Gene Therapy For Renal Diseases And Transplantation Contributions To Nephrology Vol 159

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Gene Therapy For Renal Diseases
Gene therapy is a potential modality for many renal diseases for which we are as yet unable to offer specific treatment. This article reviews the recent data on gene therapy in animal models applicable to human renal diseases and evaluates its efficacy, safety and clinical relevance.

Gene therapy for renal disorders - PubMed
technology, for example hybrid stem cell-gene therapy could promote the gene therapy of renal diseases toward clinical application. Kidney-targeted gene therapy could be an ideal treat-ment for renal diseases because the effect of therapeu-tic molecule is limited in the kidney. The harmful effects to other tissues can presumably be limited. The kidney

Gene therapy in renal diseases - Kidney International
This guidance provides recommendations to stakeholders developing human gene therapy (GT) products 1 for retinal disorders affecting adult and pediatric patients. These disorders vary in etiology ...

Human Gene Therapy for Retinal Disorders | FDA
Gene therapy in renal diseases. Kidney-targeted gene therapy could be an ideal treatment for renal diseases since the therapeutic molecule is limited in the kidney and the systemic effect may be minimized. The technical development of the gene delivery to kidney and the identification of the responsive gene for a particular disease encourage the challenge to hereditary diseases.

Gene therapy in renal diseases - Kidney International
Before gene therapy can be used to treat renal diseases, delivery of therapeutic genes to the kidney must become much more efficient. Jeffrey Rubin, Tien Nguyen, Kari Allen, Katayoun Ayasoufi, and Michael Barry from the Mayo Clinic co-authored an article published in the journal Human Gene Therapy.

Breakthrough in gene therapy to treat kidney diseases ...
Kidney-targeted gene therapy could be an ideal treatment for renal diseases since the therapeutic molecule is limited in the kidney and the systemic effect may be minimized. The technical development of the gene delivery to kidney and the identification of the responsive gene for a particular disease encourage the challenge to hereditary diseases.

Gene therapy in renal diseases - ScienceDirect
Kidney-targeted gene therapy could be an ideal treatment for renal diseases since the therapeutic molecule is limited in the kidney and the systemic effect may be minimized. The technical development of the gene delivery to kidney and the identification of the responsive gene for a particular disease encourage the

Gene therapy in renal diseases.
In the kidney, however, the development of gene therapy modifications to specific renal cells has lagged far behind those in other organ systems. Some positive strides in the past few years provide continued enthusiasm to invest the time and effort in the development of new gene therapy vectors for medical intervention to treat kidney diseases.

Gene therapy research for kidney diseases.
Ninevah Therapeutics (NTX) is a discovery company developing gene therapies for kidney rare diseases. NTX is building a discovery pipeline with gene delivery platform technologies. Our lead program is an AAV-based therapy for genetic nephrotic syndrome (NTX014). NTX is translating NTX014 from discovery to a first-in-human trial.

Gene therapy for kidney rare diseases
Gene therapy vectors based on viruses have been studied over the past 30 years, but so far, limited information has been obtained regarding the use of various gene therapy vectors for genetic modification of renal cells, particularly for pediatric diseases.

Gene therapy to the kidney using viral vectors
They found that one synthetic strain, Anc80, was successful at targeting two cell types involved in causing the irreparable damage that occurs in chronic kidney disease. The researchers also found...

Gene therapy study shows promise for chronic kidney disease
In the case of diseases, which need reconstitution of residential renal cells, such as congenital enzyme deficiency diseases, mesenchymal stem cells should be transplanted, and in contrast, hematopoietic stem cells may be used for gene delivery for diseases, which need foreign cytokines and growth factors, such as glomerulonephritis.

Stem Cell Gene Therapy for Chronic Renal Failure | Bentham ...
In the kidney, however, the development of gene therapy modifications to specific renal cells has lagged far behind those in other organ systems. Some positive strides in the past few years provide continued enthusiasm to invest the time and effort in the development of new gene therapy vectors for medical intervention to treat kidney diseases.

Gene therapy research for kidney diseases | Physiological ...
However, there are many obstacles to be overcome before gene therapy can be tested clinically for renal disorders. In particular, the major challenges include determining how to prolong and control transgene expression or antisense inhibition and how to minimise the adverse effects of viral or nonviral vectors.

Gene therapy for renal disorders: What are the benefits ...
Novartis Gene Therapies is dedicated to developing and commercializing gene therapies for patients and families devastated by rare and life-threatening neurological genetic diseases. AveXis renamed Novartis Gene Therapies, signifying the growing importance of gene therapy to Novartis corporate strategy

Novartis Gene Therapies | Novartis
Gene therapy for kidney disease. Sukhatme VP(1). Author information: (1)Renal Division, Beth Israel Hospital, Boston, USA. This article reviews the field of gene transfer with regard to applications of interest in nephrology. Excellent general reviews of gene transfer concepts and vectors have been recently published; therefore, only the ...

Gene therapy for kidney disease.
Experts at Newcastle University, UK, have shown in a cell model and in a mouse model that gene editing could be used for Joubert syndrome to stop kidney damage in patients who have the CEP290...

Gene editing possible for kidney disease -- ScienceDaily
Gene therapy with adeno-associated virus (AAV) vectors has demonstrated appropriate tropism for target tissues, including the liver, heart and skeletal muscle in animal models for GSD. AAV vectors transduced liver and kidney in GSD Ia and striated muscle in GSD II mice to replace the deficient enzyme in each disease.