A Delivery Method for a Gene Correction Reagent in Mammalian Cells Including Hematopoietic and Other Stem Cells

Published date: Aug. 14, 2019

Technology description

Current State of the Art

Gene therapy involves the use of introducing healthy genes into a patient whose own genes are defective. In theory, the genes introduced will combine with the patient’ s DNA in targeted cells to trigger production of healthy cells. Gene therapy has become a viable medical treatment option for certain diseases. Many current gene therapies use foreign DNA or viral vectors. Applications exist for oncology, cystic fibrosis, Parkinson’ s disease, Huntingon’ s disease, retinal degeneration, pancreatitis, AIDS and sensory disorders such as loss of sight and sound.

Problems with the Current Art

Although some cellular gene therapy products have been approved, only limited human gene therapy products have yet been approved, none by the USFDA and only some in Europe and China. The primary reason for this is that the foreign DNA or viral vectors introduced by the gene therapy create competing pathway uptake, negative immune responses or undesirable gene mutations. Some of the problems with gene therapies include the limited length of time the gene expresses itself, having the viral vector regain its virulence once introduced into a host organism, or finding the most effective method of delivering the gene to target cells.

Application area

The invention can be applied to treat a wide range of single-gene disorders.

Advantages

The invention provides a highly targeted and simplified method of delivery of single-gene disorder gene therapy utilizing a ZFN protein-transferrin ligand conjugate and donor template. This approach avoids the use of viral vectors and foreign DNA, thereby improving cellular uptake, suppression of competing pathways, limited negative immune response and much more effective gene correction.
It is easy to use, which may lead to increased uptake and faster treatment.

Institution

Augusta University

Inventors

Steffen Meiler
Vice Chari for Research
Anesthesiology and Perioperative Medicine
William Dynan
Professor
Radiation Oncology & Biochemistry