

Non-technical Abstract

HIV infection causes progressive destruction of the human immune system and eventually AIDS (Acquired Immune Deficiency Syndrome) in the overwhelming majority of patients. No curative treatments for HIV or AIDS currently exist. T cells are the main targets of HIV in the body. When a specific gene for a molecule called a ribozyme is introduced in the laboratory into human T cells they are protected (in the test tube) against HIV infection. The ribozyme does not have apparent harmful effects on the T cells. The ribozyme acts at two steps in the lifecycle of HIV: by cutting the genes of the virus as it enters cells and by cutting the genes of the offspring of virus in infected cells. In this study, we will evaluate the safety and usefulness of ribozyme gene therapy in four to six patients with HIV infection. We will do so by introducing the ribozyme gene into each patient's T cells in the laboratory and then transferring the cells back into the body. We will then study whether the lifespan of these cells is prolonged by having the ribozyme gene and whether there are side effects of the therapy. The results will determine whether this ribozyme can safely protect T cells in patients with HIV infection and will aid design of future trials of gene therapy for HIV and AIDS.