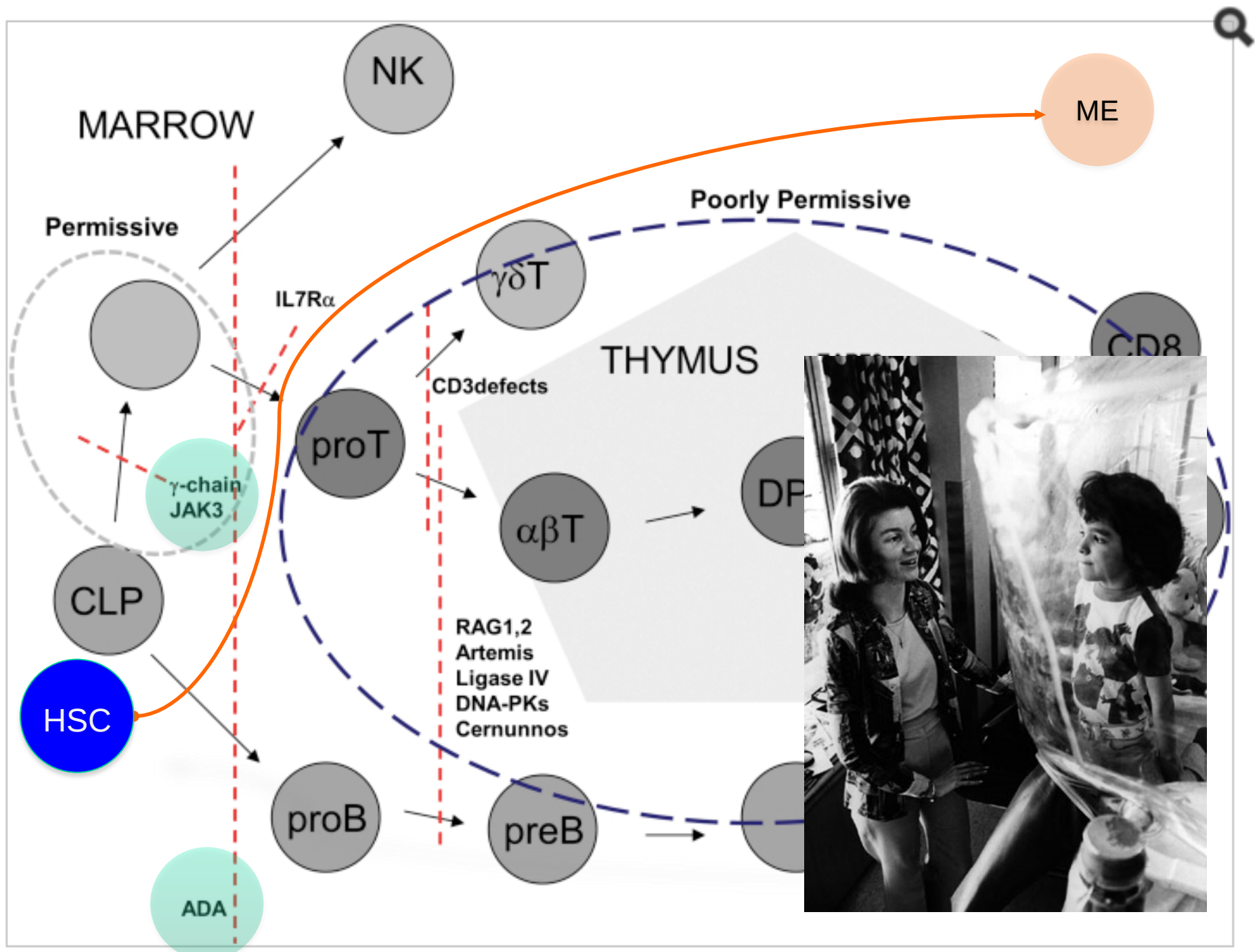


Somatic cell therapy

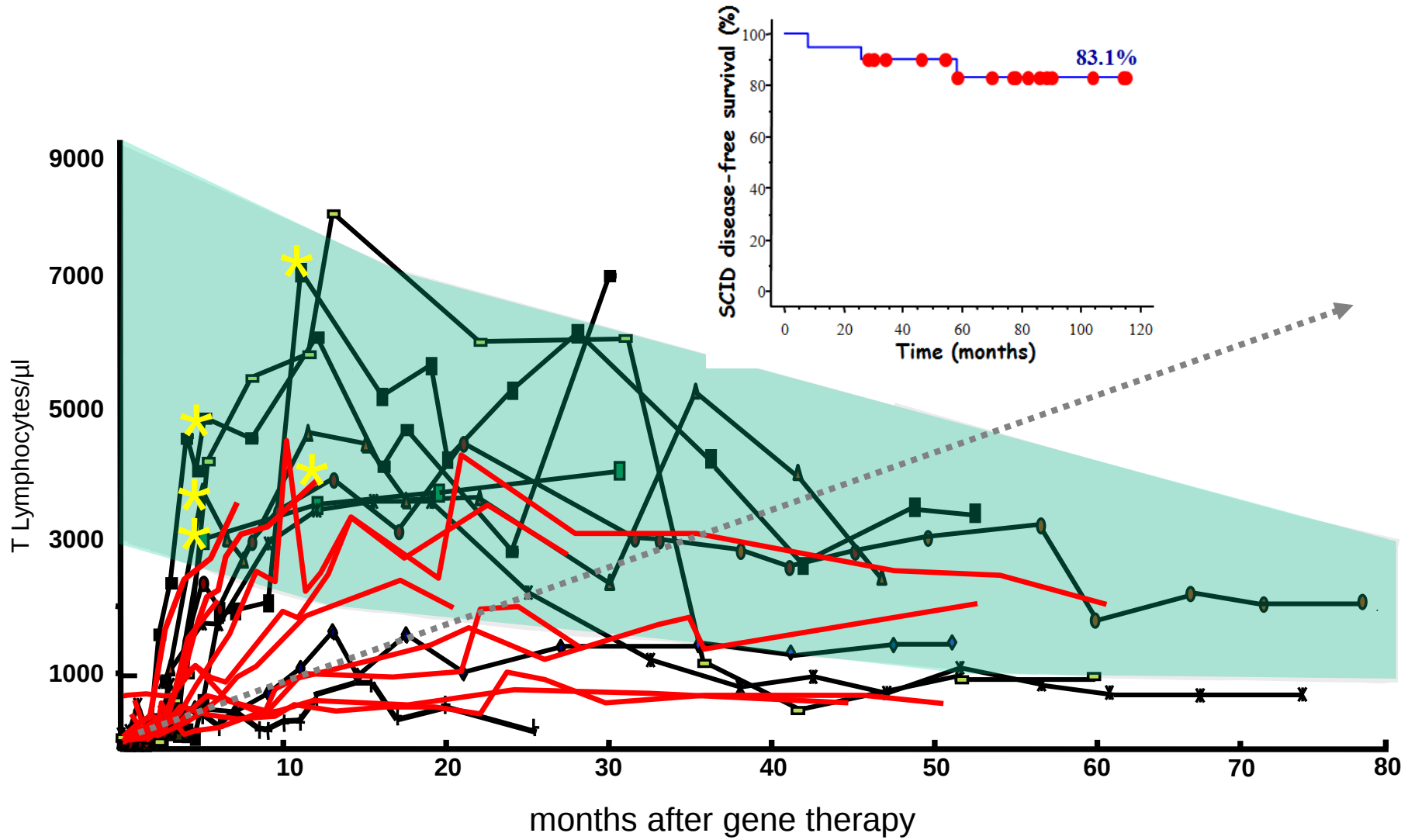
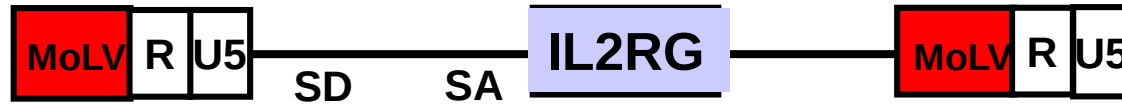
Adrian Thrasher
Fyodor Urnov
Matthew Porteus
Weizhi Ji

Chad Cowan
Marco Weinberg



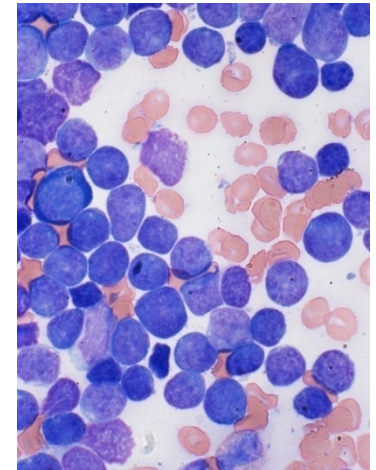
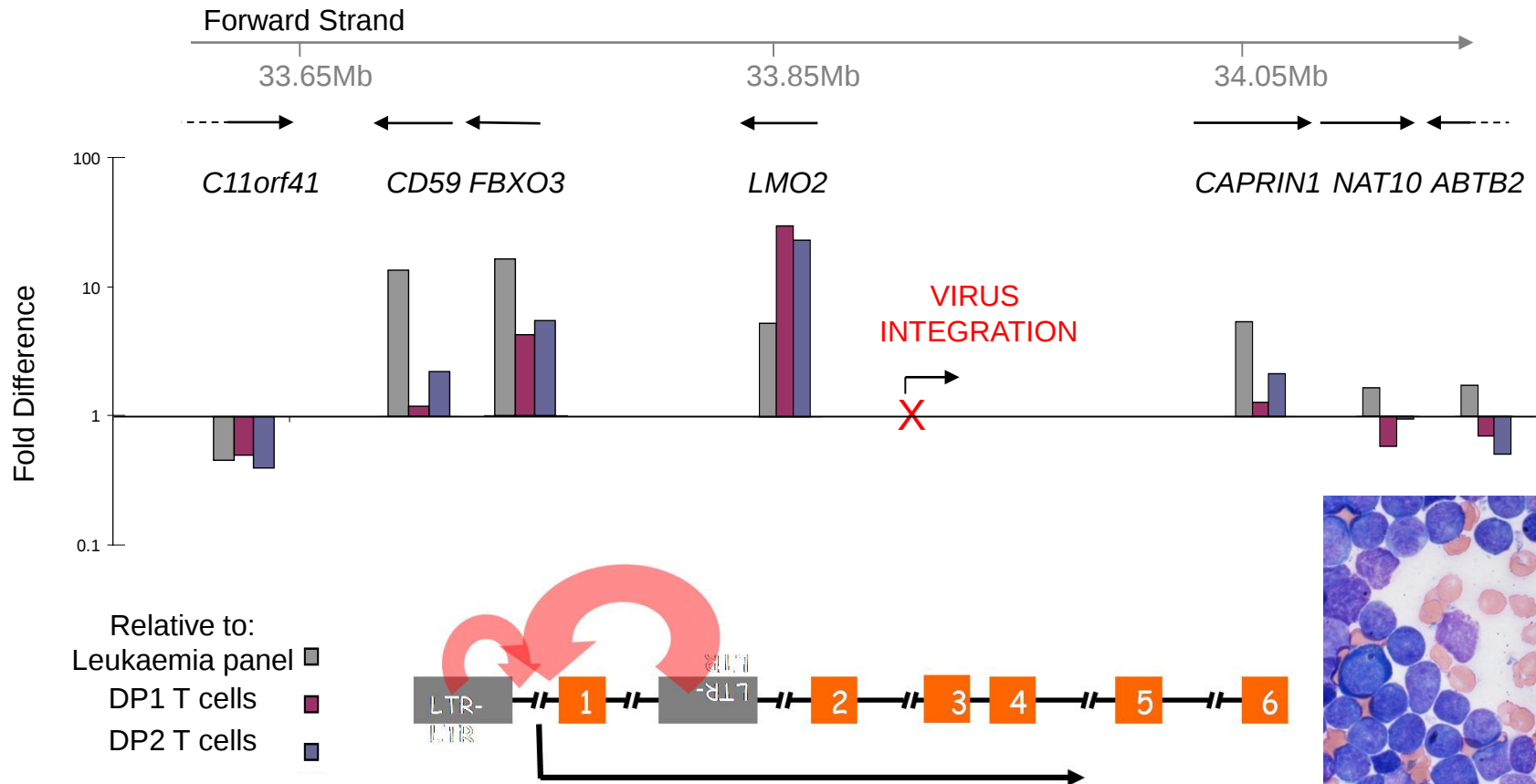


Gene Therapy for Severe Immunodeficiency (2000-)

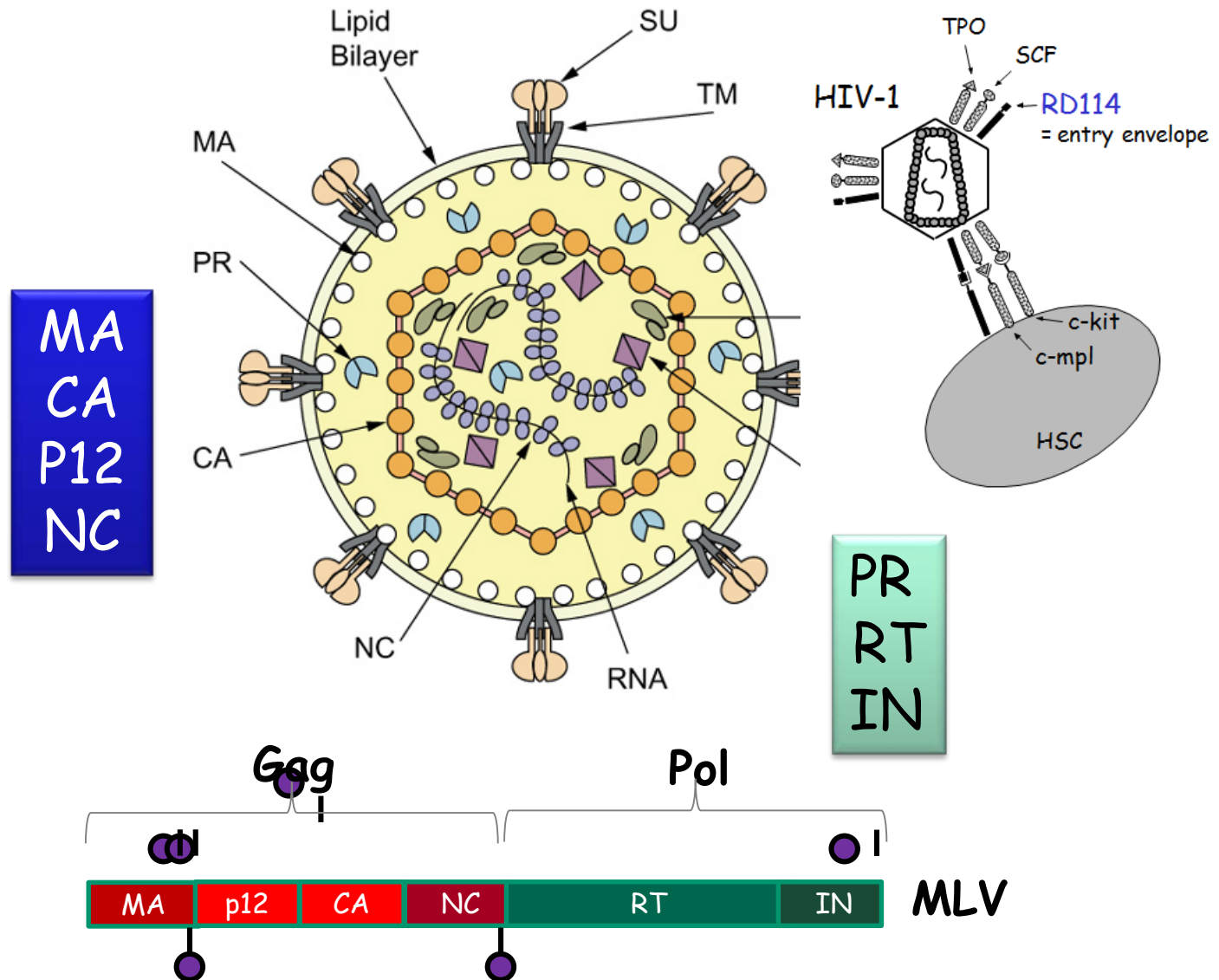


Enhancer-mediated insertional mutagenesis...

Chromosome 11p13



Evolving technologies for effective and safe gene therapies...



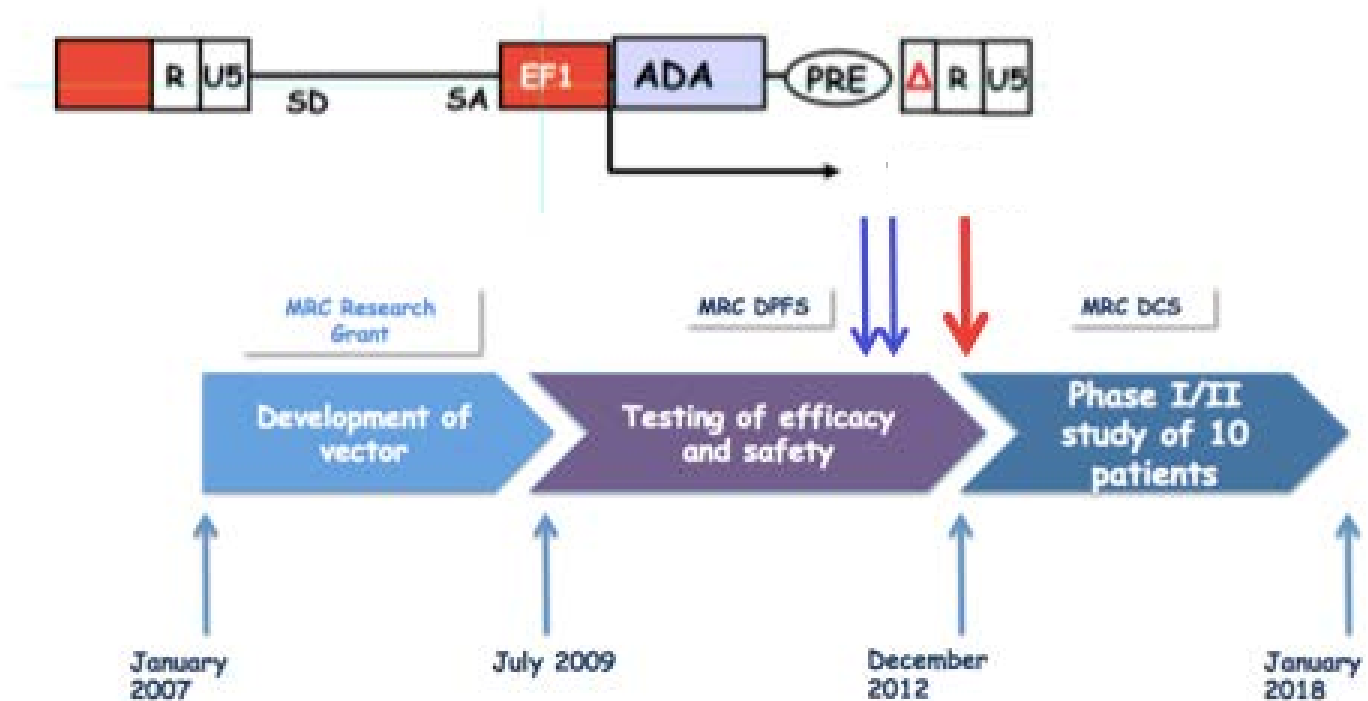
ORIGINAL ARTICLE

A Modified γ -Retrovirus Vector for X-Linked Severe Combined Immunodeficiency

S. Hacein-Bey-Abina, S.-Y. Pai, H.B. Gaspar, M. Armant, C.C. Berry, S. Blanche, J. Bleesing, J. Blondeau, H. de Boer, K.F. Buckland, L. Caccavelli, G. Cros, S. De Oliveira, K.S. Fernández, D. Guo, C.E. Harris, G. Hopkins, L.E. Lehmann, A. Lim, W.B. London, J.C.M. van der Loo, N. Malani, F. Male, P. Malik, M.A. Marinovic, A.-M. McNicol, D. Moshous, B. Neven, M. Oleastro, C. Picard, J. Ritz, C. Rivat, A. Schambach, K.L. Shaw, E.A. Sherman, L.E. Silberstein, E. Six, F. Touzot, A. Tsytsykova, J. Xu-Bayford, C. Baum, F.D. Bushman, A. Fischer, D.B. Kohn, A.H. Filipovich, L.D. Notarangelo, M. Cavazzana, D.A. Williams, and A.J. Thrasher

London, Boston, Cincinnati, Los Angeles, Paris
14 patients treated November 2015

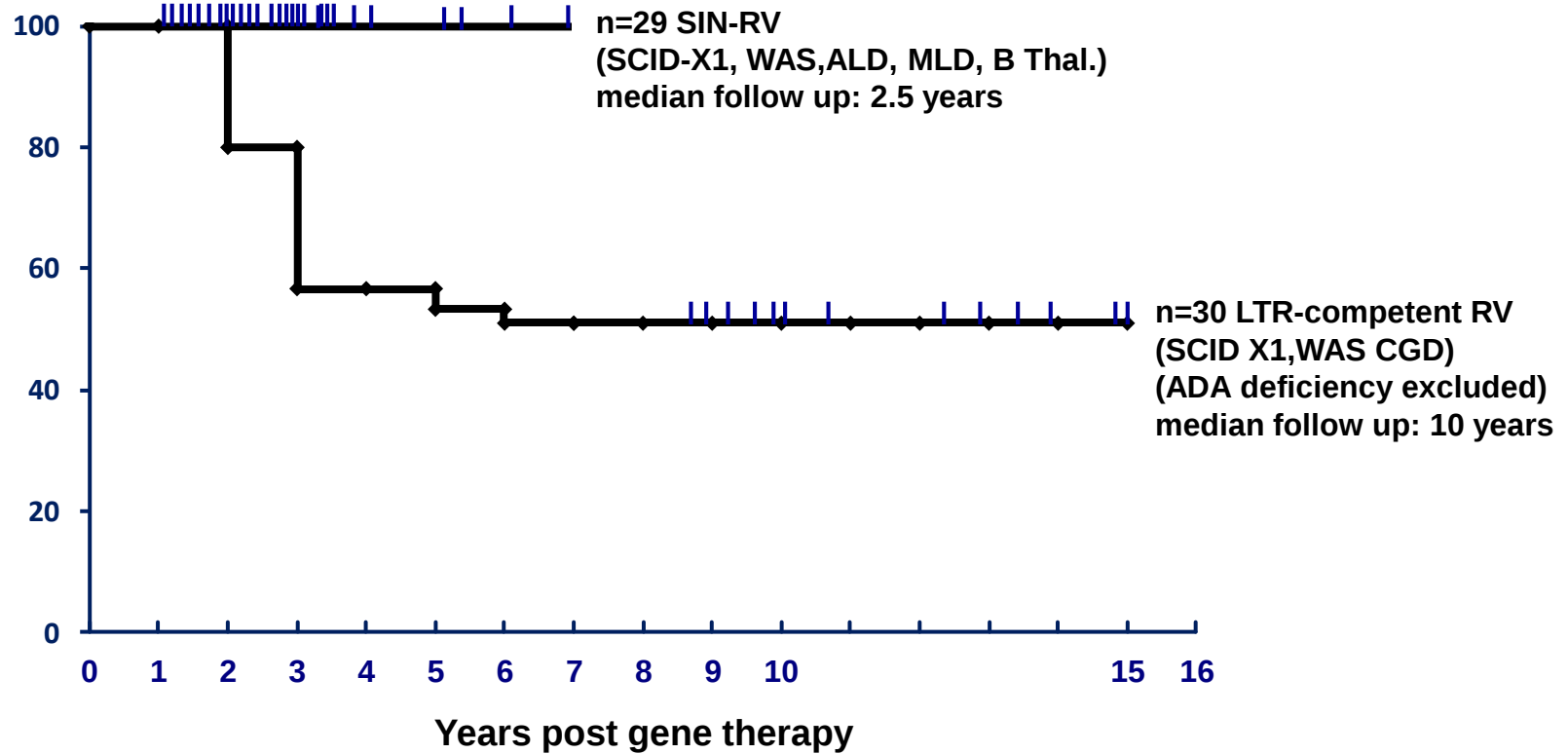
Phase I/II trial to assess the safety and efficacy of EF1 α S-ADA lentiviral vector mediated gene therapy



UCL-GOSH and UCLA: enrolled 29 patients October 2015

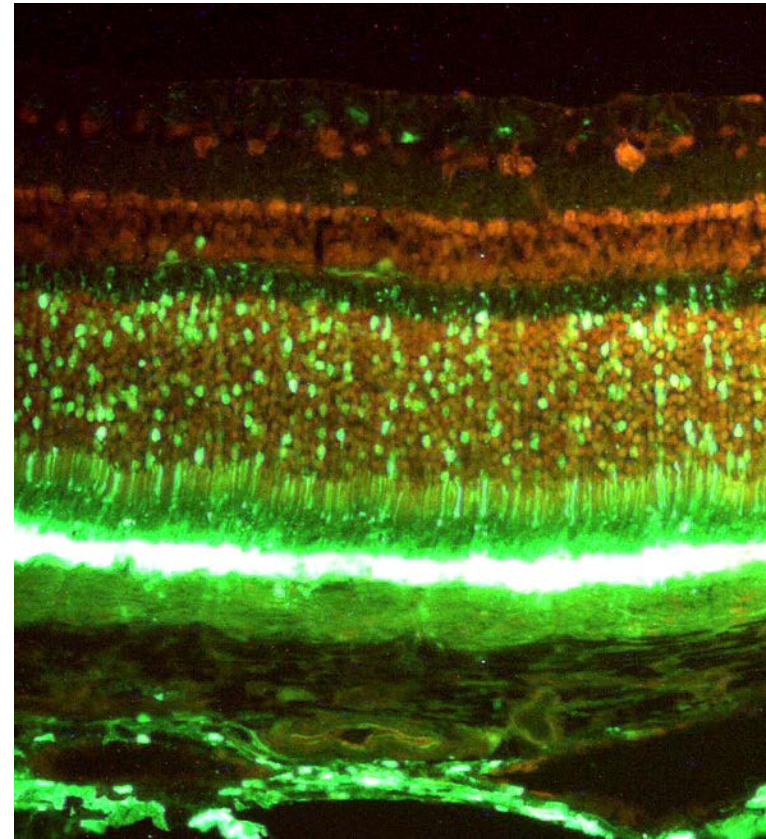
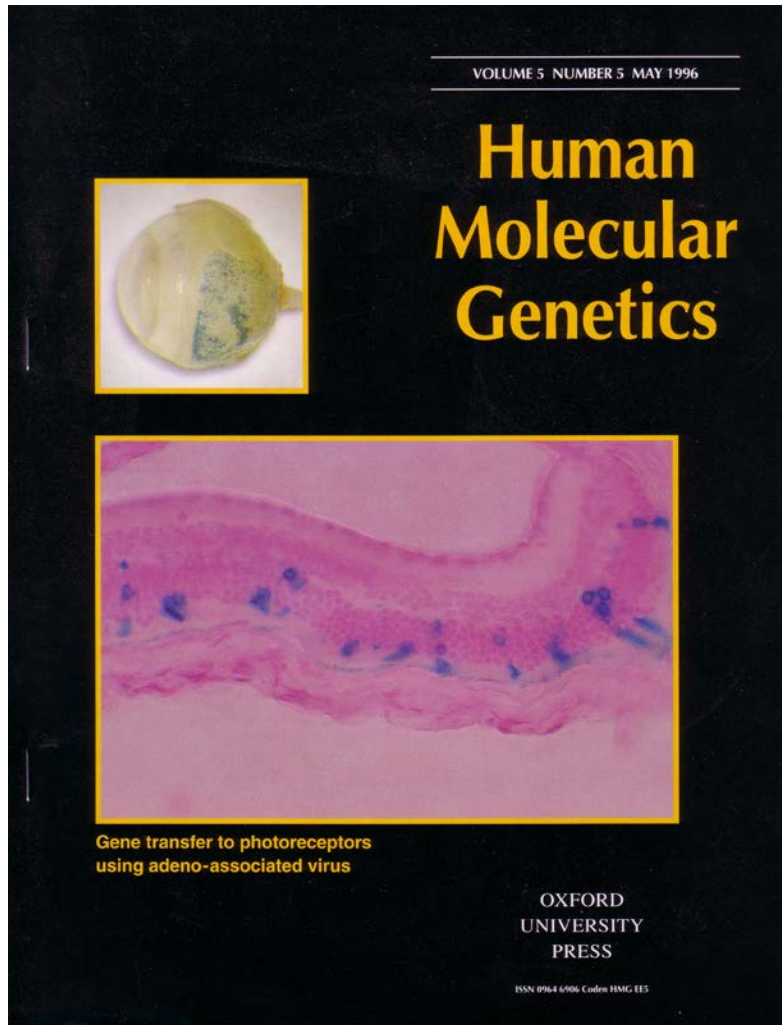
Vector-mediated toxicity with SIN-RV vectors...

Event free survival

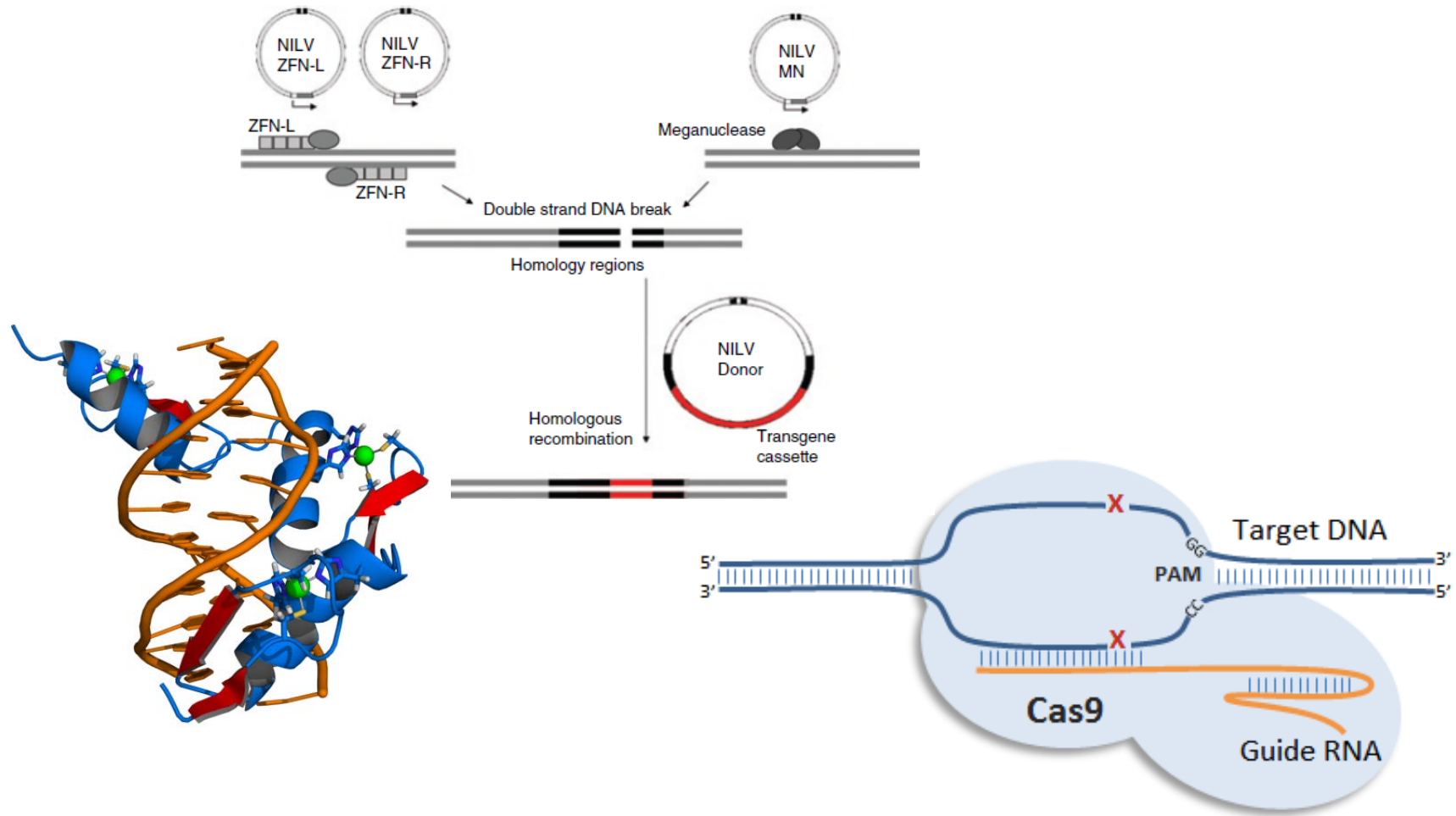


courtesy Alain Fischer

Successful gene therapy for inherited blindness...



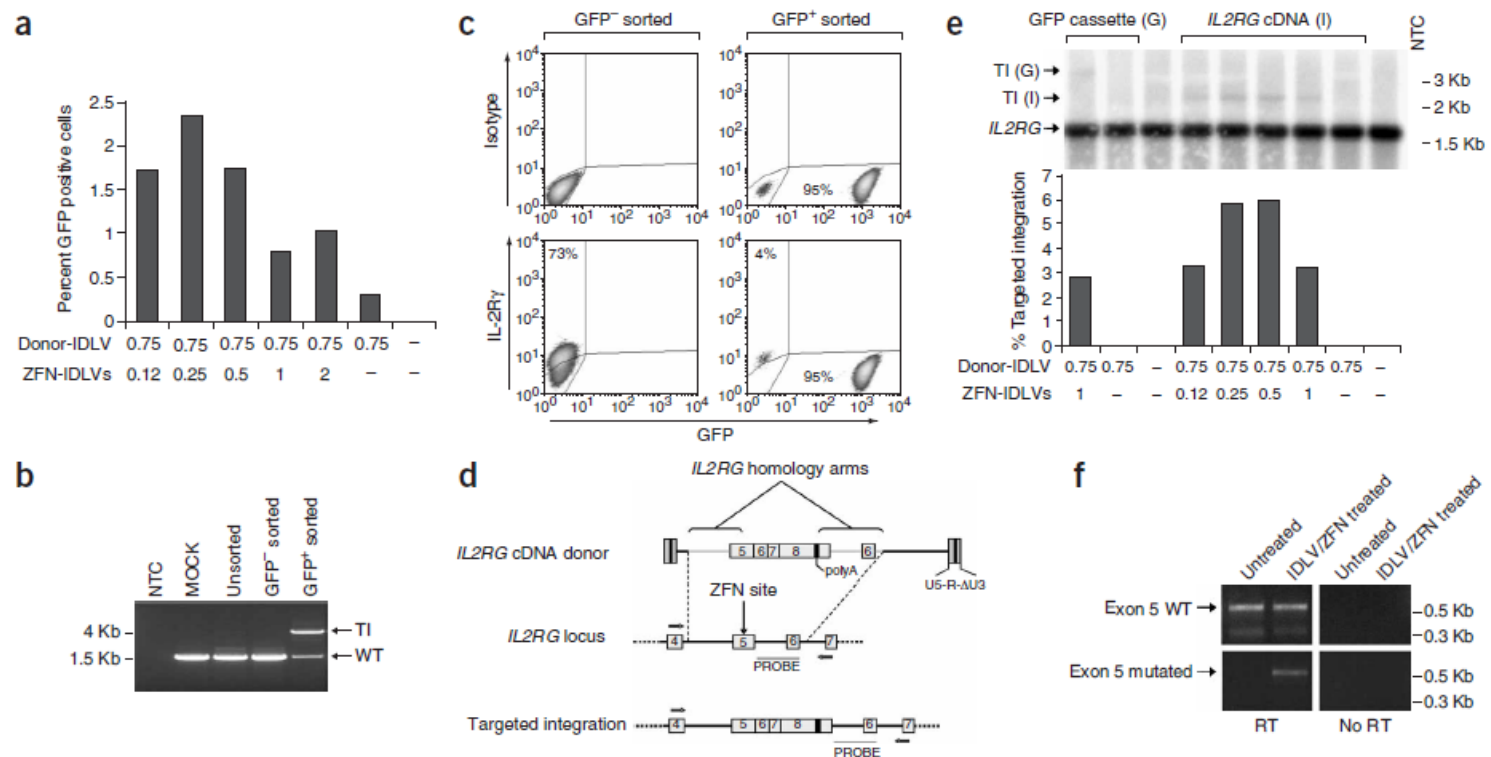
Somatic gene editing: ZFN, TALEN, CRISPR Cas9, CRISPR C1b4.....

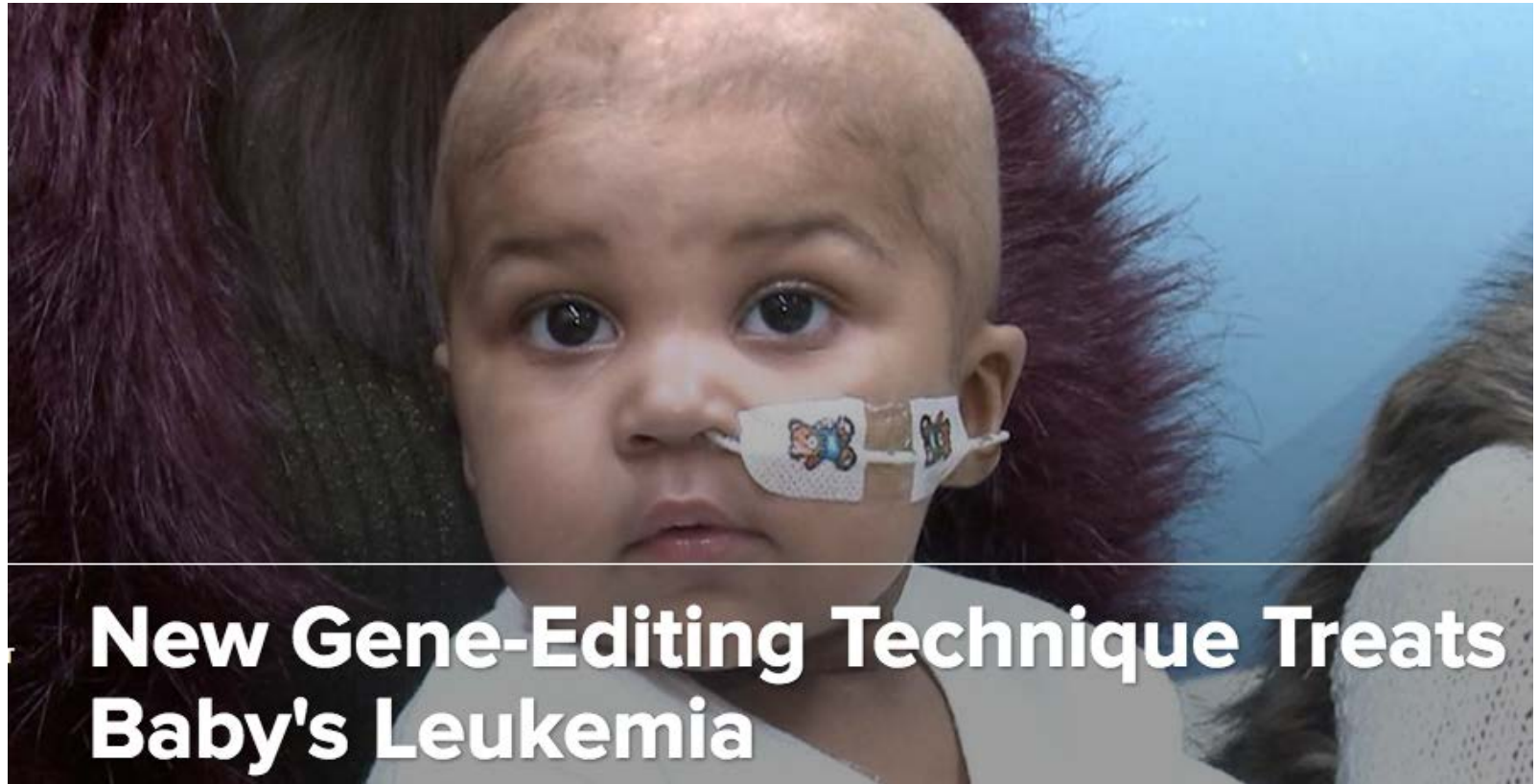


Gene editing in human stem cells using zinc finger nucleases and integrase-defective lentiviral vector delivery

Angelo Lombardo^{1,2}, Pietro Genovese^{1,2}, Christian M Beausejour^{3,6}, Silvia Colleoni⁴, Ya-Li Lee³, Kenneth A Kim³, Dale Ando³, Fyodor D Urnov³, Cesare Galli^{4,5}, Philip D Gregory^{3,7}, Michael C Holmes^{3,7} & Luigi Naldini^{1,2,7}

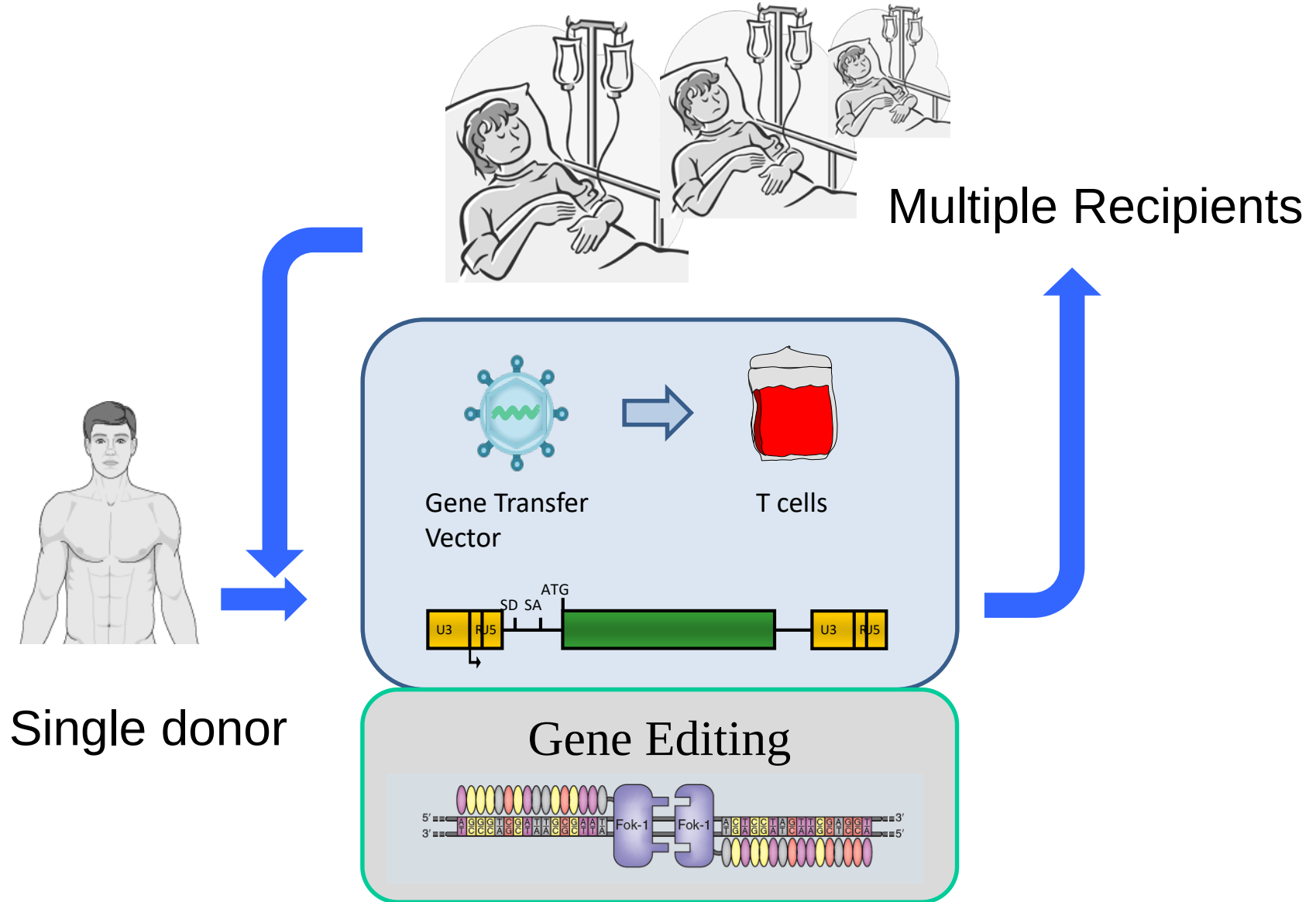
ARTICLES





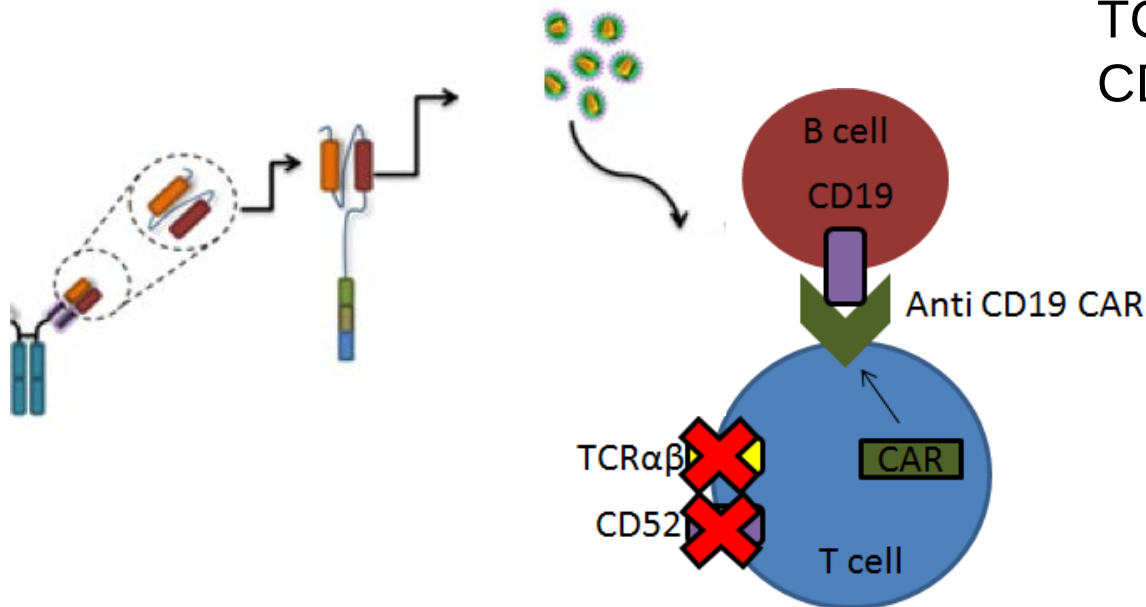
New Gene-Editing Technique Treats Baby's Leukemia

Engineering T cells for cancer therapy.....



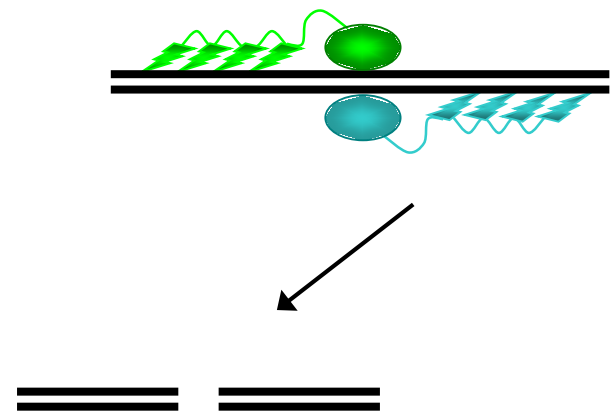
Engineering T cells for cancer therapy.....

Viral vector inserts Anti-leukaemia genes
(CAR19)



TALEN gene editing

TCR gene knockout
CD52 gene knockout



Somatic cell therapy

Adrian Thrasher
Fyodor Urnov
Matthew Porteus
Weizhi Ji

Chad Cowan
Marco Weinberg

