

**Report from the ASGT Ad Hoc Committee on Retroviral-mediated Gene Transfer to Hematopoietic Stem Cells
April 2003**

Clinical Trials of Gene Transfer to Hematopoietic Stem Cells by Retroviral Vectors									Cancer and HIV			03.03.03
Disease	PI	Disease	Source	Gene	n	Dates	# cells	# CD34/kg	% Gene Transduced		Duration	Reference
Gene Marking in Cancer/Leukemia									CFU in Transplant			
3A1	Brenner	AML	BM	neo	27							1,2,3
3A2	Brenner	Neuroblastoma	BM	neo	12							1,2,3
3A3	Dunbar	Breast Ca, MM	BM/PBSC	neo	24	12/92-11/96	20-290e6	0.1-4.0	1-55% cfu-gm		1-21 mo	4, 5
3A4	Cornetta	Acute leukemia	BM	neo	5	5/92-8/93	8e8-2e9	n.d.	2.2-6.7% CFU-GM			5A
3A5	Deisseroth	CML	PBSC	neo								6
3A6	Deisseroth	NHL	BM vs PBSC	neo	3						9 mo	7
3A7	Verfaillie-most complete data	CML	PBSC	neo	11	??	.55-8.8e8	0.7-6.9e6	<1% CFU		<24 mo	
Chemotherapy Resistance Genes												
3B1	Cowan	Breast		mdr	4							8,9
3B2	Moscow	Breast		mdr/neo	6							10
3B3	Deisseroth	Breast/Ovarian		mdr	18							11,12
3B4	Linch	NHL		mdr	3						not detected	13
3B5	Hesdorffer	Ca	BM	mdr	5						<3 mo	14
3B6	Abonour	Germ Cell Ca	PBSC	mdr	11	7/97-9/02	1.6-8.3e8	2.3-13.4	4-52%		9 mo	15
3B7	Croop	Brain Tumor-Ped/Adult	PBSC	MGMT	9	8/98-6/00	3.2-14.7e8	3.2-16.9e6	0-60%		0-13 mo	
3B8	Gerson	Sarcoma	PBSC	MGMT	1	10/02-	7.00E+07	1.08E+06	21% CFU		not detected	
HIV-1 Resistance genes												
3C1	Mitsuya	HIV	PBSC	Ribozyme	10	2/98-1/00		1.3-10	0.4-57%		6->36 mo	16,17
3C2	Zaia	HIV	PBSC	Ribozyme	5							18
3C3	Zaia	HIV/Lymphoma	PBSC	Ribozyme	5				0.6-3.2	13-26%	<112 mo	

