

Gene Therapy Gene therapy is a process in which exogenous genetic material is transferred into somatic cells to correct an inherited or acquired gene defect. Also, it is intended to introduce a new function or property into cells. These common and life-threatening diseases include cystic fibrosis, hemophilia, sickle cell anemia, and diabetes. Scientific technology has developed safe and efficient means to transfer genes into cells. Consequently, genetic and molecular delineation of the underlying pathophysiology of many of the primary immunodeficiency disorders has occurred, and gene-based therapy is now a viable option as long as the transferred genetic material can be delivered to the appropriate target cell or tissue. **Controversial ethical considerations over genetic intervention of germ line cells have fostered bioengineering to focus on gene therapy of somatic cells.** Because somatic cells are end-stage differentiated cells, research has examined the use of a self-renewing stem cell population for therapeutic transfer of genetic material. Stem cells can renew themselves, and the inserted gene will remain in place through subsequent generations of differentiated cells or tissue populations. As an example, a patient's cells (e.g., T lymphocytes) are harvested and grown in the laboratory. The cells receive the gene from a viral carrier (e.g., Moloney murine leukemia virus) and start to produce the missing protein necessary to correct the deficiency. These cells with the extra functional gene are then returned to the patient, and the normal protein is produced and released, alleviating the disease. **The genetic cause of numerous primary immunodeficiency disorders has been discovered and described.** As a result, gene therapy can now be used as an alternative therapy, particularly in patients for whom bone marrow transplantation may not be suitable (e.g., a bone marrow donor cannot be identified, or preparation for transplantation carries substantial risk to the patient).