Non-Viral Gene Delivery (NVGD) Using Polymeric Nano-Particles

Any Payload, Anywhere, Anytime

Battelle's NVGD platform helps solve the most substantial hurdle to gene editing therapies: delivery. It uses nanoparticles capable of loading at least ten times the five kilobase limit of virus vectors.[1] By combining a robust yet versatile synthesis platform with in vivo tracking and machine learning directed design, it addresses the payload challenge, unlocking high-throughput parallel in vitro and in vivo screening of thousands of nanoparticles.[2,3]

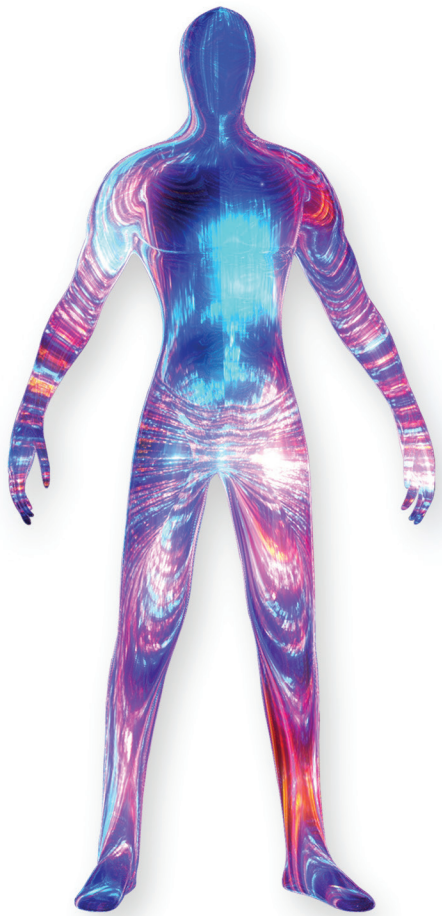
Limitations of Current Delivery Vehicles

Low cargo capacity of virus vectors prevents the delivery of large genes, and next generation gene editors

Immunogenicity of viruses causes hepatotoxicity at doses required for effective gene therapy

Expensive and inefficient manufacturing due to low purity and yield and high-cost separations of cell products

Lack of tissue targeting outside of liver with LNPs, due to their chemical similarity to low density lipoprotein and lack of diversity



Potential of Battelle's Polymer Nanoparticle (PNP) Platform[4]

Can deliver large genes and gene editors, greatly expanding the set of treatable diseases

PNPs can be engineered to evade the immune response due to the diversity of polymer chemistry

Robust polymer synthesis methods can be scaled up to produce a high-yield product at a low cost

Diverse chemistry space for PNPs enables extra-hepatic targeting to a diversity of tissue types beyond LNPs

Our Capabilities

15 PhDs, spanning the fields of chemical engineering, materials science, polymer chemistry, molecular genetics, cell biology, toxicology, immunology, and data science

4 patents covering polymer nanoparticles and other non-viral delivery compositions, and DNA barcoding for polymer nanoparticles

BATTELLE

Bringing the Possibilities of **Genetic Medicine to Everyone**

KEY PARAMETERS	RATIONALE
Payload Capacity	Greater than 15kB loading capacity[5]
Payload Diversity	Nucleic acids and combinations of proteins & nucleic acids[6]
Targetability	Potential to differentially target various tissues[3]
Immunogenicity	Repeat application possible
Manufacturability	High throughput chemical synthesis; fast and predictable

References
[1]Sims et. al. (2021) *Cationic Diblock Copolymer Nanoparticles Improve Intercellular Delivery of Large pDNA Payloads.* [Poster Presentation]. 2021 ASGCT.
[2]Duong et. al. *High-throughput screening of polymer nanoparticles as non-viral delivery vehicles for genetic payloads.* [Poster Presentation]. 2022 Controlled Release Society.
[3]Sims et. al. *Advancing Non-Viral Delivery Vehicle Discovery via Battelle's HIT SCAN Platform.* [Poster Presentation]. 2023 SFB.
[4]Sims et. al. *Advancing NF-1 Schwann Cell Targeted Therapy via Gene Regulatory Protein Development and Non-Viral Delivery Vehicle Discovery.* [Poster Presentation]. 2023 ASGCT.
[5]Gupta et. al. *Cationic Diblock Polymeric Nanoparticles Encapsulate and Delivery a Full-length 20kb cDNA to Neuronal Cells.* [Poster Presentation]. 2023 ASGCT.
[6]Sims et. al. *Cationic Diblock Copolymer Nanoparticles Improve Intercellular Delivery of Large pDNA Payloads.* [Poster Presentation]. 2021 ASGCT.

800.201.2011 | solutions@battelle.org | www.battelle.org

ID 787 06/23

BATTELLE
It can be done