Ultrasound-Sensitive, Fluorine-Based Nanoparticles for Delivery of Therapeutic Proteins

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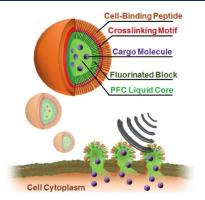
Intellectual Property:

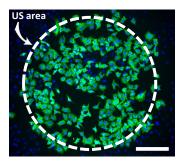
Pending Applications: - U.S.: 16/634,679

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Spatiotemporal intracellular protein delivery

Clinical Need:

All clinically approved protein therapies in oncology act on extracellular disease targets; however, >60% of the human proteome resides within the cell. Current therapeutic protein delivery vectors rely on endosomal uptake and escape to access the cell cytosol and cannot be monitored or guided once administered. Furthermore, targeted, stimuli-responsive methods currently in development are limited to accessible tumor sites.

Value Proposition:

To provide high-precision intracellular delivery of therapeutic proteins, guided and activated on-demand using clinically available ultrasound equipment, with the potential to reduce side effects and improve patient outcomes.

Technology Solution:

Penn State researchers in the College of Engineering have developed an ultrasoundsensitive, fluorine-based nanocarrier for intracellular delivery of proteins with spatiotemporal control. This innovative approach circumvents endocytosis and can be used to deliver a wide variety of proteins, including antibodies and enzymes. In experimental studies, IgG-loaded formulations have been guided and activated in animal tumor tissue in vivo to deliver blocking antibodies that inhibit oncogenic signaling pathways (Ras -> pERK). The technology has the potential to provide targeted delivery in deep tissues and co-delivery of multiple protein biotherapies.

Market Opportunity:

The global nanopharmaceuticals market is forecasted to reach nearly \$50 billion by 2025, expanding at a compound annual growth rate of 7.3%. There are over 230 nanopharmaceuticals that are currently in clinical development, 75% of which are for oncology indications with most being in early stages (i.e., Phase I or Phase II). There is significant potential value in the cancer treatment market for a high-precision, intracellular protein delivery system that can guide delivery of both novel and existing compounds while mitigating off-target effects.

Path Forward:

Continue optimization of material formulations and conduct preclinical safety and efficacy studies using in vivo cancer models. Test efficacy in a monotherapy system and in combination with clinical inhibitors.



PennState Senior Vice President for Research

Biotech Discovery Day



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