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## **Abstract**

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### **“The Irrefutable Success of Gene Transfer for Therapy of Human Disease”**

It has been approximately 30 years since the concepts of gene transfer for therapy of human disease initially emerged. As is typical for so many areas of biomedical innovation, delivery of the underlying concepts to effective clinical application has come through a combination of initial insight, incremental advances and proof of principle studies together with clinical failures and setbacks. Nevertheless, there is no remaining doubt that the introduction into humans of additional genetic material to provide the function of disease-causing endogenous genes has finally provided truly effective therapy, perhaps even cures, for several human disorders, most notably several inborn errors of immune function. Very promising clinical results are also being obtained after the introduction of foreign genes in other more common disorders such as some forms of cancer, cardiovascular disease, neurodegenerative disease and others. Furthermore, techniques are emerging for the even more definitive chemical correction of the mutations responsible for some human disorders. Human gene therapy therefore has moved from theory to true clinical research that will almost certainly lead to increasingly effective treatments for many of our society's most serious human diseases.