Virus-like Particles as Protein Delivery Vehicles

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Research Lead:

Tony Schmitt, Ph.D.
Professor

Department of Veterinary and Biomedical Sciences

Center for Molecular Immunology and Infectious Disease

Huck Institutes of the Life Sciences

College of Agricultural Sciences

Website:

https://pennstate.pure.elsevier.com/en/persons/anthony-paul-schmitt

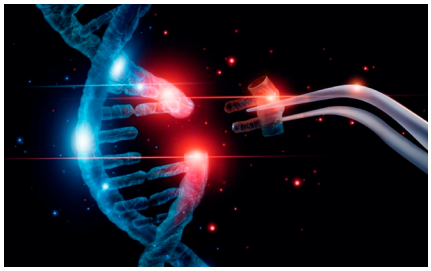
Intellectual Property:

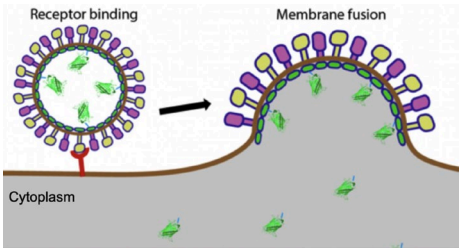
Issued Patents:
- U.S. 10,316,295

Licensing Contact:

Melissa Long

Office of Technology Management
814-865-5730
mklong@psu.edu





Clinical Need:

Over the past decade, advances in genome-editing technology have revolutionized fundamental and applied biology research. Gene therapy has the potential to cure a wide range of diseases including diabetes, cancer, heart disease and AIDS. However, gene delivery is inherently risky and can lead to off-target editing and unintended genome changes.

Value Proposition:

To use virus-like particles (VLPs) for the therapeutic delivery of functional proteins, such as gene-editing nucleases, directly into the cytoplasm or nucleus of a target cell.

Technology Solution:

Virus particles are biological delivery vehicles and are designed to transport viral genomes into infectable target cells. VLP-based delivery vehicles could potentially provide a highly flexible and safe platform for therapeutic delivery of functional proteins to cells. However, foreign proteins do not naturally package into VLPs. Researchers in Penn State's College of Agricultural Sciences recently discovered specific modifications which can be made to foreign proteins that cause them to be recognized during virus assembly and efficiently packaged into paramyxovirus VLPs, which in turn are naturally capable of binding to target cells and delivering the cargo to the cell interior. Using this approach, VLPs loaded with different cargos, including superoxide dismutase and Cas9, can be efficiently delivered to target cells; furthermore, cargos bearing a nuclear localization sequence can successfully transit into target cell nuclei.

Market Opportunity:

As a platform technology, VLPs can be used to deliver a wide range of proteins and peptides, lending themselves to a variety of applications such as gene therapy and antibody delivery. Given that paramyxovirus VLPs bind to sialic acid present on most cell surfaces, *in vivo* treatment of respiratory illness via an inhaled delivery mechanism is a practical first application, with cystic fibrosis being an initial target indication.

Path Forward:

Demonstrate cargo delivery to primary cells, functional delivery of a transcriptional activator to target cell nuclei and functional delivery of Cas9 nuclease to target cell nuclei.