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Table 1a

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	Age at therapy (months)	Follow up (months)	Mutation	Gamma- chain expression	Total cells infused (x10 ⁶)	Clinical status
P1	10	59	R289X	++	180	AW*
P2	10	55	S238N	-	180	AW
P3	4	50	Y125C	+/-	78	AW*
P4	36	42	R289X	++	115	AW*
P5	10	30	R222C	++	200	AW*
P6	10	27	PolyA	-	200	AW*
P7	6	20	M1i	-	84	AW*
P8	13	6	C182Y	+	207	AW
P9	7	2	S108P	+	160	AW
P10	12	0	del	-	60	AW

* Off prophylactic therapy, AW: Alive and well. Del: gene deletion

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Table 1b

	Patients		Pre-engraftment		Healthy Donor		Total	
	n	%	n	%	n	%	n	%
Exactly mappable RIS	304	100	134	100	140	100	578	100
RIS in RefSeq genes	126	41	60	45	69	49	255	44
RIS in RefSeq genes including the 10 kb surrounding region	188	62	88	66	95	68	371	64
RIS near TSS (+/- 5 kb)	69	23	33	25	41	29	143	25
RIS close to CpG islands (+/- 1 kb)	39	13	32	24	20	14	91	16

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Table 2

Pat.	Source	Chromosome	RefSeq Gene	5' of TSS [bp]	3' of TSS [bp]	Intron	3' of RefSeq Gene [bp]	UCSC Locus
2	post	2p15	B3GNT1	14710				62383086
2	post	2p15	B3GNT1	14191				62383605
1	post	20q13.2	BCAS1				8358	53237581
1	post	20q13.2	BCAS1				2307	53243632
2	post	19q13.32	BCL3	7654				49936227
2	post	19q13.32	BCL3	3255				49937616
1	post	1q32.2	CD34		20481	4		205147833
1	post	1q32.2	CD34		7313	1		205160901
3	post	11q21	FN5		11636	1		92953031
3	post	11q21	FN5		11339	1		92953328
1	post	19q13.31	KCNN4		2388	1		48974861
2	post	19q13.31	KCNN4		3011	1		48974238
3	post	8q11.23	LOC115294	35852				52859609
3	post	8q11.23	LOC115294	35924				52859681
2	post	2q37.3	LRRFIP1	3273				238884406
2	post	2q37.3	LRRFIP1	3205				238884474
3	post	4q31.1	MAML3		60909	1		141591951
3	post	4q31.1	MAML3		60822	1		141592038
1	post	10p11.23	MAP3K8				36329	30791096
1	post	10p11.23	MAP3K8				39821	30794588
1	post	12p11.22	MLSTD1		17400	1		29285265
5	post	12p11.22	MLSTD1		16713	1		29284578

4	post	6p24.2	NEDD9	150399				11491283
5	post	6p24.2	NEDD9	157120				11498004
3	post	20q13.12	PRKCBP1		479	1		46670417
5	post	20q13.12	PRKCBP1	5009				46675905
2	post	4p12	TEC		21977	1		48165379
2	post	4p12	TEC		21779	1		48165577
1	post	1q21.3	TSRC1				5581	147755571
4	post	1q21.2	TSRC1				6239	147756234
6	pre	11q13.4	FLJ20625		3546	1		71537084
6	pre	11q13.4	FLJ20625		1756	1		71538874
nd	pre	2p11.2	CAPG		4496	1		85607518
nd	pre	2p11.2	CAPG		3717	1		85608297
nd	pre	10q11.21	HNRPF		643	1		43187977
nd	pre	10q11.21	HNRPF	10998				43199618
nd	pre	17q21.32	MYL4	4830				45756236
nd	pre	17q21.32	MYL4	1573				45759493

Molecular Function					Biological process			
Patient Samples	Level	Category	List hits	Fisher exact	Level	Category	List hits	Fisher exact
	2	kinase activity	21	0.0000016	5	protein amino acid phosphorylation	16	0.0000254
	4	phosphotransferase activity, alcohol group as acceptor	18	0.00000944	3	phosphorus metabolism	19	0.0000326
	3	protein kinase activity	16	0.0000186	4	phosphate metabolism	19	0.0000326
	3	transferase activity, transferring phosphorus-containing groups	19	0.0000437	5	phosphorylation	16	0.0000831
	4	protein serine/threonine kinase activity	12	0.000115	4	protein modification	22	0.000143
Pre-engraftment samples	5	SH3/SH2 adaptor protein activity	3	0.000925		RAS protein signal transduction	3	0.00026
	4	transmembrane receptor protein tyrosine kinase docking protein activity	3	0.000997	5	small GTPase mediated signal transduction	6	0.000524
	3	transmembrane receptor protein tyrosine kinase signaling protein activity	3	0.00141	4	Intracellular signaling cascade	10	0.0041
	3	small GTPase regulatory/interacting protein activity	4	0.00239				
	2	Receptor signaling protein activity	4	0.00852				
Control Samples	3	cytokine binding	3	0.00382	3	cellular defense response	5	0.000143
	2	enzyme activator activity	4	0.00935	4	response to biotic stimulus	13	0.000515
					4	response to pest/pathogen/parasite	9	0.000581
					3	immune response	11	0.00126
					5	defense response	11	0.0028
					4	response to wounding	6	0.00282

5 **Table 3**

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Table 4

	Patients 4, 5,10		Patients 1,2,6,7,8,9		total	
	absolute	percent	absolute	percent	absolute	percent
Exactly mappable IS	210	100	362	100	572	100
IS in RefSeq genes	81	39	135	37	216	38
IS in RefSeq genes including the region 10 kbps upstream and downstream of the gene	130	62	226	62	356	62
IS within +/- 5 kb around TSS	59	28	98	27	157	27
IS close to CpG islands (+/- 1 kb)	34	16	66	18	100	17

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Table 5

	P1 (56)	P2 (101)	P4 (141)	P5 (52)	P6 (23)	P7 (94)	P8 (79)	P9 (9)	P10 (17)
	2	1	3	2		1			
ZNF217			2	1		1	3		1
LMO2	1		2	1			1		
NOTCH2	2	1							
RUNX3			2				1		
RUNX1	1	2							
C14orf4		1	1	2					
AFTIPHI			2			1			
LIN									
FAM9C		2					1		
PDE4B	1			1					1
PRKCBP		1					2		
1									
PTPRC			1	1		1			
TOMM20	1					1	1		
TSRC1			1		1	1			

5 Table 6

Molecular Function

Level	Category	List hits	Fisher Exact
2	kinase activity	25	0.00018
2	receptor signaling protein activity	10	0.000574
3	protein kinase activity	20	0.000244
3	transferase activity, transferring phosphorus-containing groups	25	0.000373
3	DNA binding	46	0.000398
4	phosphotransferase activity, alcohol group as acceptor	23	0.000111
4	protein serine/threonine kinase activity	15	0.000717

Biological Process

Level	Category	List hits	Fisher Exact
2	death	17	0.000657
3	phosphorus metabolism	24	0.000542
3	cell death	17	0.000601
4	phosphate metabolism	24	0.000542
4	intracellular signaling cascade	26	0.00122
4	programmed cell death	17	0.000315
4	cell proliferation	29	0.00162
5	apoptosis	17	0.000305
5	protein amino acid phosphorylation	18	0.00194