

Public Lecture by Theodore Friedmann, M.D.
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“Success of Gene Therapy”
Wednesday, September 14, 2005 at 7:00 p.m. in the Garren Auditorium, Basic Science
Building
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Treatment of many human diseases is difficult and often ineffective, at least partly because their causes are unknown and because the existing treatments are aimed at symptoms rather than the underlying causes. Most human diseases have complex causes that include both genetic factors as well as environmental influences. With the explosion of knowledge of human genetics during the past several decades, the genetic aspects of much of human diseases has come to be better understood and nay of genes responsible for disease have been identified, isolated and studied.

It has become apparent that recognition of the genetic influences in many of our society’s most common diseases such as cancer, Alzheimer’s and Parkinson’s diseases, heart disease, arthritis, infectious diseases such as AIDS and many others has opened doors not only to improved concepts of drug treatment but also to completely new kinds of treatments that are aimed not at the results of the disease but rather at their causes. In the late 1960s and early 1970s, it became apparent to some investigators that if a specific gene is recognized to be important in the development of a disorder, a uniquely new and definitive kind of therapy might be possible by replacing the defective form of the disease-causing gene with normal copies of the gene; i.e., gene therapy.

Since 1990, many hundreds of clinical studies have been carried that have slowly but surely given credibility to this concept, despite the difficulty of the science and a number of severe scientific and procedural setbacks. During the past few years, gene therapy has moved from an attractive but unproven concept to real therapy for a number of diseases. Children with at least three different kinds of genetically-caused immune deficiencies have been treated with disabled and inactivated viruses armed with normal copies of their disease-causing genes and have responded with a complete restoration of their immune systems, completely normalized childhood lives, some for as long as 6 years. But the price for this advance has been high, because at least in one study, several of the children have developed leukemia as a direct result of the treatment. In that regard, gene therapy is not different from most other complex new therapies like cancer chemotherapy, organ transplantation – such important advances usually carry many dangers and risks, some even life-threatening.

The field of gene therapy has now become a clinical reality, although still a very young one - still inefficient and full of technical and conceptual problems. Fortunately, many new approaches are in the wings, some of which will make gene therapy of many more diseases and disabilities like cancer, neurological disease, some kinds of blindness and many others feasible and more effective, but some of which will raise important and even intractable social and ethical problems of what human non-disease traits we choose to modify. The solution to these difficulties lies in both better science but also in more enlightened public and vigorous public discussion.