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Development of gene and stem cell based therapies for the treatment of alpha 1 antitrypsin deficiency

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Genetic diseases such as alpha-1 antitrypsin (AAT) deficiency, hemophilia, and cystic fibrosis are some of the major health problems affecting many people's lives worldwide. Although recent advances of DNA sequencing technology and bioinformatics have greatly enhanced the understanding of pathogeneses of genetic disorders, treatments of these diseases are challenging since they require genetic modification. Gene and stem cell mediated therapies have been developed and some of them have recently shown impressive successes in clinical studies. Our research has been focusing on the development of gene and stem cell-base therapies for the treatment of AAT deficiency, which mostly results from signal nucleotide mutation (PiZZ) and can lead to emphysema and liver cirrhosis. Preclinical studies have shown that long-term and therapeutic levels of hAAT can be achieved by a single injection of a recombinant adeno-associated virus (rAAV) vector in mouse models. Phase I clinical studies have proven the safety of rAAV vector mediated muscle gene delivery of AAT. Phase II clinical studies are currently ongoing. For the treatment of liver disease associated with mutant AAT accumulation, several strategies have recently been developed and tested in animal models. These include liver regeneration by the transplantations of healthy hepatocytes, genetically modified liver progenitor cells, bone marrow cells, adipose-tissue derived mesenchymal stem cell and iPS derived hepatocytes. In conclusion, recent advances of gene therapy and stem cell-based therapy have provided hope of a cure for alpha 1 antitrypsin deficiency.

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 peer-reviewed papers in gene and stem cell therapy and has been serving as an editorial board member of 2 scientific journals.

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