Gene targeting within stem cells using directly evolved adeno-associated viral vectors

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Delivery of genes encoding molecules capable of regulating stem cell function can serve as an effective means to both investigate stem cell biology and to control cell fate for therapeutic applications. Additionally, gene delivery coupled with gene targeting has the potential to introduce mutations, and thereby generate disease models, as well as to correct deleterious genetic mutations in stem cell populations. However, a major obstacle to such applications continues to be the development of efficient and safe gene delivery vectors. Adeno-associated viral (AAV) vectors, which are being broadly explored in clinical trials, have significant promise as therapeutic vectors due to their safety and delivery efficiency, as well as their potential for gene targeting. In this presentation, a powerful tool (i.e. directed evolution) to create de novo bio-inspired nanoparticles (i.e., AAV vectors) that can significantly enhance the capabilities of gene delivery as well as gene targeting within stem cells will be introduced, and a variety of potential applications using gene-targeted stem cells will be discussed.