

2nd International Conference on Pharmaceutics & Novel Drug Delivery Systems

20-22 February 2012 San Francisco Airport Marriott Waterfront, USA

TITLE

Gene and Stem Cell Based Protein Drug Delivery

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Protein and large peptide drug delivery is often limited by production, purification formulation and host immune response. Gene and stem cell based protein drug delivery may provide an alternative to overcome these limitations. We have developed recombinant adeno-associated virus vectors (rAAVs) expressing therapeutic genes, such as alpha-1 antitrypsin (AAT), IL-10, and IL-4. We have shown that rAAV vectors can mediate greater than 1 mg/ml levels of AAT in the circulation by a single IM, IP or portal vein injection of the vector. We have tested these vectors and shown therapeutic effect for type 1 diabetes and rheumatoid arthritis in animal models. One of our vectors is currently used for clinical studies. We have also investigated host immune response to the transgene product from rAAV vectors, and shown rAAV1 induces strong host immune responses while rAAV8 leads to a transgene specific tolerance. These results imply distinct applications of rAAV vectors for the development of positive or negative vaccines. To avoid the unwanted vector distribution, we have tested the feasibility of stem cell mediated gene delivery, and shown that adult stem cells can be used for liver gene delivery and regeneration. In conclusion, rAAV is a safe and effective vector for protein drug delivery. rAAV vector can also be used for adult stem cell based protein drug delivery to the target organs.

Biography

Sihong Song received his Ph.D in 1996 and completed postdoctoral training in 1999 from University of Florida. He is an associate professor in the Department of Pharmaceutics at University of Florida College of Pharmacy. He has published more than 40 papers in reputed journals and serving as an editorial board member of Journal of Drug Metabolism & Toxicology and Journal of Cell Science & Therapy.