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Session Topic : Gene Therapy / RNA Therapy

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## **Tailoring AAV Capsid Proteins for Diverse and Efficient Gene Therapy**

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In numerous preclinical research, remarkable therapeutic results have been documented using gene therapy approaches for the regeneration of bone, cartilage, and nerve tissue. In particular, numerous gene delivery vectors, including as nanoparticles and recombinant viruses, have been developed for the purpose of central nervous system (CNS) gene therapy and tissue engineering. However, their clinical applicability has been constrained by the inadequate delivery of genes to CNS target cells and tissues, mainly caused by the poor penetration of vectors through blood-brain barrier (BBB). Among these vectors, adeno-associated virus (AAV) is a preferred gene delivery vector owing to its safety profile, long-lasting expression in non-dividing cells, and relatively low immunogenicity compared with other viral vectors. However, the gene transfer efficacy of AAV to CNS tissues after intravenous injection is at least an order magnitude lower than to liver, which poses a significant challenge in CNS gene therapy. Therefore, we aimed to develop novel AAV capsid variants with greater specificity to desired CNS tissues to enhance the gene transfer efficacy and minimize the unwanted transduction in other tissues including liver.

**Keywords:** Adeno-associated virus (AAV), BBB penetration, CNS gene therapy, Directed evolution, nerve regeneration