



Keywords

遺伝子治療
遺伝子導入
ウイルスベクター
挿入変異
ゲノム編集

遺伝子治療はどこまで進んだか — 遺伝子治療の歴史と最近の動向 —

History and Present Status of Gene Therapy

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Summary

Gene therapy is an innovative medical technology to treat intractable diseases such as genetic diseases and cancer by introducing new genes into the patients. The first clinical trial of gene therapy was conducted in 1990 at the NIH to treat a girl with severe combined immunodeficiency. Since then, nearly 2,000 clinical trials have been conducted in the world. In the early stage, it was difficult to show the clinical efficacy, mainly because of the inefficiency of gene delivery. The first real success of gene therapy was reported by French group in 2000. They clearly demonstrated that severe combined immunodeficiency can be treated by stem cell gene therapy. This clinical study was a milestone in the history of gene therapy, but unfortunately some of treated patients developed leukemia due to insertional mutagenesis of integrated viral vector. In the 2,000s, various new vector systems were developed to increase the gene transfer efficiency and minimize the risk of pathogenesis. Recently, clinical successes of gene therapy for various diseases have been reported. Gene therapy entered in the new era of becoming a practical medicine.

はじめに

遠い将来の夢の治療法と思われていた遺伝子治療は、1970年代に組換えDNA技術が発展したことで現実のものとなった。ウイルスベクターが開発され、倫理

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