

Supplementary Appendix

This appendix has been provided by the authors to give readers additional information about their work.

Supplement to: Hacein-Bey-Abina S, Le Deist F, Carlier F, et al. Sustained correction of X-linked severe combined immunodeficiency by ex vivo gene therapy. *N Engl J Med* 2002;346:1185-93.

SUPPLEMENTARY APPENDIX 1

TABLE 1. LEVELS OF LYMPHOCYTE SUBGROUPS AT THE LAST FOLLOW-UP VISIT.*

VARIABLE	PATIENT 1	PATIENT 2	PATIENT 4	PATIENT 5	CONTROLS
Months of follow-up	26	21	17	13	
Lymphocytes (cells/mm ³)	2800	5400	6100	9850	4000–6000
CD3+ (%)	80	83	77	83	75–85
α/β T-cell receptor	93	98	91	97	90–95
γ/δ T-cell receptor	6	2	8	3	0–10
CD4+ (%)	25	41	40	56	40–55
CD45RO+	41	18	8	7	20–40
CD45RA+	60	82	92	92	60–80
CD8+ (%)	46	31	27	23	15–30
CD45RO+	25	9	8	4	20–40
CD45RA+	74	92	94	97	60–80
CD16+CD56+	1	0.7	1	0.6	3–10
CD19+ (%)	19	18	19	20	5–20
CD27+	3	9	11	8	>10

*Control values are given for age-matched children. Because of rounding, not all percentages total 100.

TABLE 2. T-CELL PROLIFERATION AT THE LAST FOLLOW-UP VISIT.*

VARIABLE	PATIENT 1	PATIENT 2	PATIENT 4	PATIENT 5	CONTROLS
Months of follow-up	20	21	17	13	
Phytohemagglutinin (counts/min)	220,000	188,000	180,000	37,000	>40
Anti-CD3 (counts/min)	167,000	35,000	131,000	45,000	>20
Tetanus toxoid (counts/min)	5,500	40,000	26,000	3,400	>10
Tetanus toxoid and interleukin-2 (counts/min)	ND	62,000	45,000	19,000	>10
Interleukin-2 (counts/min)	ND	2,700	6,000	8,000	<10
Poliovirus (counts/min)	8,300	24,000	15,000	ND	>10

*Patients' T-cell proliferation was studied in vitro after immunization with tetanus toxoid and polioviruses. In unstimulated conditions, values were less than 5000 counts per minute. ND denotes not done. Control values are given for age-matched children.

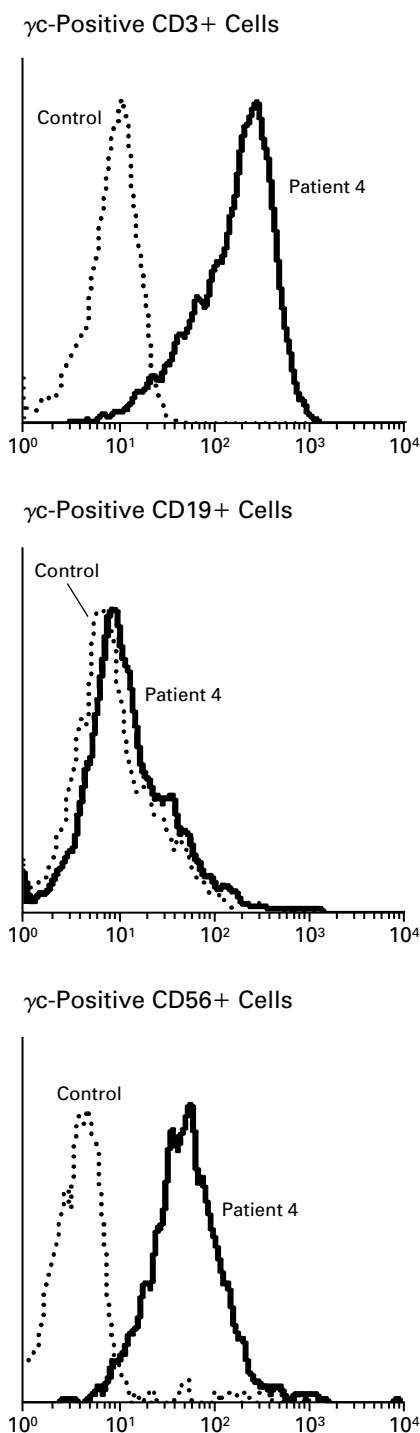


Figure 1. Flow-Cytometric Analysis of the Expression of the Common γ (γ_c) Chain by CD3+, CD19+, and CD56+ Lymphocytes in Patient 4 17 Months after Gene Therapy and in a Control.

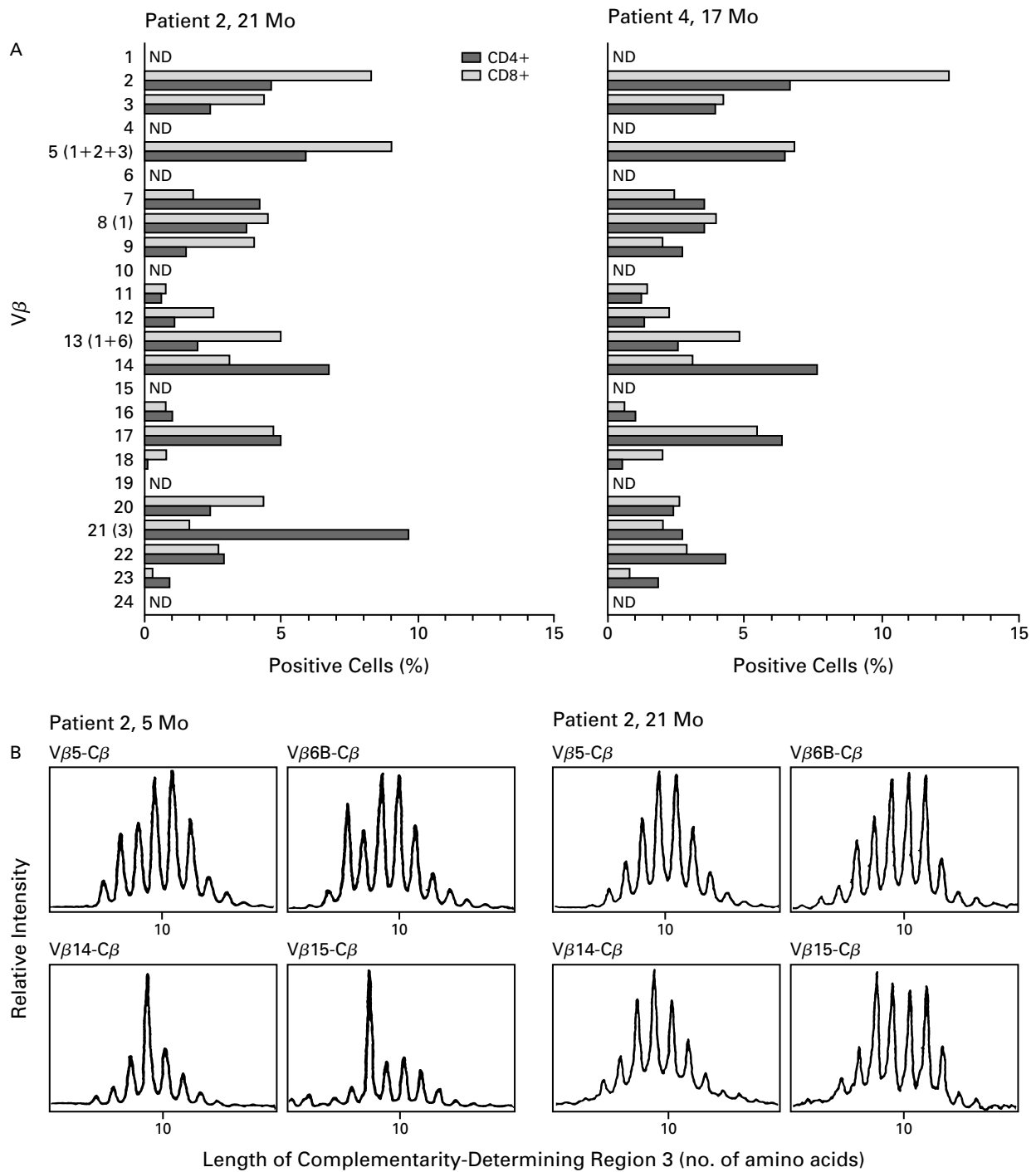


Figure 2. Diversity of T-Cell–Receptor $V\beta$ Repertoire.

As shown in Panel A, CD4+ T cells and CD8+ T cells from Patient 2 at 21 months and Patient 4 at 17 months were stained with antibodies specific for 17 different $V\beta$ families, as indicated. ND denotes not done. Panel B shows length profiles for complementarity-determining region 3 for the $V\beta 5$, $V\beta 6$, $V\beta 14$, and $V\beta 15$ T-cell populations of Patient 2 at 5 months and 21 months. Numbers in parentheses are the monoclonal antibodies used to stain the different families of $V\beta$ receptors.