

## SCIENTIFIC ABSTRACT

Gene therapy may provide a new therapeutic approach to pediatric AIDS. Our central hypothesis is that a gene encoding an anti-HIV RNA molecules (an RRE decoy) can be inserted into CD34+ cells from bone marrow of HIV-1 infected children leading to production of mature T cells and monocytes which express the anti-HIV gene and are thereby resistant to HIV-1 replication. The de novo development of functional T lymphocytes from hematopoietic stem cells is most likely to be successful with young patients with intact thymic function. The most direct way to test this hypothesis is with a clinical trial of CD34+ cell transduction, in which the engraftment and survival of cells transduced by a retroviral vector carrying an anti-HIV-1 gene, an RRE decoy (L-RRE-neo) are compared to a frequency of cells transduced by a retroviral vector transduced by a neutral marker vector (LN). Although this initial trial of gene transfer into CD34+ cells may not have significant impact on the course of disease, it should provide valuable data on the potential efficacy of these techniques.