

## Establishment of a xenograft model of human myelodysplastic syndromes

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Online Supplementary Table S1. Clinical characteristics of the MDS and AML-MRC patients.

Patient N.	Age, y/sex	WHO (FAB)	Peripheral Blood				Bone Marrow			BM cytogenetics [% abnormal at diagnosis] <sup>†</sup>	
			WBC (×10 <sup>9</sup> /L)	Bl (%)	Hb (g/dL)	Plt (×10 <sup>9</sup> /L)	Bl (%)	Dysplasia*			
							Mye	Ery	Meg		
1	83/F	RCMD (RA)	5.6	0	8.3	383	0.4	-	±	±	46,XX
2	79/M	RCMD (RA)	11.4	0	6.7	319	0.4	±	±	-	46,XY
3	75/M	RCMD (RA)	3.4	0	12.9	125	0.9	±	±	-	46,XY
4	64/F	RAEB-1 (RAEB)	1.5	1	9.3	34	5.4	-	±	±	46,XX
5	72/M	RAEB-1 (RAEB)	1.4	0	7.5	35	6.7	+	+	+	43,XY,-5,-7,del(7)(q22),14,add(17)(p13),dms{40}/44,idem,-4,+mar2{45}
6	70/M	RAEB-2 (RAEB)	3.0	4.0	5.9	438	10.5	+	±	+	46,XY,del(5)(q13q33),del(7)(q11),del(20)(q11){90}
7	69/M	AML-MRC (RAEB-T)	1.6	0.5	9.4	110	28.6	+	±	+	46,XY
8	67/M	AML-MRC (RAEB-T)	29.2	26.0	9.0	164	7.8	+	±	+	47,XY,+8{45}
9	69/M	AML-MRC (RAEB-T)	2.0	0	16.5	40	21.4	+	±	+	45,XY,-7{100}
10	76/M	AML-MRC (RAEB-T)	15.4	4.0	6.8	19	29.6	+	+	+	46,XY,del(7)(q22){75}
11	67/M	AML-MRC (RAEB-T)	5.6	1.0	10.4	143	22.3	+	±	+	45,XY,-7,del(12)(p12p13){75}
12	73/F	AML-MRC (AML)	27.5	82.0	9.4	137	67.2	+	+	+	46,XX
13	53/M	AML-MRC (AML)	15.1	23.0	11.0	101	60.1	+	+	+	45,XY,-7{95}
14	77/M	AML-MRC (AML)	12.2	39.0	5.4	650	52.9	+	-	+	46,XY,i(17)(q10){100}

WHO indicates World Health Organization classification in 2008; RCMD, refractory cytopenia with multilineage dysplasia; RAEB, refractory anemia with excess blasts; AML-MRC, acute myeloid leukemia with myelodysplasia-related changes; FAB, French-American-British (classification); RA, refractory anemia; RAEB-T, refractory anemia with excess blasts in transformation; WBC, white blood cell; Bl, blasts; Hb, hemoglobin; Plt, platelets; Mye, myeloid; Ery, erythroid; Meg, megakaryocytes. \*Bone marrow dysplasia was categorized based on the percentages of morphologically abnormal cells in each lineage of cells indicated in the table; (+), more than 50%; (±), 10-50%; (-), less than 10%. †A total of 20 cells were examined for each patient.

Online Supplementary Table S2. Immunophenotypes of bone marrow cells at diagnosis.

Patient N.	CD7	CD11b	CD13	CD14	CD33	CD34	CD36	CD38	CD41b	CD56	CD61	CD64	CD117	MPO	HLA-DR
1	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT
2	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT
3	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT
4	44.0	16.6	89.7	13.1	91.5	89.9	26.0	98.4	12.7	12.7	15.1	13.5	65.6	15.1	96.1
5	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT	NT
6	34.4	8.1	32.7	2.8	14.8	27.1	41.3	78.9	19.0	12.0	NT	NT	NT	NT	50.9
7	1.3	1.0	87.0	4.1	74.7	81.1	16.1	93.2	4.0	1.1	5.4	12.7	38.1	32.7	90.0
8	33.4	2.1	97.2	0.5	15.6	98.6	1.0	27.7	1.5	0.6	2.4	1.3	93.1	64.0	96.6
9	0.9	9.8	88.7	19.7	50.8	85.5	31.1	98.7	2.6	69.2	5.3	10.1	52.9	22.1	97.3
10	19.5	37.1	62.8	0.9	64.0	47.5	41.1	93.2	2.3	14.0	26.7	25.8	36.6	48.1	84.5
11	15.0	40.5	71.7	29.8	49.7	50.0	49.1	89.9	35.4	47.1	34.3	37.1	22.0	77.4	86.4
12	3.3	7.7	61.4	4.6	77.8	94.1	57.5	22.6	5.5	1.1	7.9	NT	15.5	7.8	98.8
13	34.7	15.2	50.5	4.3	42.9	65.7	NT	92.2	26.3	4.4	NT	NT	NT	NT	44.2
14	8.9	31.5	91.2	9.7	50.6	55.9	32.6	96.2	39.3	1.5	46.9	8.7	56.7	11.1	90.3

Numerical figures in the table indicate the percentages of cells in the blast gate (CD45 low positive and SSC middle) expressing the indicated antigens. NT indicates not tested.

**Online Supplementary Table S3. Cytogenetic data for mice engrafted with human cells.**

Patient N.	FISH probe	% abnormal cells (abnormal/total nuclei)
6	7q31	100 (10/10)
9	7 centromere	0 (0/3)
10	7 centromere	0 (0/100)
11	7 centromere	100 (100/100)
13	7 centromere	100 (100/100)

**Online Supplementary Table S4. Engraftment of AML-MRC cells in serial transplants.**

Serial Tx.	N. of injected cells, x10 <sup>4</sup>	Injected cells	Auxiliary cells	Injection route	Percentage of human cells	Percentages of CD34 <sup>+</sup> cells in human cells*	Percentages of lineage cells in human cells* CD33 <sup>+</sup>	CD19 <sup>+</sup>
2 <sup>nd</sup>	40	WBM	allo	iBMT	15.56	31.43	91.84	1.79
3 <sup>rd</sup>	1980 <sup>†</sup>	WBM	-	iv	15.27	58.68	87.57	0.28
4 <sup>th</sup>	18	CD34 <sup>+</sup> CD33 <sup>-</sup>	-	iBMT	3.36	62.80	87.73	0
	12.5	CD34 <sup>+</sup> CD33 <sup>+</sup>	-	iBMT	3.18	59.75	94.33	0
	12.5	CD34 <sup>+</sup> CD33 <sup>+</sup>	-	iBMT	2.09	49.76	92.16	0
	4.4	CD34 <sup>+</sup> CD33 <sup>+</sup>	-	iBMT	0	NT	NT	NT
5 <sup>th</sup>	50	WBM	-	iv	48.95	76.77	94.93	0.63
	50	WBM	-	iv	18.6	76.68	93.85	0.30
6 <sup>th</sup>	90	hCD45 <sup>+</sup>	-	iv	10.86	77.59	93.62	0.26
	90	hCD45 <sup>+</sup>	-	iv	14.19	78.36	95.19	0.21
7 <sup>th</sup>	50	hCD45 <sup>+</sup>	-	iv	2.08	70.19	84.86	0
	50	hCD45 <sup>+</sup>	-	iv	1.29	76.56	89.26	0
8 <sup>th</sup>	15	hCD45 <sup>+</sup>	-	iv	<0.05	NT	NT	NT
	15	hCD45 <sup>+</sup>	-	iv	<0.05	NT	NT	NT

TX indicates transplantation; WBM, whole bone marrow cell; allo, allogeneic MSC; iBMT, intramedullary transplantation; iv, intravenous transplantation; -, not applicable; NT, not tested. \* Shown here are percentages of cell surface marker-expressing cells in the human CD45<sup>+</sup> gate. <sup>†</sup>The population includes 1x10<sup>6</sup> human CD45<sup>+</sup> cells by calculation.