

2nd World Congress on
MOLECULAR GENETICS AND GENE THERAPY
July 03-04, 2017 Bangkok, Thailand



Guangping Gao

University of Massachusetts Medical School, USA

Human gene therapy - The road to today and beyond

Since the first proof-of-concept human application in the early 90's, gene therapy, particularly viral vector-mediated gene therapy, has now entered a stage of unprecedented revolution in clinical translation and commercialization. Hundreds of clinical trials have been done or are under way, hundreds of gene therapy companies established and went to IPO worldwide. To date three gene therapy drugs have been commercialized: Two in China and one in Europe, and several others are in the process of final approval by FDA in the U.S. Gene therapy can be accomplished through *in vivo* and *ex vivo* approaches by gene replacement for loss-of-function genetic diseases, gene silencing for gain-of-function genetic disorders, gene editing for any genetic diseases and gene addition for treating acquired infectious diseases or cancer. A major challenge in gene therapy is how to efficiently and safely deliver the therapeutic gene to the tissue and cell types needed and make it work as long as possible, ideally accomplishing lifetime gene correction by a single dose. The vehicle to deliver the gene payload, called vector, is the key element for gene therapy. The progress of human gene therapy in the past decades has been primarily driven by vector development. Among all different vectors available for gene therapy to date, adeno-associated virus (AAV)-based vector stands out for its high efficiency, stability, and low immunogenicity/toxicity, holding great promise for different gene therapy applications. AAV is a common benign residential virus that can persist in primate tissues for life time without integrating into host genomes and causing disease. This presentation will review the key principles, history, current status, main challenges and future directions of human gene therapy. The presentation will showcase discovery and development of novel AAV vectors and examples of AAV gene therapy development for treating inborn metabolic errors, neurodegenerative diseases, infectious diseases and cancer by gene replacement, somatic *in vivo* gene editing, gene silencing and gene addition therapy as well as using rAAV for simple and robust embryonic genome editing to create rodent and nonhuman primate animal models of human diseases.

Biography

Guangping Gao received his Bachelor's degree in Medicine from the West China Medical School of Sichuan University. He completed his PhD training in Molecular Genetics at Florida International University with his work involving the isolation and characterization of the human aspartoacylase gene and the genetic mutations responsible for Canavan disease, a severe form of inherited neurodegenerative diseases. He joined the University of Pennsylvania (UPenn) in 1994 where he has developed his career in Viral Vector Biology for Gene Therapy, served as the Director of Vector Program of Institute for Human Gene Therapy to oversee the vector discovery and development, process development, and vector core and quality control testing.

Guangping.Gao@umassmed.edu