Seminar

医学生命科学セミナー 兼 TR セミナー

Medical and Life Science Seminar & TR Seminar

Gene Therapy: Current Status and Future Directions

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日時：２０１５年８月７日（金）１７：３０～１８：３０
17:30-18:30 August 7th (Fri), 2015

会場：医学教育図書棟３階 第２講義室
Med. Edu. Lib. Bldg., 3rd FL., Lecture Room 2

After the occurrence of leukemia owing to insertional mutagenesis following hematopoietic stem cell gene therapy for severe immunodeficiencies using retroviral vectors, clinical trials of gene therapy remained stagnant for many years. However, clinical gene therapy has been revived because a number of successful clinical trials have been reported recently. For example, gene therapy of Leber's congenital amaurosis, Parkinson's disease, and hemophilia B, showed adeno-associated virus (AAV) vectors were safe and clinically effective gene delivery vehicles. AAV vectors can transduce non-dividing, terminally differentiated cells efficiently, and long-term transgene expression can be obtained after single gene transfer. Another superior gene delivery vector is the lentiviral vector, which was successfully used for hematopoietic stem cell gene therapy of X-linked adrenoleukodystrophy. Even in the case of classical hematopoietic stem cell gene therapy of severe immunodeficiencies, safety was improved using self-inactivating retroviral vectors. Regarding cancer gene therapy, targeting technology is important to improve its efficacy. In this regard, there has been increasing focus on chimeric antigen receptor (CAR) technology for gene therapy of hematological malignancies. Recent clinical trials of CD19-targeted CAR-T gene therapy revealed this therapeutic strategy is promising for the treatment of relapsed/refractory B cell malignancies, including chronic lymphocytic leukemia (CLL), acute lymphoblastic leukemia (ALL), and non-Hodgkin lymphoma (NHL).

Clinical gene therapy was a lively and growing field in the 1990s, but became stagnant in the 2000s. However, it is now undergoing revival in the 2010s as the innovative treatments for intractable diseases.

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