Cancer Gene Therapy—Fantasy or Foresight?

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Abstract

Gene therapy is an exciting new method of treatment that may have far-reaching implications on the way we manage diseases in the future. Cancer has become the principal focus of this futuristic research. The breathtaking pace of gene discovery in the last two decades, coupled with the birth of recombinant DNA technology, gave rise to the concept that genes may be manipulated and used as drugs. Genetic modification of cells can be carried out in petri dishes (ex vivo) or within the living system (in vivo). In order for the therapeutic genes to exert their effect, they have to be transported into the cell nucleus where transcription takes place. Liposomes and genetically-modified viruses have been extensively used as gene vectors. The ideal vector remains elusive. It would be one that can achieve tumour-specific, sustained, and regulatable gene expression without host toxicity. As a result of the past decade of intense gene therapy research, we have learned that it is a rational scientific concept that works remarkably well in petri dishes and in laboratory animals. However, early clinical gene therapy experimentations paled in comparison. This apparent disparity between dramatic preclinical successes and the very modest clinical results of gene therapy does not in anyway nullify the concept of gene therapy. Instead, it exposes the folly of underestimating the technical complexity of gene manipulation in human diseases. Fortunately, technical hurdles such as those confronting gene therapy today are not insurmountable; they need, however, much ingenuity, resolution and time to be overcome. It is reassuring that recent advances in gene therapy provide abundant evidence that the premature infant, born of unrealistic pressure, is indeed healthy and thriving. With proper nurturing and patience, there is no doubt that, in time, it will bear fruit.

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