Cell Therapy-2013

OMICS Group Conferences
Accelerating Scientific Discovery

2nd International Conference and Exhibition on
Cell & Gene Therapy

October 23-25, 2013 Holiday Inn Orlando International Airport, USA

Gene therapy for autoimmune diseases: Preclinical and clinical studies

Paul D. Robbins

The Scripps Research Institute, USA

Abstract

Gene therapy approaches for treating autoimmune disorders such as rheumatoid arthritis and type I diabetes have shown significant efficacy in numerous animal models. The effective approaches include *ex vivo* methods involving genetically modified dendritic cells (DC), exosomes derived from genetically modified DC as well as direct, *in vivo* gene transfer of immunomodulatory cytokines to joints and to endogenous islets. We have performed several clinical trials for gene therapy of rheumatoid arthritis using intra-articular injection of genetically modified synovial fibroblasts expressing the interleukin-1 receptor antagonist protein (IL-1Ra). Currently we are developing adeno-associated virus (AAV)-based approaches for treating osteoarthritis by intra-articular gene transfer of IL-1Ra. The significant pre-clinical and clinical progress made towards developing viable gene therapy approaches for treating autoimmune disorders using rheumatoid arthritis and type 1 diabetes as model diseases will be presented.

Biography

Robbins is a Professor of Metabolism and Aging at The Scripps Research Institute in Jupiter, Florida and an Adjunct Professor of Microbiology and Molecular Genetics at the University of Pittsburgh School of Medicine. Previously he was Director of the Virus Vector Core Facility, Director of Basic Research for the Molecular Medicine Institute and Co-Director of the Paul Wellstone Cooperative Muscular Research Center at the University of Pittsburgh. Dr. Robbins has co-authored over 300 peer-reviewed manuscripts and 170 book chapters and reviews and has edited four books on gene therapy.