

**Theodore Friedmann, M.D.**

Director  
Program in Human Gene Therapy  
University of California School of  
Medicine, San Diego  
Department of Pediatrics  
CMGB Building  
La Jolla, CA 92093-0634  
United States

**In Vivo Gene Transfer with Retrovirus Vectors**

The efficient use of retrovirus and lentivirus vectors for in vivo gene delivery in gene therapy applications will be greatly facilitated by improved methods to characterize the bio-distribution and fate of the vectors after systemic administration and to understand the mechanisms that determine tissue tropism of vectors in vivo. We have been developing methods to detect virus particles in vivo that take advantage of the existence of the cell-derived membrane that the viruses acquire in the process of budding from producer cells. We have labeled virus particles with a fluorescent membrane marker and studied factors affecting the distribution and virus particle attachment to a variety of tissues, especially the vascular endothelium and the liver, after systemic administration of the fluorescent-tagged virus particles. Systematically delivered virus is rapidly cleared from the circulation concomitant with rapid uptake into tissues of the reticuloendothelial system, particularly the liver and spleen. Fluorescent particles are also found to associate with the microvascular endothelium by mechanisms that may involve P-selectin.

- |               |   |
|---------------|---|
| 1956          | A.B., Chemistry, University of Pennsylvania, Philadelphia, PA                                       |
| 1960          | M.D., University of Pennsylvania  |
| 1960-1961     | Intern in Pediatrics, Children's Hospital and Medical Center, Boston, MA                            |
| 1961-1962     | Resident in Pediatrics, Children's Hospital, Boston, MA   |
| 1962-1963     | U.S. Air Force, Captain 10th Tactical Hospital, Alconbury, UK                                       |
| 1963-1964     | Research Fellow in Colloid Science, University of Cambridge, Cambridge, UK                          |
| 1964          | Biology, University of Cambridge  |
| 1964-1965     | Senior Researcher in Pediatrics/Research Fellow, Children's Hospital and Medical Center, Boston, MA |
| 1965-1965     | Fellow in Pediatrics and Research Fellow, Harvard University, Cambridge, MA                         |
| 1965-1967     | Fellow in Laboratory of Chemical Biology, NIAMD, NIH, Bethesda, MD                                  |
| 1967-1968     | Medical Officer, Laboratory of Human Genetics, NIAMD, NIH   |
| 1968-1969     | Postdoctoral Fellow, The Salk Institute, La Jolla, CA   |
| 1969-1973     | Assistant Professor of Pediatrics, University of California, San Diego, La Jolla, CA                |
| 1973 to 1981  | Associate Professor of Pediatrics, University of California, San Diego                              |
| 1977          | Biology, University of Cambridge  |
| 1981-present  | Professor of Pediatrics, University of California, San Diego  |
| 1984          | Pathology, University of Oxford, Oxford, UK   |
| 1993-1998     | Co-leader of Cancer Genetics Program, University of California, San Diego Cancer Center             |
| 1989-present  | Muriel Jeannette Whitehill Chair in Biomedical Ethics, University of California, San Diego          |
| 1994-present  | Director, Program in Human Gene Therapy, University of California, San Diego                        |
| <b>Honors</b> |   |
| 1989-present  | Editor, <i>Molecular Genetic Medicine</i> and <i>Adv. Genetics</i> , Acad. Press                    |
| 1992          | University of California Chancellor's Association Award for Excellence in Research                  |
| 1994-1995     | Newton-Abraham Visiting Professor and Fellow, Lincoln College, University of Oxford                 |
| 1995-present  | Chairman, Scientific Advisory Board, Lesch Nyhan Syndrome, Children's Research Foundation           |
| 1998-present  | Chairman, Government Affairs Committee, American Society of Human Gene Therapy                      |