

Adeno-associated vector for gene therapy

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医学教育図書棟 3 階 第 2 講義室

Abstract

Since 1989, nearly 2,000 clinical gene therapy trials have been approved worldwide. Initial applications employed retrovirus vectors targeting the lymphohematopoietic system, and adenovirus vectors followed to tackle intractable cancer. Naked DNA's are also used, mostly for cardiovascular diseases. Vectors derived from adeno-associated virus (AAV) consist 'the third wave' of viral vehicles for gene transfer, and are highlighted for the recent success in treating hemophilia B and inherited retinal blindness. AAVs are peculiar small, nonpathogenic viruses requiring helper viruses (e.g. adenovirus or herpes virus) to proliferate. Our institution has pioneered in developing AAV vectors since mid-1990's, and accomplished a phase I/II clinical trial for Parkinson disease successfully. This seminar will overview the history and the current status of gene therapy with AAV vectors, and focus on our preclinical study for phenylketonuria, a well-known inborn error of metabolism.

Inviter: Prof. Takumi Era (Cell Modulation)

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