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 com- mon 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 delin- eation of the underlying pathophysiology of many of the primary immunodeficiency dis orders has occurred, and gene-based therapy is now a viable option as long as the trans- ferred 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 ther- apy 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 differen- tiated cells or tissue populations. As an example, a patient's cells (e.g., T lym- phocytes) are harvested and grown in the lab- oratory. The cells receive the gene from a viral carrier (e.g., Moloney murine leukemia virus) and start to produce the missing protein neces- sary to correct the deficiency. These cells with the extra functional gene are then returned to the patient, and the normal protein is pro- duced and released, alleviating the disease. The genetic cause of numerous primary immunodeficiency disorders has been dis- covered 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 transplanta- tion carries substantial risk to the patient).

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