

VIRUS-LIKE PARTICLES THAT CAN DELIVER PROTEINS AND RNA

SUMMARY

The present invention describes novel virus-like particles (VLPs) that are capable of binding to and replicating within a target mammalian cell, including human cells. The claimed VLPs are safer than viral delivery because they are incapable of re-infecting target cells. The National Cancer Institute's Protein Expression Laboratory seeks parties interested in licensing the novel delivery of RNA to mammalian cells using virus-like particles.

REFERENCE NUMBER

E-264-2011

PRODUCT TYPE

- Therapeutics

KEYWORDS

- RNA delivery
- Virus-like particles
- protein expression
- antibody synthesis

COLLABORATION OPPORTUNITY

This invention is available for licensing.

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DESCRIPTION OF TECHNOLOGY

The National Cancer Institute's [Protein Expression Laboratory](#) seeks parties interested in licensing the novel delivery of RNA to mammalian cells using virus-like particles.

Current methods of delivering proteins or RNA to mammalian cells are limited by a lack of target specificity and toxicity, among other shortcomings. NCI researchers have created novel virus-like particles (VLPs) that are capable of binding to and replicating within a target mammalian cell, including human cells. The claimed VLPs are safer than viral delivery because they are incapable of re-infecting target cells. The present VLPs can optionally comprise inhibitory recombinant polynucleotides, such as microRNA, antisense RNA or small hairpin RNA, to down regulate or turn off expression of a particular

gene within the target cell. Alternatively, recombinant polynucleotides packaged within VLPs can comprise a gene encoding a therapeutic protein so as to enable expression of that protein within the target cell. Specifically, VLPs of the invention are composed of an alphavirus replicon that contains a recombinant polynucleotide, a retroviral gag protein, and a fusogenic envelope glycoprotein.

While the claimed VLPs have a variety of applications, therapeutic uses of the VLPs include directing antibody synthesis and converting cancer cells into antigen presenting cells. Additional applications include using VLPs to induce fast (approx. 3-4 hrs) and high levels of protein production in mammalian cells.

POTENTIAL COMMERCIAL APPLICATIONS

- Delivery of microRNA and small hairpin RNA to reduce expression of targeted genes in a human cell
- Delivery of coding RNA for robust expression in mammalian systems
- Direct antibody production by in vivo injection of replicons (no antigen purification)

COMPETITIVE ADVANTAGES

- Obviates the need to use expensive antigen purification for proteins or antigens produced inside target cells
- High level (~million copies per cell) of RNA production/synthesis within target cell
- Fast expression (approx. 3-4 hrs compared to 1-2 days) following VLP introduction into target cells

INVENTOR(S)

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DEVELOPMENT STAGE

- Pre-clinical (in vivo)

PATENT STATUS

- **U.S. Filed:** PCT/US2013/31876 (26 Sept. 2014)
- **Foreign Filed:** Pending for Australia, Europe, and Japan

RELATED TECHNOLOGIES

- [E-010-2008 - Method for Targeted Therapeutic Delivery of Proteins into Cells](#)

THERAPEUTIC AREA

- Cancer/Neoplasm
- Infectious Diseases