Multi-Functional Nanotherapeutics for Targeted Drug and Gene Therapy (2014-027)

Novel Polymeric Micelle Nanoparticle Enables Targeted Drug and Nucleic Acids Delivery

Market Overview

This multi-functional polymeric micelle nanocarriers serve as a platform technology for combinatorial delivery of multiple bioactive molecules for the treatment of pathological diseases or repair of injured tissues. With a growing population of cancer patients and the increasing quest for an effective cure, gene therapy represents a $100 billion cancer treatment market. The most commonly used treatment for cancer patients is chemotherapy. However, the efficacy of anticancer drugs has been limited by their toxic side effects in normal cells and drug resistance acquired by cancer cells. Current gene therapy methods involve nucleic acid therapeutics; however, the naked DNA/RNA used in these treatments are rapidly degraded by DNase/RNase in the blood or cellular lysosome and cannot pass through the cell membrane sufficiently due to its negative charge. To overcome these challenges, Clemson University researchers developed a multi-functional nanoparticle to be used as the drug/gene delivery vehicle for combinatorial therapy. This multi-functional polymeric micelle nanotherapeutic is capable of delivering multiple therapeutic agents within a single formulation and therapeutic intervention.

Technical Summary

This invention consists of polymeric micelle nanoparticles for targeted drug and nucleic acids delivery. Three key features of this successful nanoparticle include: a hydrophobic core that provides a reservoir for loading of the hydrophobic drug, targeting moieties for tissue/cell specific treatment, and a hydrophilic shell that delivers therapeutic nucleic acids. These

Application

Non-viral gene delivery; Tissue and organ specific studies cancer treatment, neural regeneration

Development Stage

Validated Prototype; *In vivo* animal models

Advantages

- Allows for any hydrophobic drugs to be loaded in the nanoparticle core while any therapeutic nucleic acids can be simultaneously loaded in the shell, resulting in effective delivery of drugs and nucleic acid for the combinatorial therapy
- Improves efficiency with lower cytotoxicity in the presence of serum, providing an advantage over commercially available non-viral vectors
- Utilizes non-viral gene delivery, creating a safer delivery mechanism than traditional viral vector delivery

features are achieved by the composition composed of an amphiphilic graft copolymer, poly (lactide-co-glycolide)-graft-polyethylenimine (PLGA-g-PEI) that spontaneously self-assemble in aqueous solution to form micelles. The hydrophobic PLGA core provides a reservoir for loading of hydrophobic drugs with limited water solubility, while the PEI hydrophilic shell can electrostatically bind nucleic acids such as plasmid DNA, antisense oligonucleotides, or siRNAs. The PEI shell contains primary amines to which a variety of targeting ligands can be covalently conjugated. This Clemson University invention allows for the concomitant delivery of chemotherapeutic and MDR knockdown gene, overcoming drug resistance and increasing therapeutic efficiency.

App Type	Country	Serial No.	Patent No.	CURF Ref. No.	Inventors
Utility	United States	14/966,614	NA	2014-027	Jeoung Lee

About the Inventors

Dr. Jeoung Soo Lee

Assistant Professor of Bioengineering at Clemson University

Dr. Jeoung Soo Lee earned a Ph.D. in Medicinal Chemistry/Pharmaceutical Sciences from Pusan National University, Korea where she worked on development of prodrugs and improving the therapeutic efficiency of drugs. Before joining the Clemson faculty, Dr. Lee worked as a postdoctoral research associate at the Center for Controlled Chemical Delivery at the University of Utah. Her research interests focus on the application of nanomaterials to drug and gene delivery, medical diagnostics, and regenerative medicine.

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