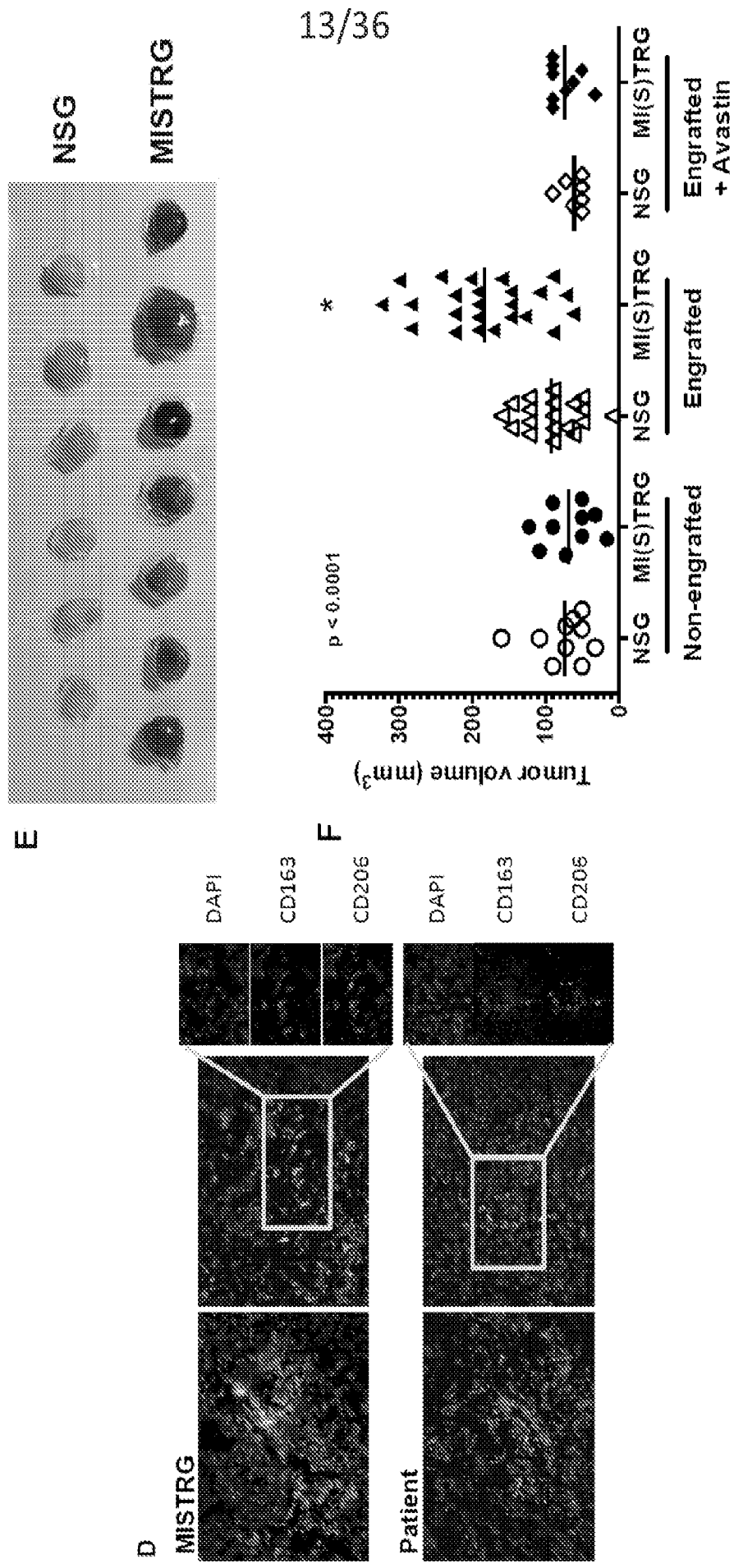


Figure 4D-4F

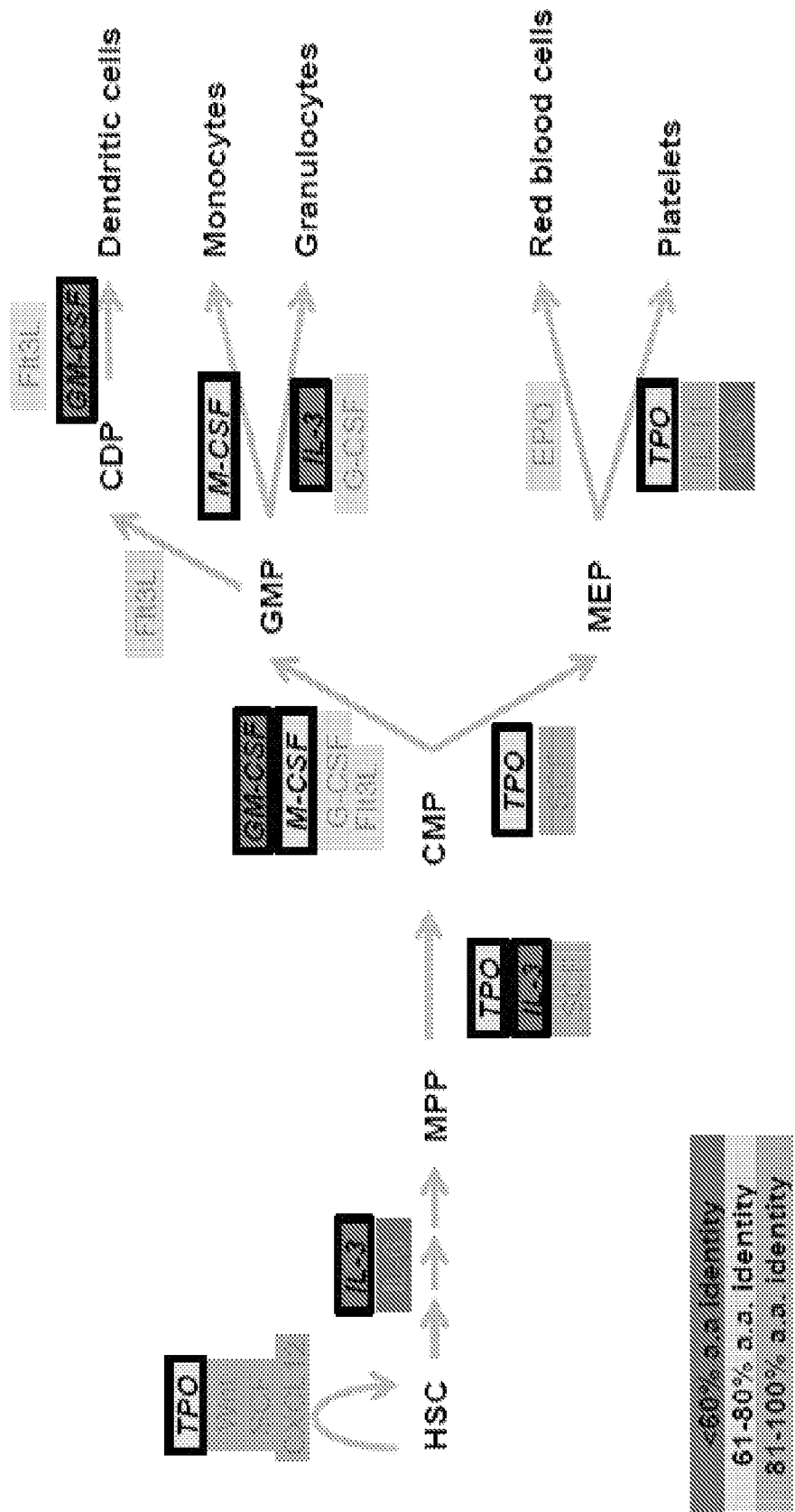


Figure 5

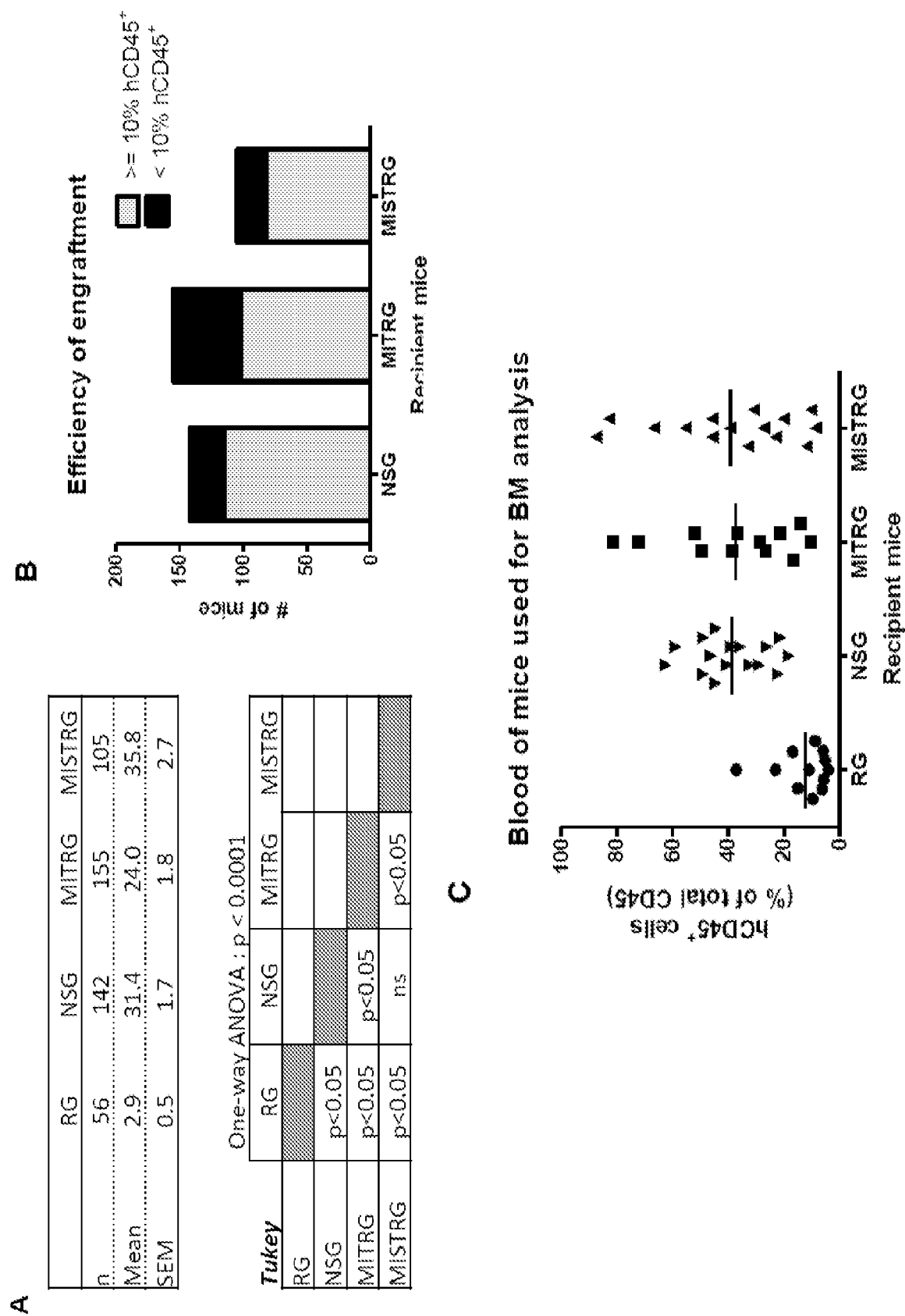


Figure 6A-6C

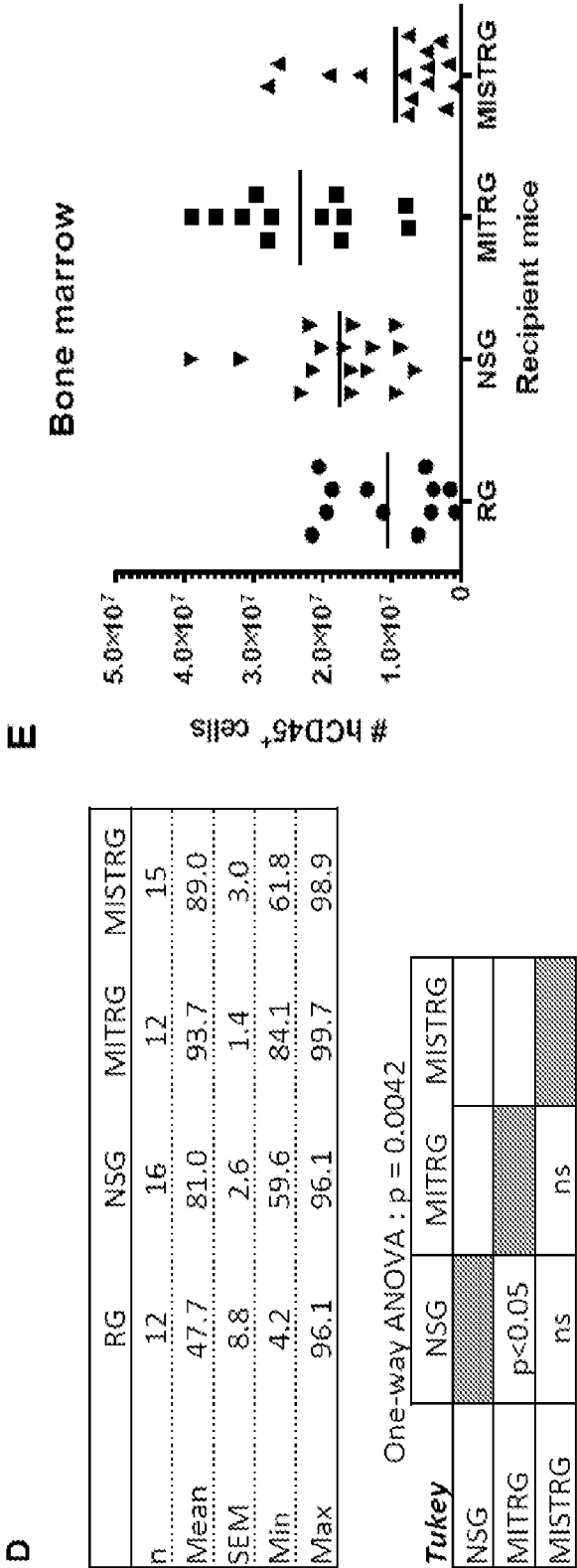
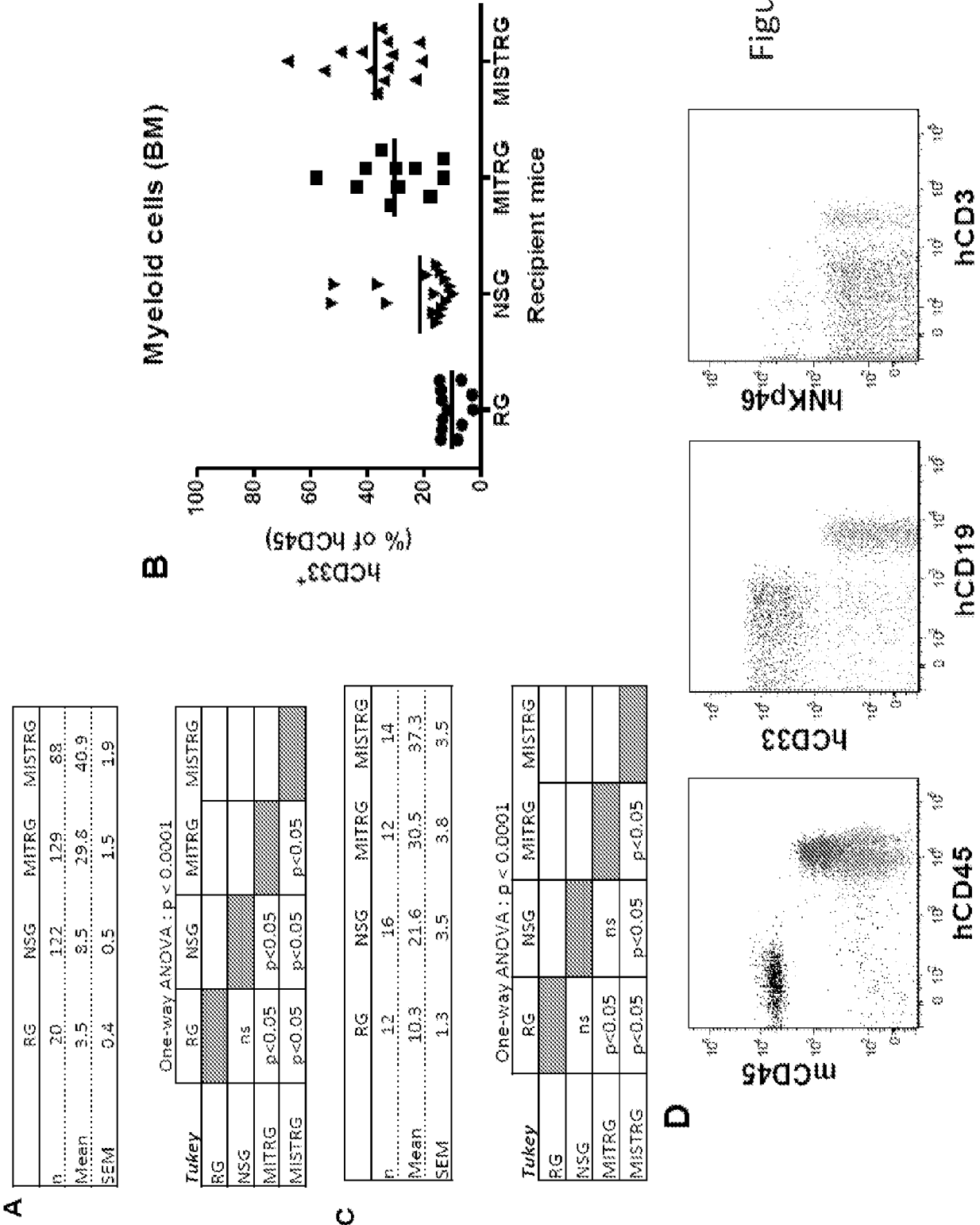


Figure 6D-6E



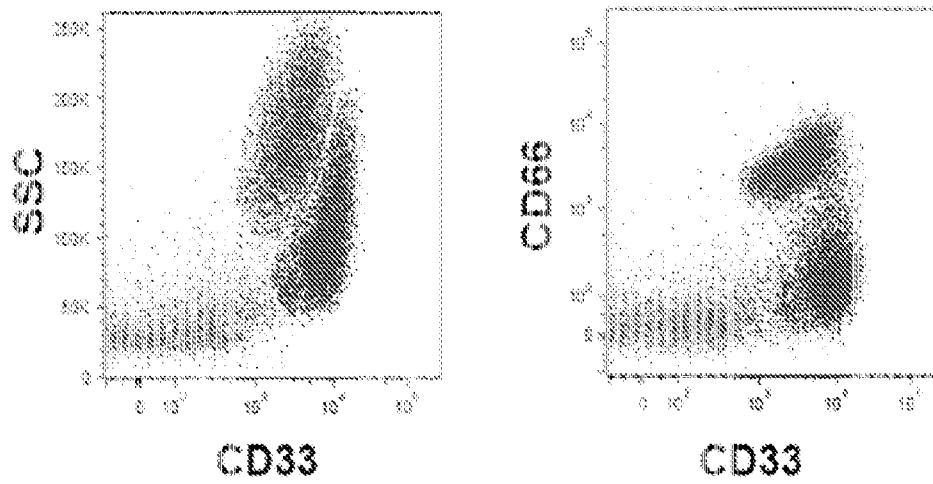
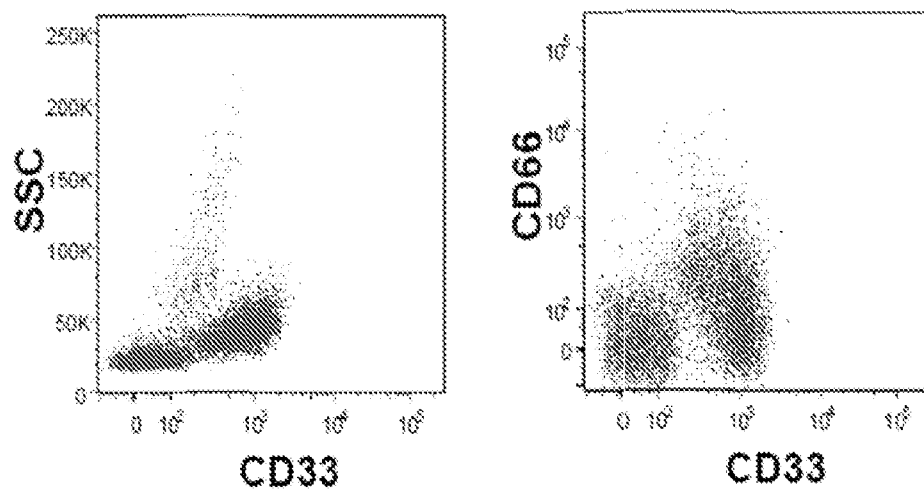
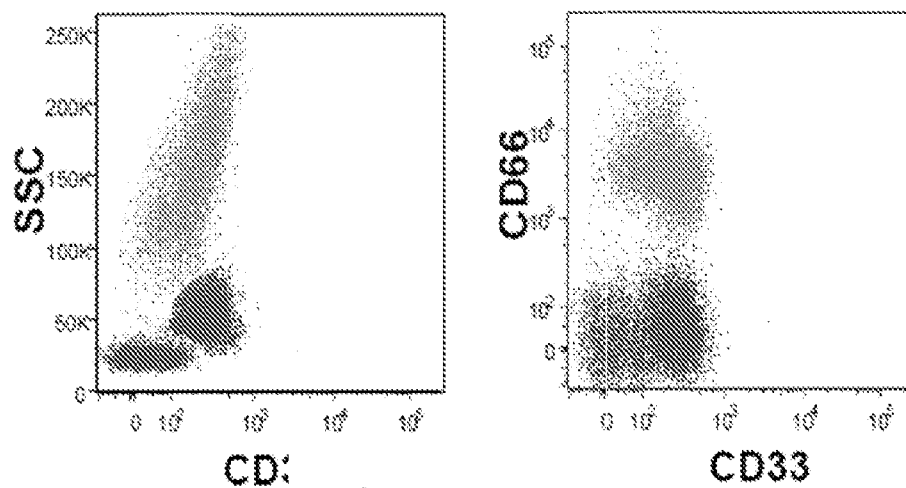
E**MISTRG BM (gated on hCD45⁺)****F****MISTRG blood (gated on hCD45⁺)****Human blood**

Figure 7E-7F

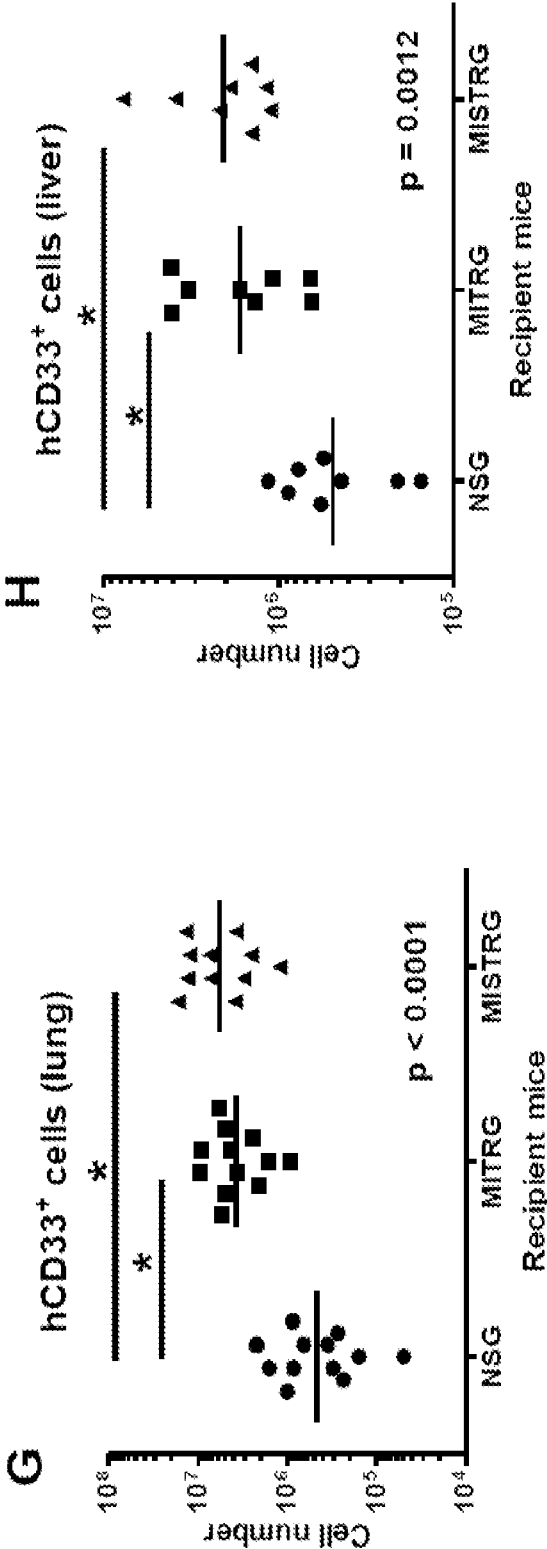


Figure 7G-7H

A

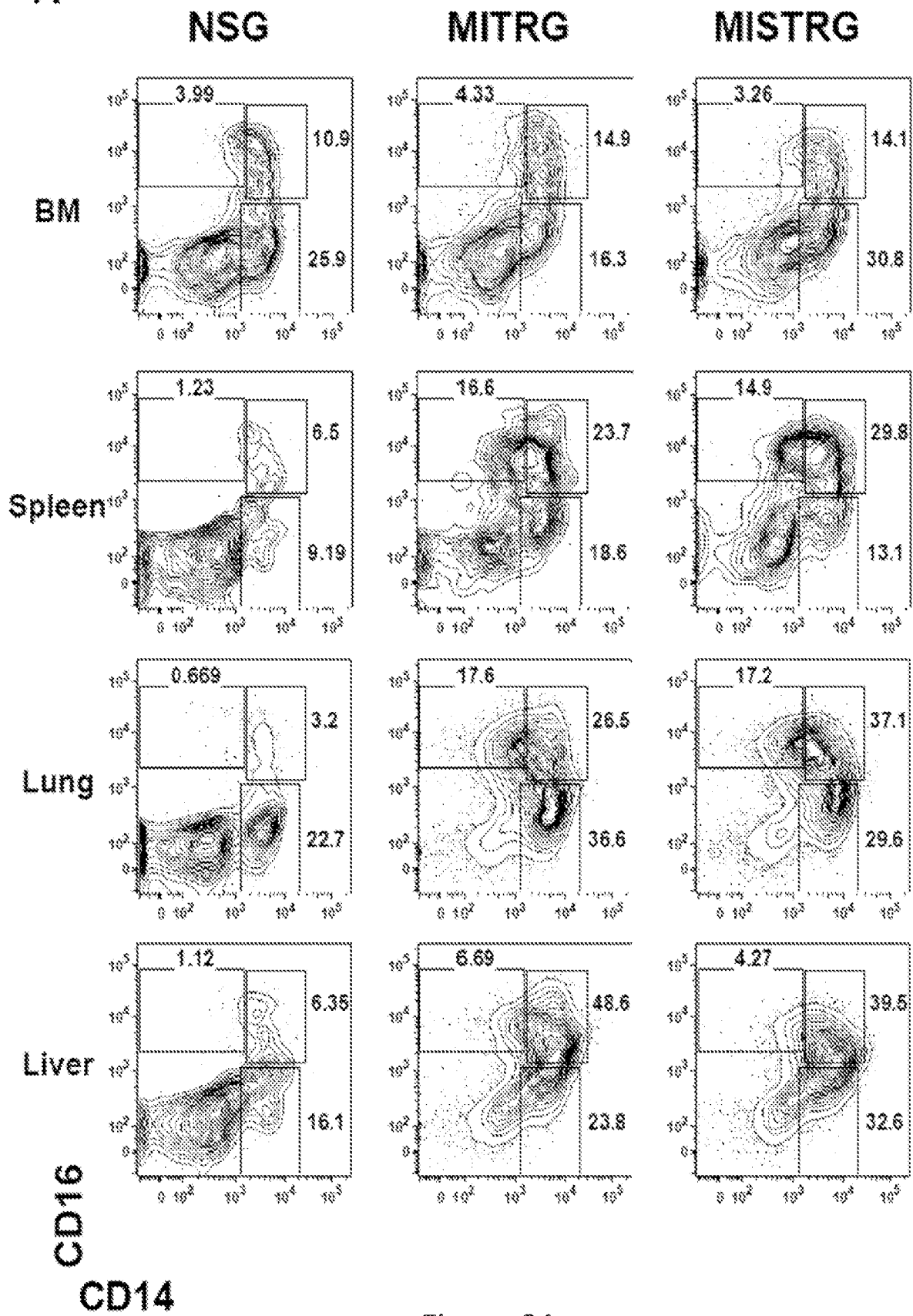


Figure 8A

B

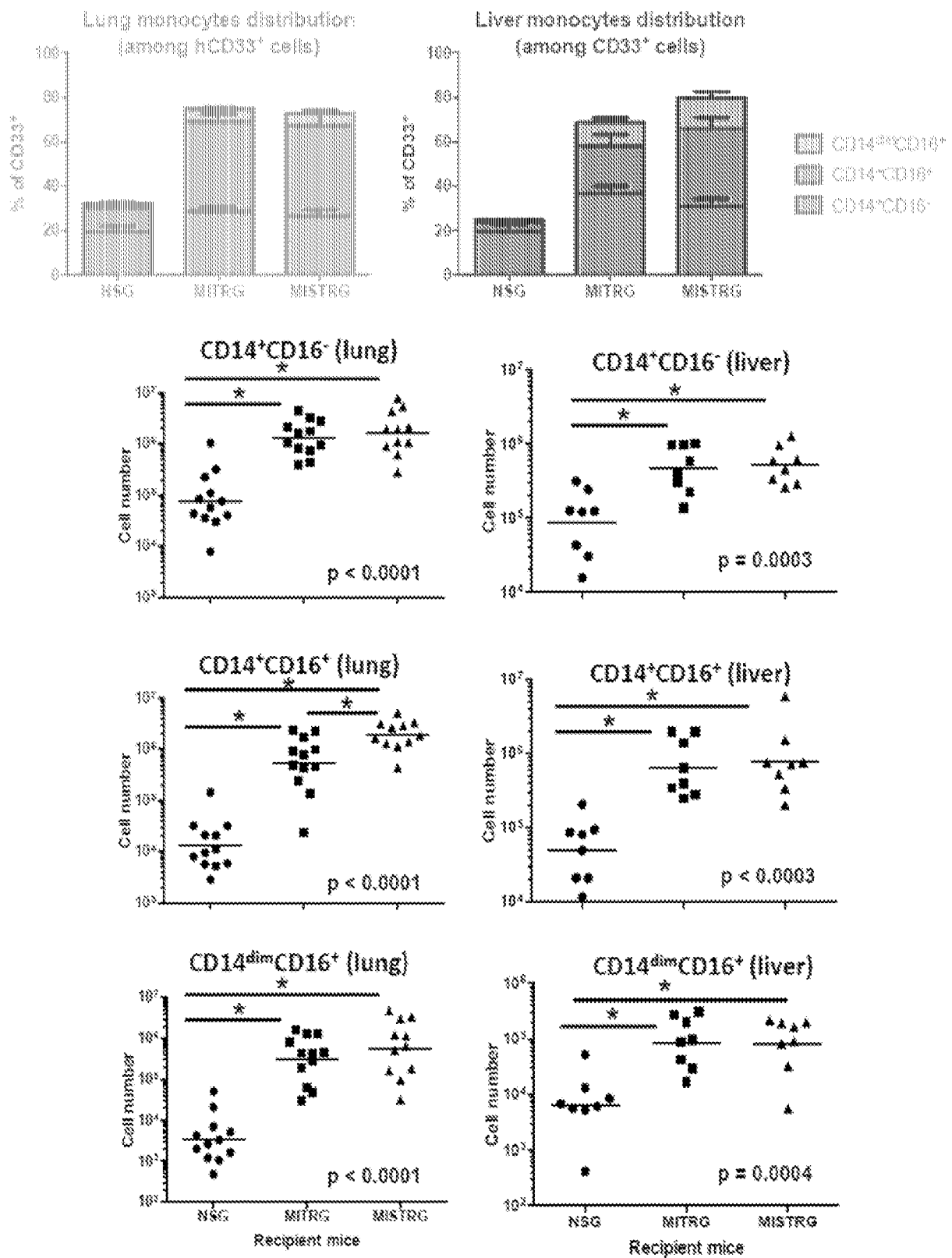


Figure 8B

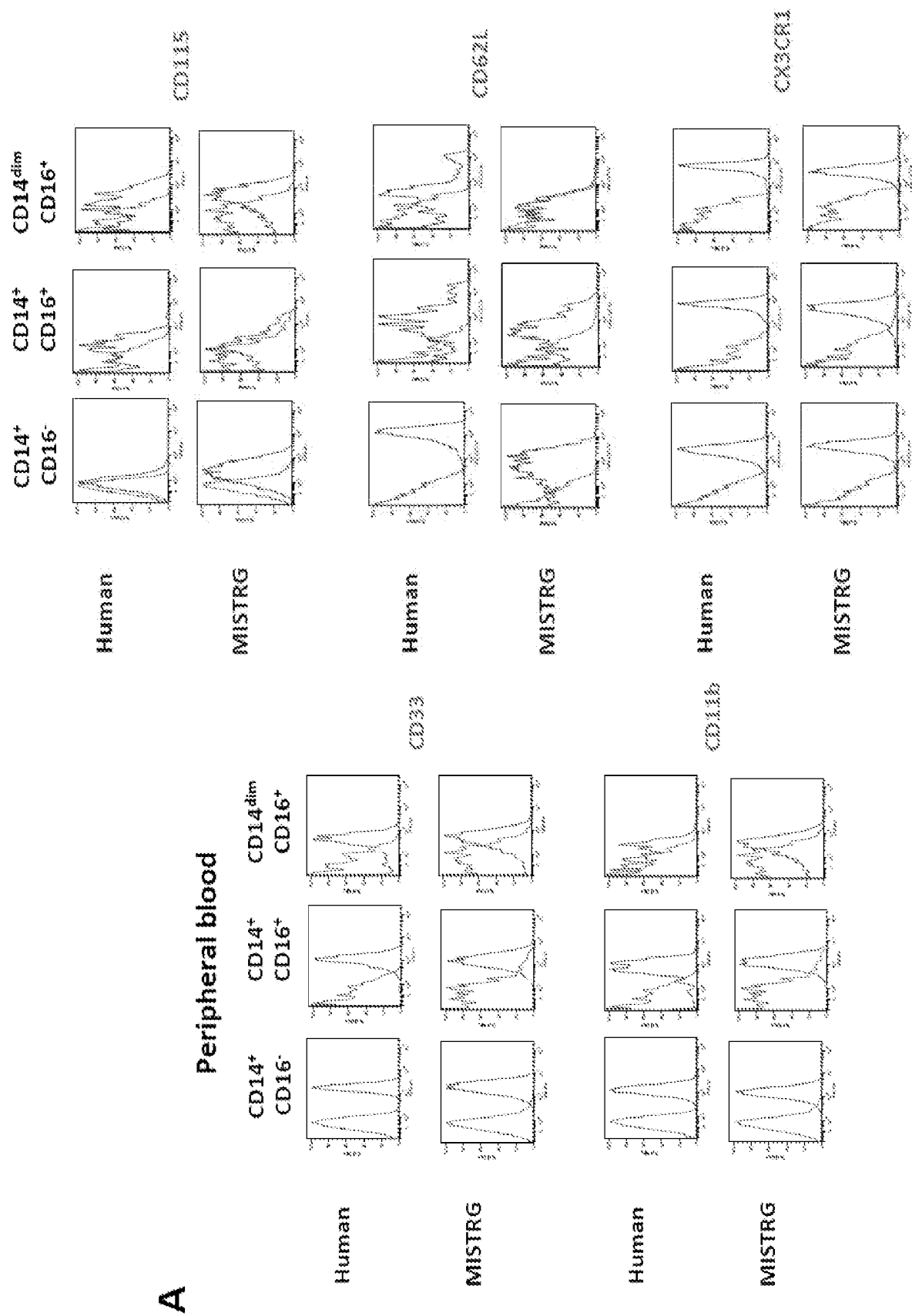


Figure 9A

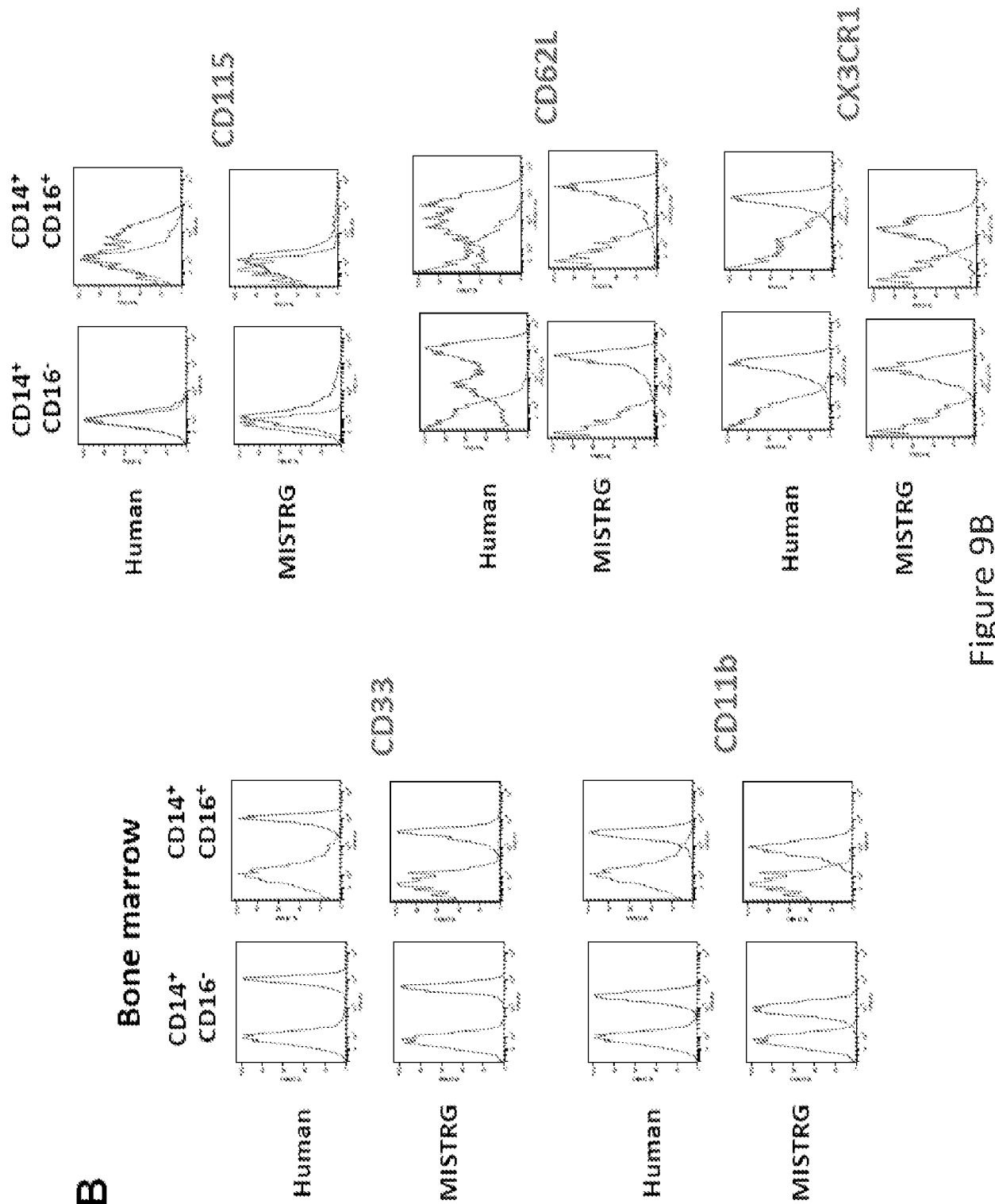


Figure 9B

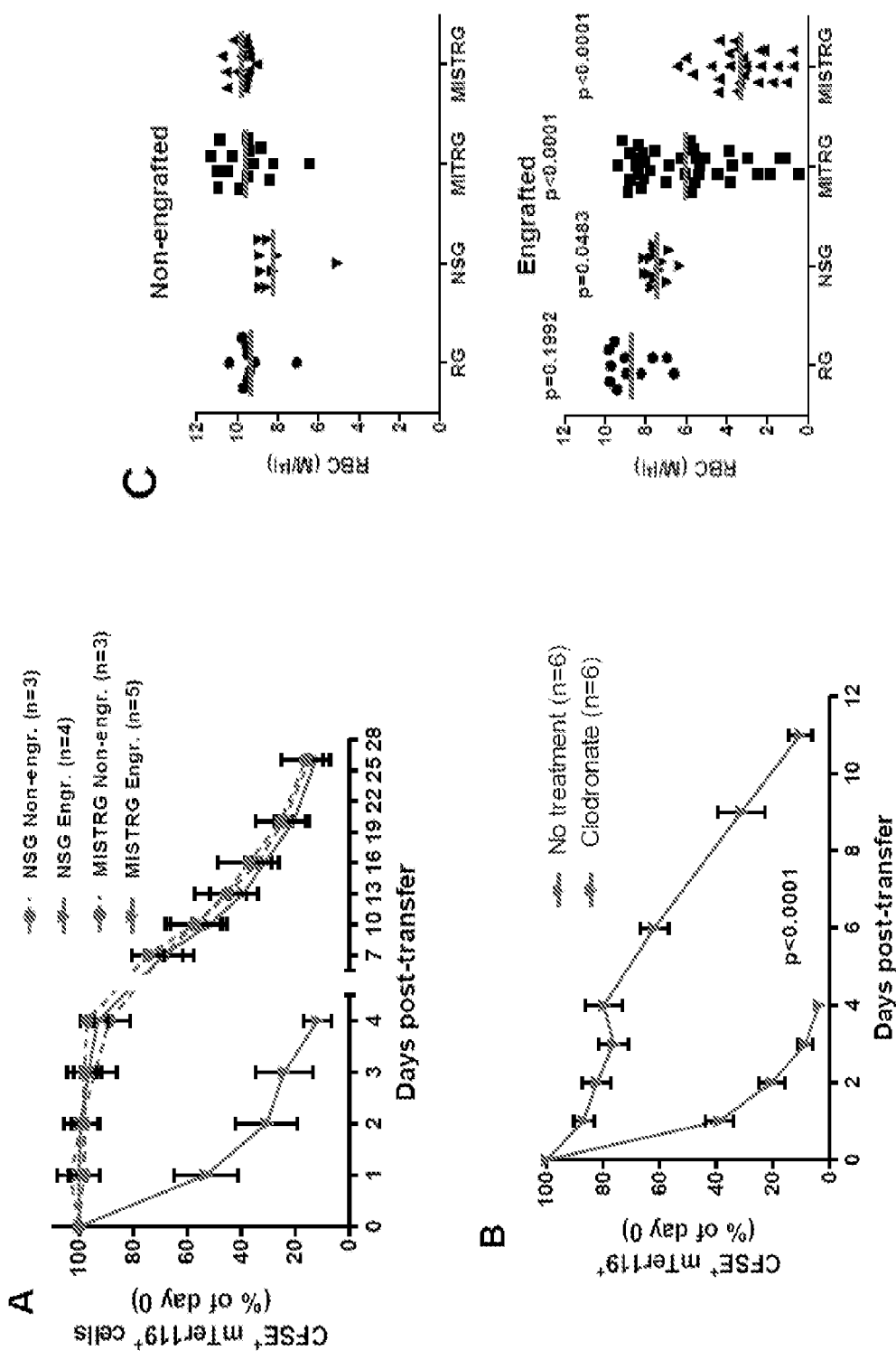


Figure 10A-10C

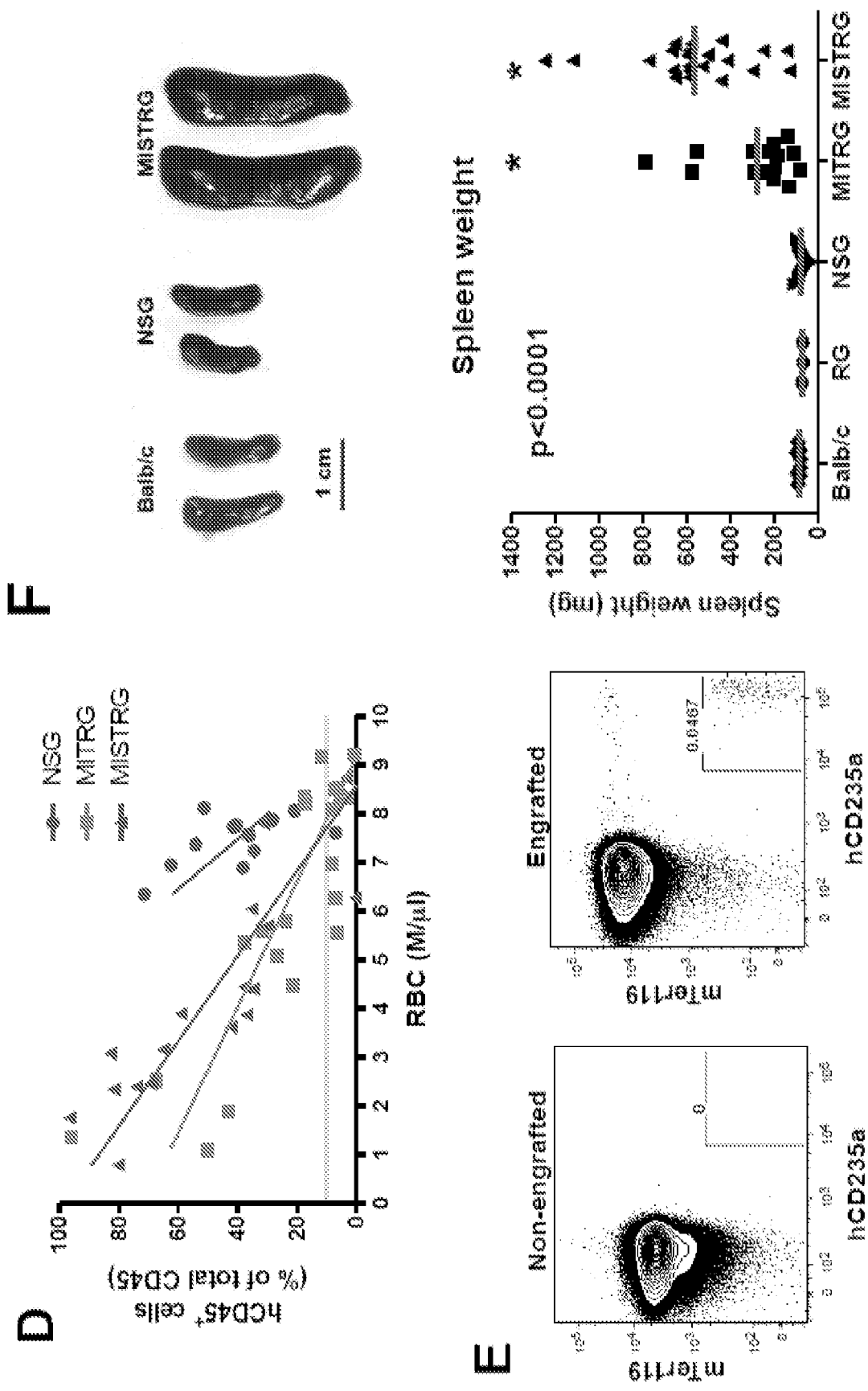


Figure 10D-10F

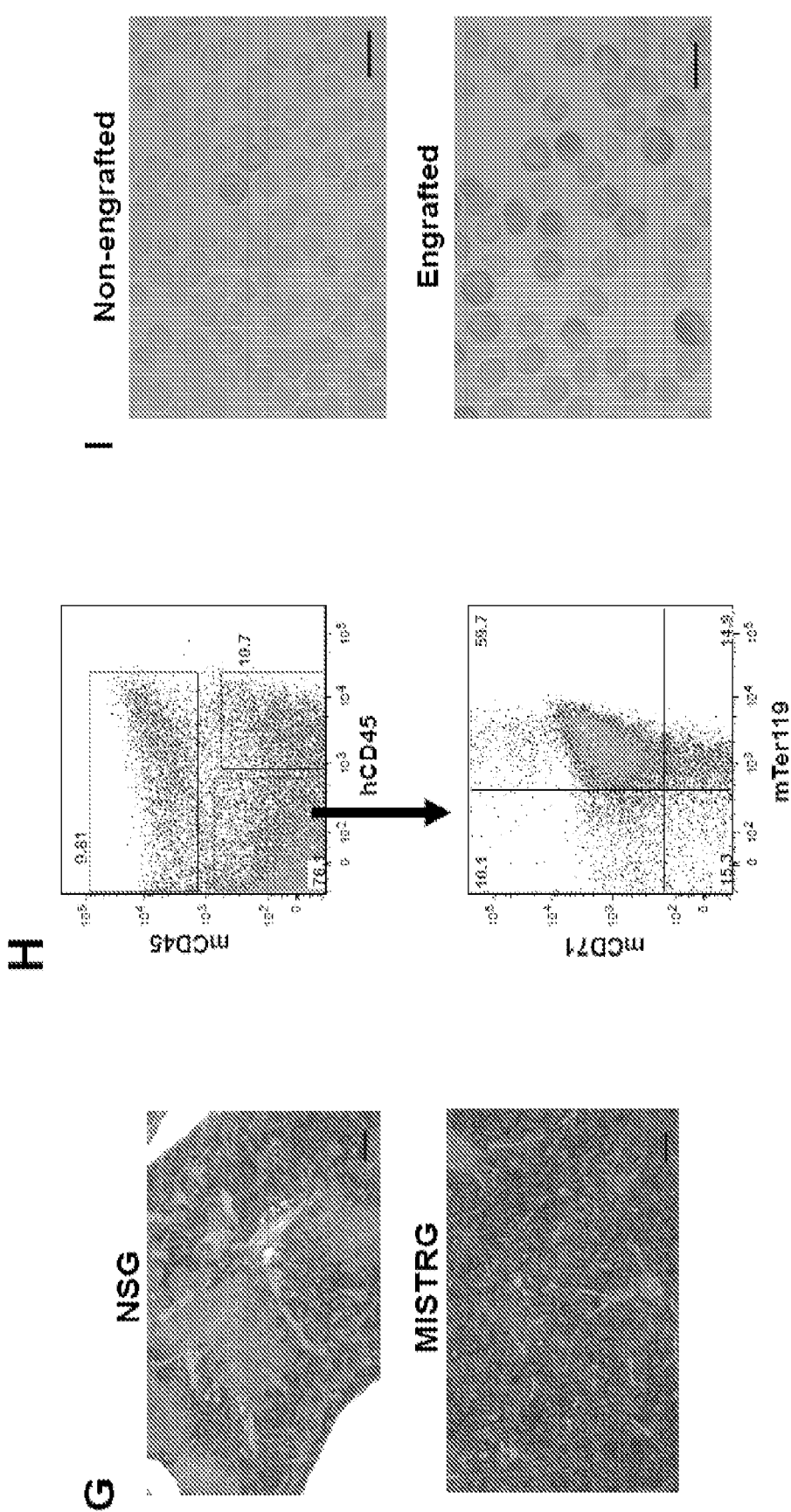


Figure 10G-10I

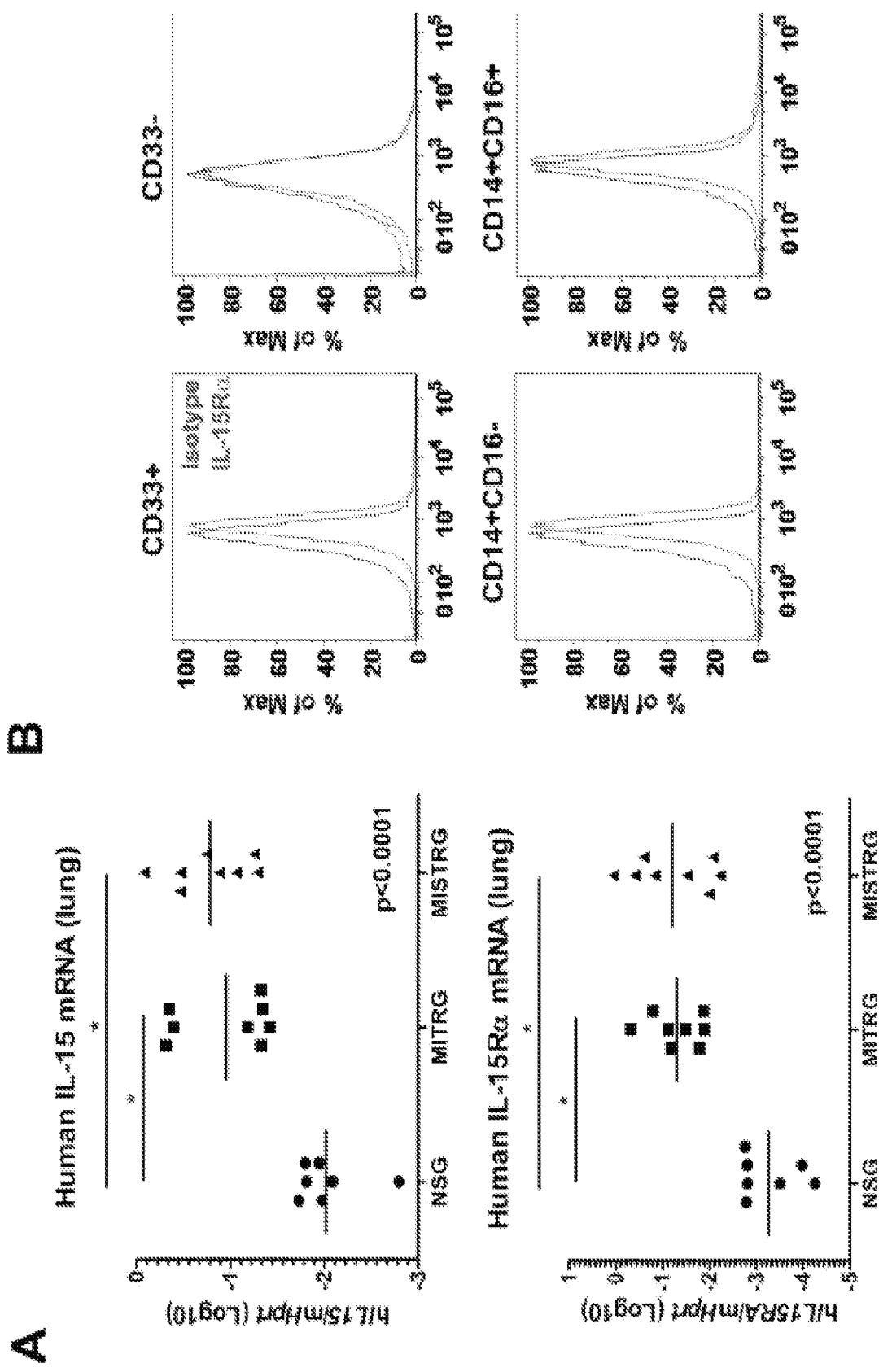


Figure 11

A

NK cells

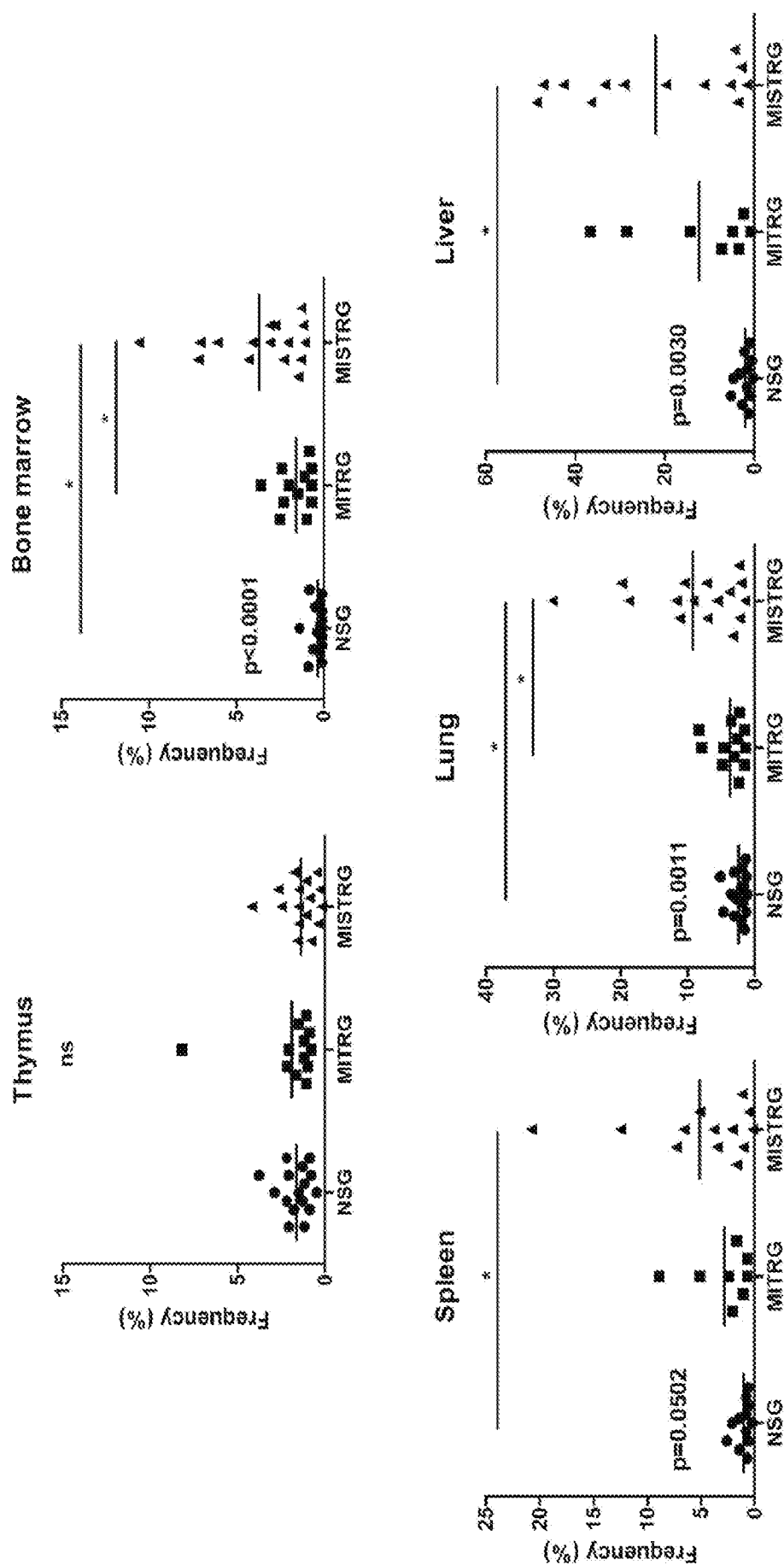


Figure 12A

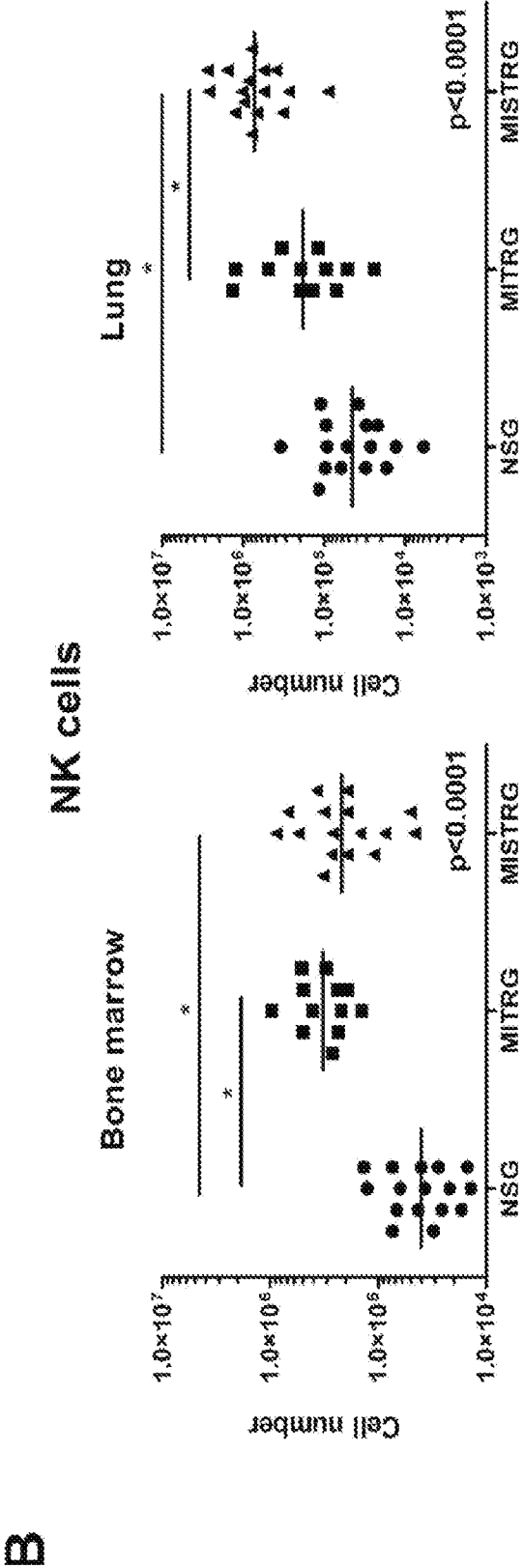


Figure 12B

A

30/36

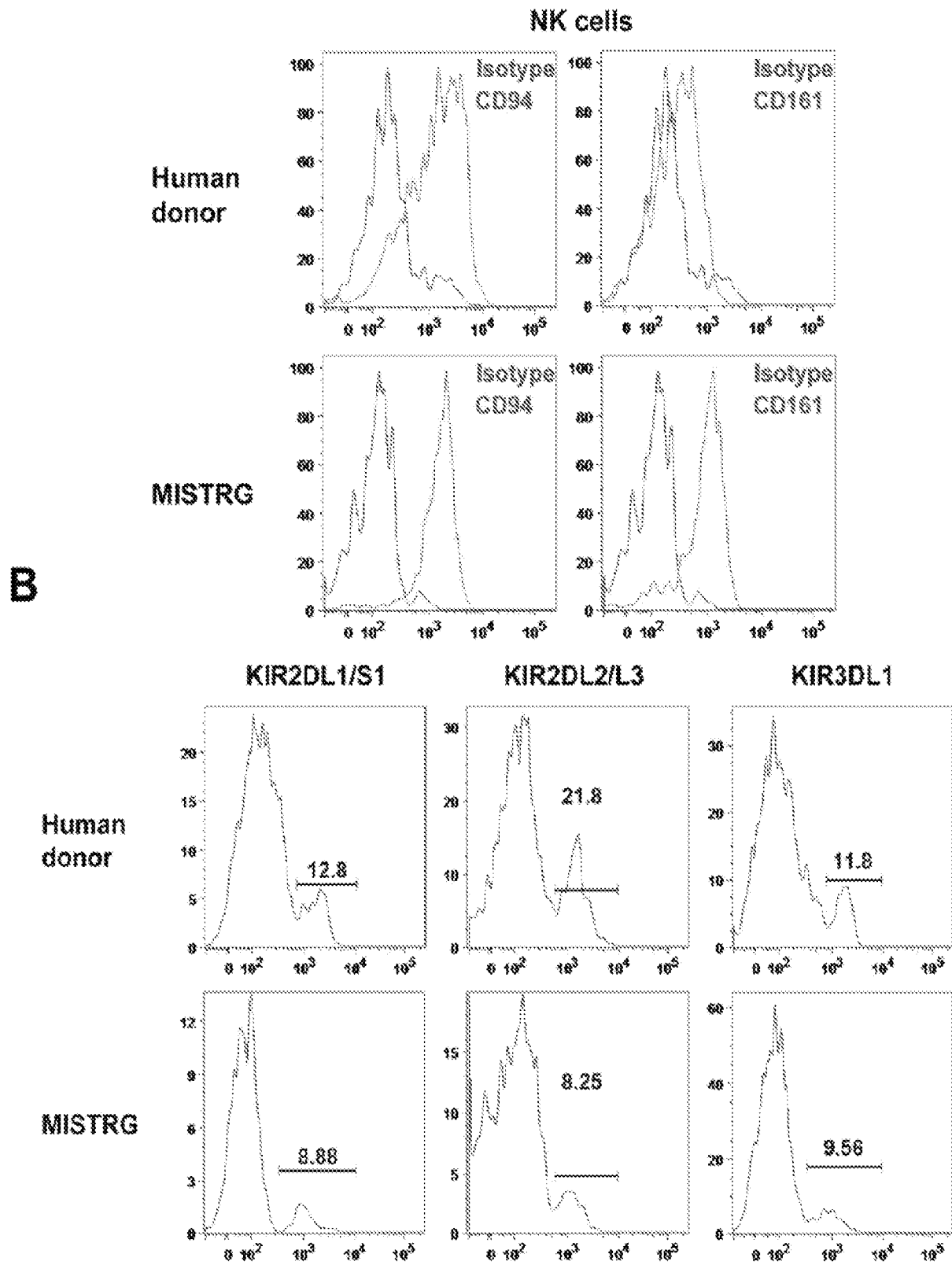


Figure 13A-13B

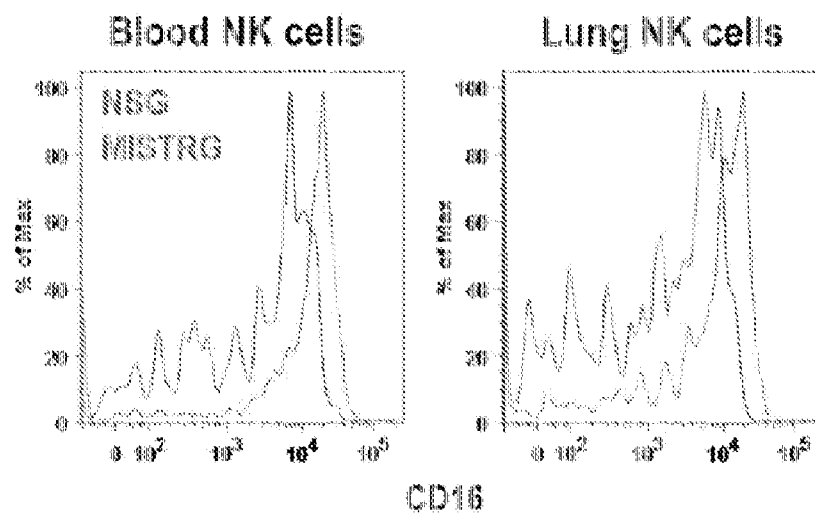
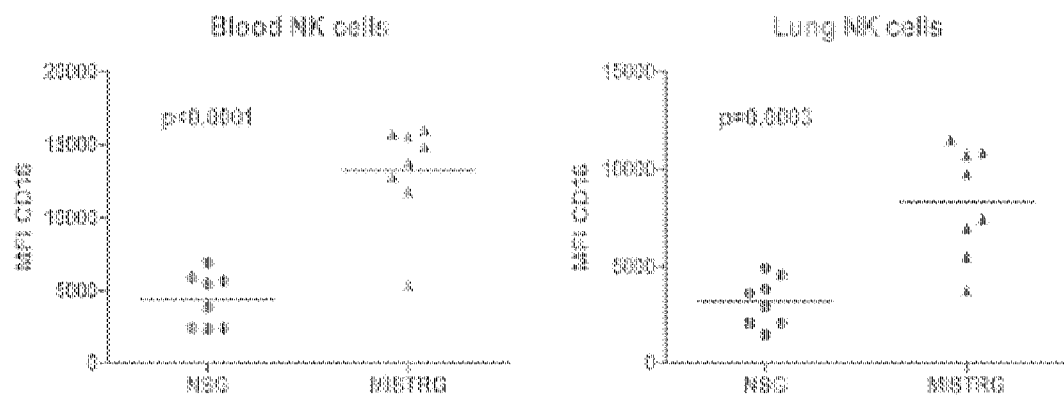
C**D**

Figure 13C-13D

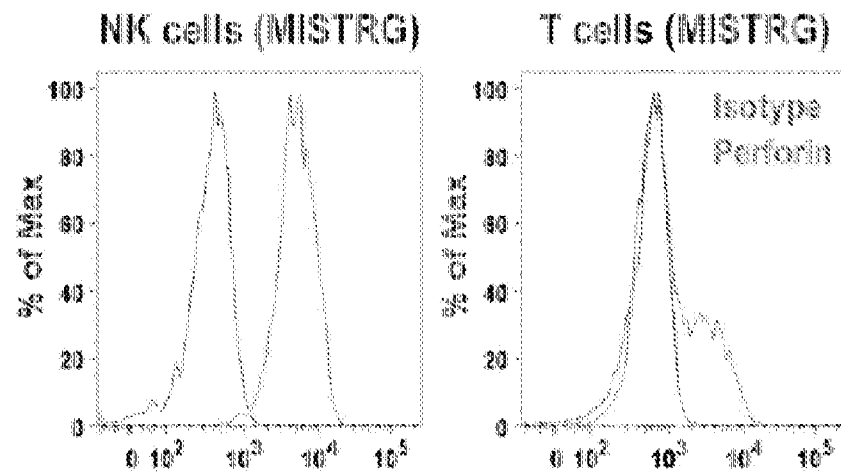
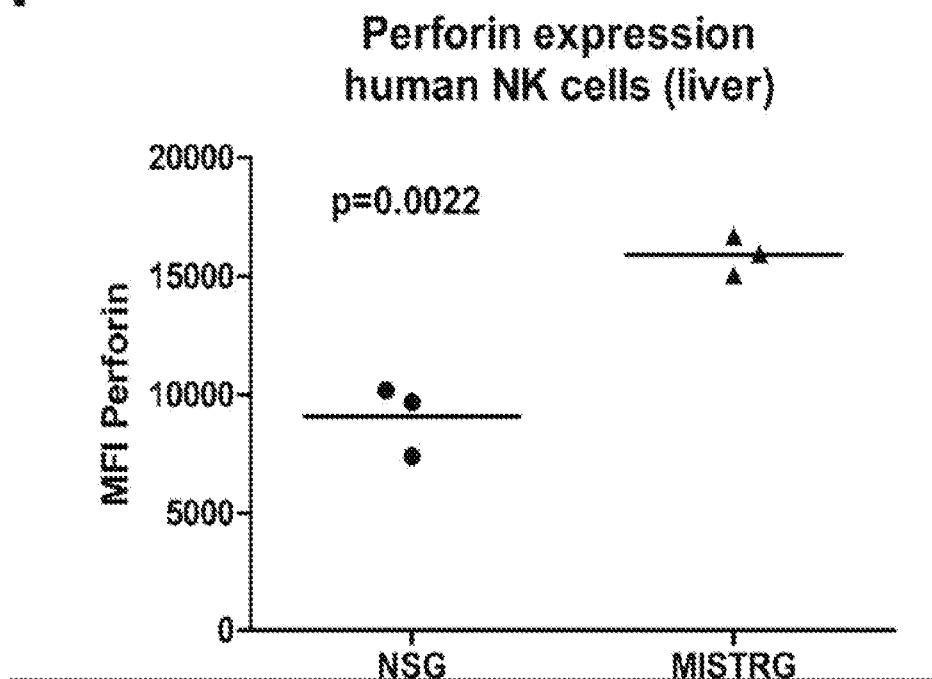
E**F**

Figure 13E-13F

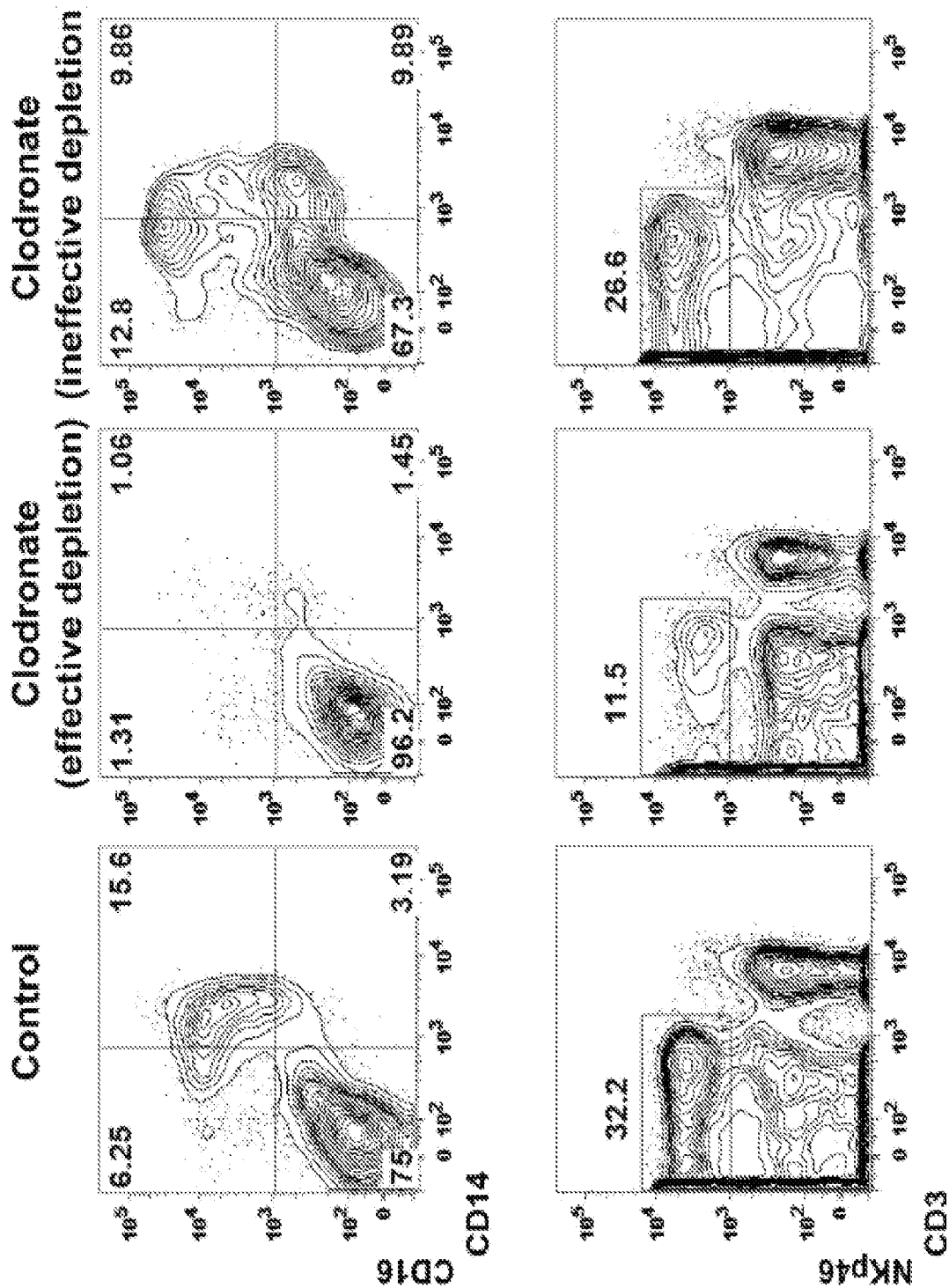


Figure 14

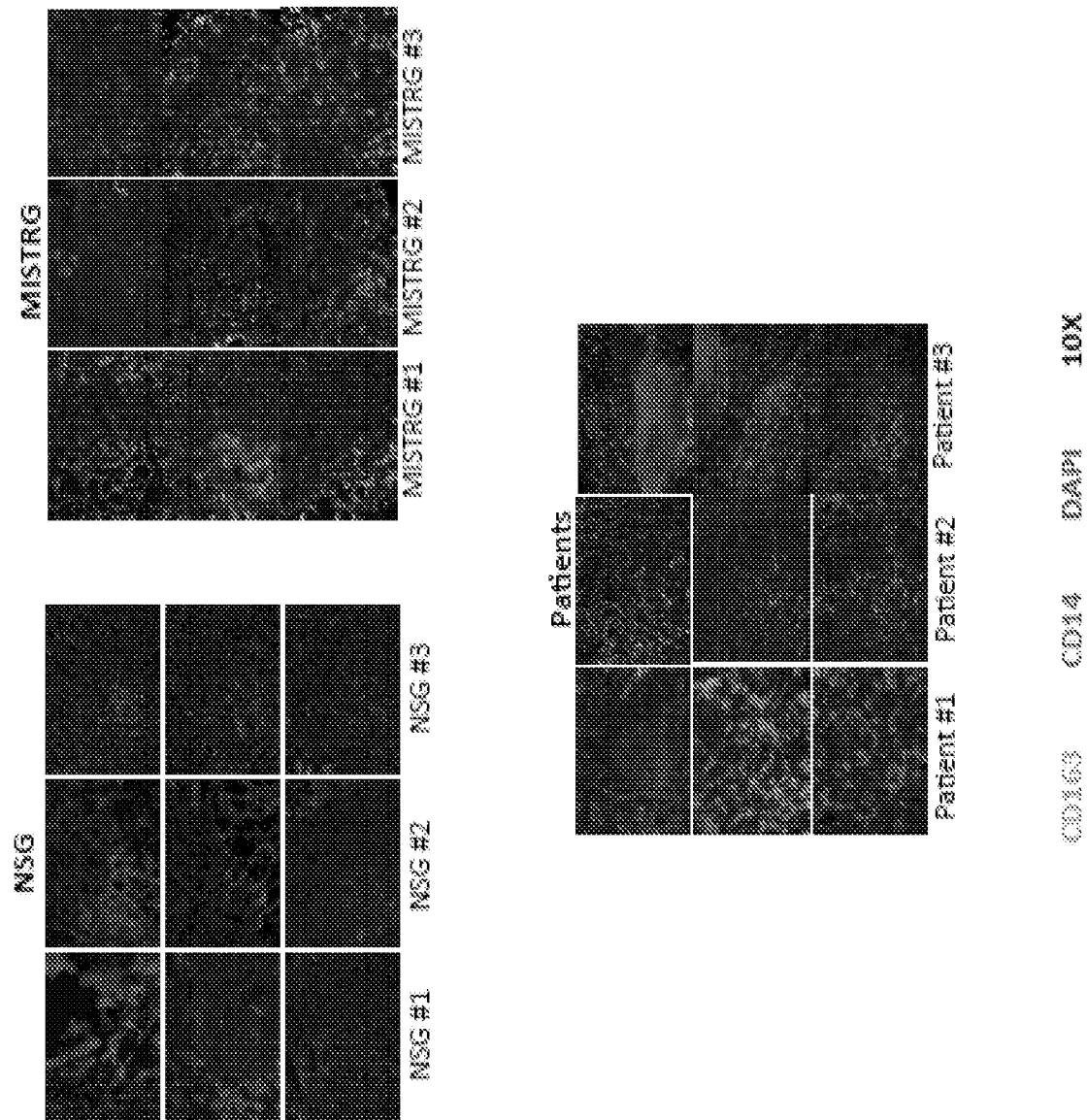


Figure 15

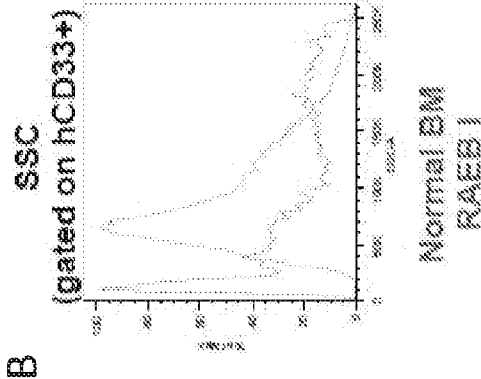
Comparison of engraftment levels and immune cell development and function in recipient mice with single gene replacement, in NSG, MISTRG and in humans							
	RAG2 ^{+/+} IL2Rγ ^{+/+} {RG}	RG TPO ^{h/h}	RG IL-3/GM-CSF ^{h/h}	RG M-CSF ^{h/h}	hSIRPα ^{h/h} RG / NSG	MISTRG	Human
Blood hCD45 ⁺ cells (%)	5-20	5-20	5-20	20-40	10-60	10-80	100
BM hCD45 ⁺ (%)	20-80	75-90	20-80	20-80	60-90	85-99	100
Human CD33 ⁺ myeloid cells (% of hCD45)	1-7	10-20	5-10	10-30	5-15	30-50	50-70
Human NK cells (% of hCD45)	0-1	ND	ND	ND	0.5-2	1-7	1-6
Human NK cell function	-	ND	ND	ND	-	+	+
Serum TNFα (ng/ml, 90 min post LPS)	ND	ND	ND	ND	0.3-3	10-200	ND
Lung alveolar macrophages	-	-	+	-	-	+	+
ND: Not determined							

Figure 16

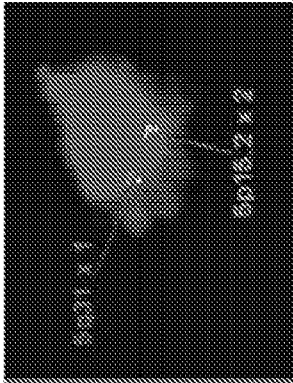
A

Disease	Cell number per mouse	Engrafted	Time after transplant in weeks	Bone Marrow		Genetic abnormality		Comment
				hCD45	hCD33	Patient sample	Engrafted	
RCUD	9x10e4 CD34+ enriched	1/1	11	1.9	89.9	Normal	n.d.	Low granularity of CD33+ cells
RAEB I	1-4x10e5 CD34+ enriched	4/4	10	2.2-38.7	37.3-49.8	Trisomy 8 in 5% of cells	nl FISH x 1	Low granularity of CD33+ cells
RAEB II	7x10e5 CD34+ enriched cells	2/2	15	0.07-0.88	31.9-85.5	5q-	5q- in 24/25 cells	
CMMML	7.5x10e6 T-cell depleted	1/2	15	0.8	48	del(6)(q11.1q22.1)	del(6)(q11.1q22.1)	
2° AML from RAEB II	4.5x10e6 T-cell depleted	2/2	12	73.8-81.5	77.5-84.1	Complex including 5q-	Complex including 5q-	

B



C



D

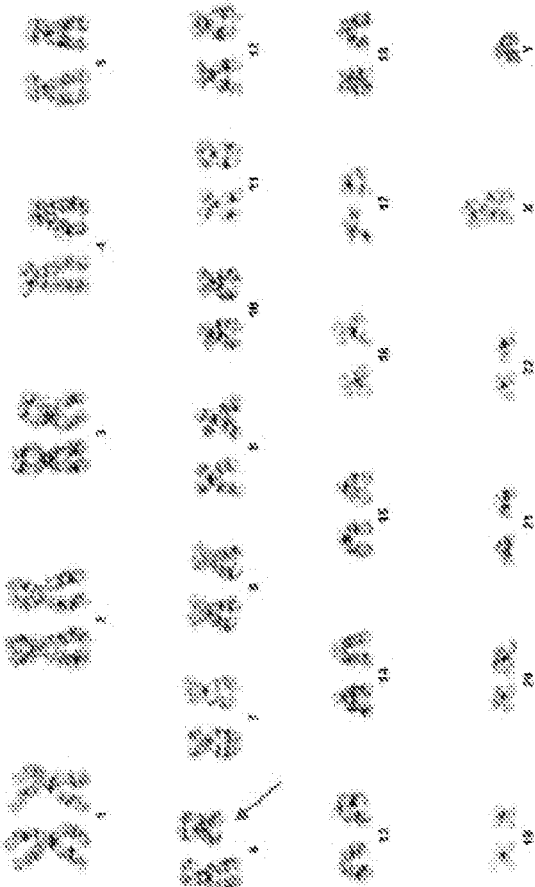


Figure 17