Viral Vector Nanocapsule for Targeting Gene Therapy

Tech ID: 23490 / UC Case 2010-502-0

SUMMARY

UCLA researchers in the Department of Chemical and Biomolecular Engineering have developed a novel viral vector nanocapsule that may improve the targeting specificity and efficiency of gene transfer therapeutics.

BACKGROUND

Gene therapy has shown promise in treating many global health threats, including cancer, cardiovascular, autoimmune and infectious diseases. In many gene therapy applications, the ability to target specific tissues and organs with precision and high efficiency is key to success. Yet, precise targeting and efficient gene transfer remain as great challenges to the field and continue to limit widespread use of gene therapy. Some of the hurdles preventing efficient targeting and gene transfer using viral vectors result from the innate features of the vectors themselves; such features include their natural tropism for certain organs, particularly the liver, and a limited ability to genetically modify the viral envelope. In addition, while some modified vectors have shown promise, they have often been marred by immunogenicity issues and an overall lack of therapeutic gene expression. Further, viral based gene therapy protocols have suffered from ineffective cell entry and poor environmental stability in the body. Thus, new approaches that can improve viral vector targeting, entry, and stability in vivo will be vital to expanding the use of gene therapy.

INNOVATION

Dr. Irvin Chen from UCLA's Department of Microbiology, Immunology, and Molecular Genetics (MIMG) and Dr. Yunfeng Lu of the Department of Chemical and Biomolecular Engineering and colleagues have developed a degradable nanocapsule for encapsulation of single viral vectors. The nanocapsule increases the stability of the viral vector and can be chemically modified with targeting agents, such as antibodies or peptide ligands, to confer a diversified and controllable targeting ability to the viral vector. The researchers have demonstrated such versatility through introduction of an RGD peptide to the polymer shell for targeting. **A striking improvement in transduction efficiency into human cells was observed with the inclusion of the targeting peptide**. Overall, the specific technique utilized by the researchers imparts enhanced shielding of the native viral vector's infectivity as compared to existing encapsulation technologies and thereby may mitigate the undesired consequences of vector administration.

APPLICATIONS

- Targeted gene delivery to specific tissues and organs for gene therapy
- Basic research tool for tissue- or organ-specific gene expression

ADVANTAGES

- These encapsulated viral vectors are stable at room temperature and are resistant to serum inactivation in vivo.
- These encapsulated viral vectors can infect dividing and non-dividing cells with high efficiency.
- The thickness and density of the polymer shell are controllable.
- These encapsulated viral vectors are versatile. Since the nanocapsule can be chemically modified in different ways, the viral vector can be engineered to have diversified targets.
- The enscapsulated viral vectors may be tuned to degrade at lower pHs, thereby allowing viral vector release in late endosomal compartments.

CONTACT

UCLA Technology Development Group
ncd@tdg.ucla.edu
tel: 310.794.0558.

INVENTORS

- Chen, Irvin S Y
- Lu, Yunfeng

OTHER INFORMATION

KEYWORDS

Gene therapy, viral vector, targeting, nanocapsule, polymer, gene delivery, protein delivery

CATEGORIZED AS

- **Biotechnology**
  - Health
- **Medical**
  - Delivery Systems
  - Gene Therapy
- **Nanotechnology**
  - NanoBio

RELATED CASES

2010-502-0

STATE OF DEVELOPMENT

The researchers have conducted proof-of-concept studies, where they created encapsulated viral vectors and tested their transduction efficiency on human cell lines in the presence of human serum. Initial findings report that encapsulation shields the native binding ability of the viruses while still enabling cell transduction with high efficiency. The encapsulated viruses also displayed enhanced stability in the presence of human serum, indicating protection of the virus from environmental deactivation.

RELATED MATERIALS

- **Retargeting vesicular stomatitis virus glycoprotein pseudotyped lentiviral vectors with enhanced stability by in situ synthesized polymer shell**. *Hum Gene Ther Methods*. (2013)

PATENT STATUS

Country	Type	Number	Dated	Case
Japan	Issued Patent	6797959	11/20/2020	2010-502
Japan	Issued Patent	6502261	03/29/2019	2010-502
United States Of America	Issued Patent	10,179,112	01/15/2019	2010-502

ADDITIONAL TECHNOLOGIES BY THESE INVENTORS

- Voltage-Responsive Coating for Lithium-Sulfur Battery
- Highly-Stablized Nanocapsules for siRNA Delivery
- Hierarchially Porous Carbon Particles for Electrochemical Applications
- Making Nanostructured Porous Hollow Spheres with Tunable Structure
- Hyperbranched Polyglycerol Encapsulated Proteins for Oral Protein Delivery
- Viral Expression Vector Targeting HIV-1
- A Method Of Making Carbon Coated Oxides As High-Performance Anode Materials

UCLA Technology Development Group
10889 Wilshire Blvd., Suite 920,Los Angeles,CA 90095
tdg.ucla.edu
Tel: 310.794.0558 | Fax: 310.794.0638 | ncd@tdg.ucla.edu