

Create Transformative Medicines $^{^{\text{TM}}}$ 

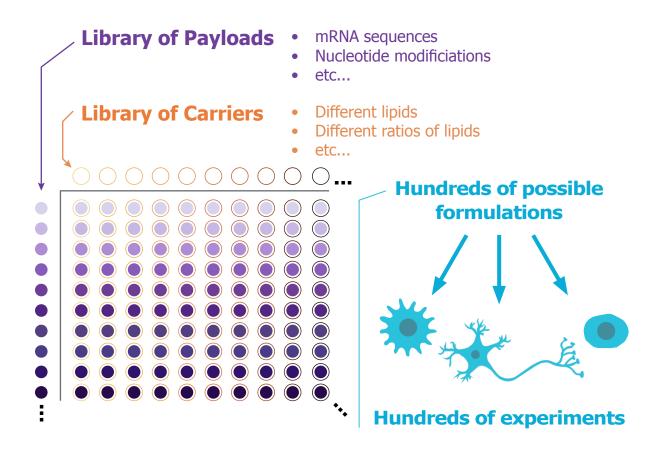
# NanoAssemblr<sup>®</sup> Spark<sup>™</sup>

Freedom to Discover



# **Explore Novel Genomic Medicine Formulations**

Rapid, reliable, small-scale formulations are needed to streamline discovery of revolutionary new medicines.



These materials can be costly or in short supply. Rapid, reliable, small-scale formulations are needed.



# **Accelerate Nanomedicine Development**

# The NanoAssemblr<sup>®</sup> Spark<sup>™</sup> is ideal for nanomedicine formulation discovery and screening at microliter scales



# **Spark provides:**



# UNMATCHED RECOVERY

Advanced microfluidics allows microliter formulation volumes with near complete sample recovery.



### **ROBUST PROCESS**

Electronic control minimizes batch-to-batch and user variability.



# INTUITIVE OPERATION

Simply pipette starting materials into wells, push a button, and pipette completed formulations out.



# WORKFLOW INTEGRATION

The Spark system is designed for operation in a sterile biosafety hood, so formulations can be made on-demand, and applied to cells in culture.



### **RAPID PRODUCTION**

Process requires less than 10 seconds, allowing hundreds of formulations to be made in hours



### **SCALABLE**

NxGen microfluidic mixing technology allows formulations to be scaled across the NanoAssemblr Platform to accelerate future development.

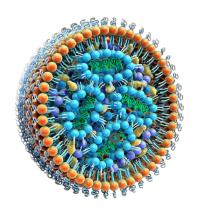
# **Powerful Screening Capabilities**

# **Spotlight: Lipid Nanoparticles (LNPs)**

# LNPs are the most clinically advanced RNA delivery technology

### Inside an LNP

Ionizable cationic lipids contribute to excellent encapsulation and payload release. Excipients, payloads and their relative quantities affect potency.



### **Excipients**

Ionizable Cationic Lipid

Cholesterol

Helper Lipid

PEG-lipid

### **Payload**

MM siRNA

mRNA

Plasmid

# **Screen LNP formulations efficiently with Spark**

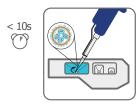
1. Pipette lipid mix and mRNA



2. Insert into Spark and press "Run"

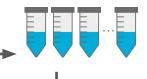


3. Pipette out and dilute



4. Repeat x N

5. N Formulations

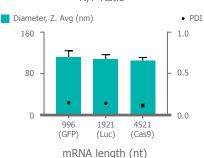


Test and Read out



# Formulate LNPs in seconds using less than 25 µg of mRNA and 1 mg of ionizable lipid

Diameter, Z. Avg (nm) • PDI 1.0 160 0.5 80 N/P Ratio



Encapsulation Efficiency 100 50 N/P Ratio

 Encapsulation Efficiency 100 50 996 1921 4521 (GFP) (Cas9) (Luc)

mRNA length (nt)

N/P ratio is a key factor in performace. Here, mRNA LNPs were rapdily made at 3 N/P ratios with Spark

LNPs encpsulating 3 different mRNAs were made with different mRNA lengths on the Spark with no change in size.

# **Case Study**

# Screening mRNA LNPs in human neurons for neurotherapeutic applications

### **Background**

**Therapeutic paradigm:** Development of a gene-based therapy for neurodegenerative disease applications.

**Formulation:** mRNA encoding a green fluorescent protein (GFP) reporter gene encapsulated in an ionizable, cationic-lipid based nanoparticle.

### Challenge

Human neurons are sensitive, difficult-to-transfect cells and are highly susceptible to harsh gene delivery methods. Small LNP formulation volumes under consistent conditions are ideal for screening novel lipid excipients tailored towards safe and effective neuronal gene delivery.

### Methodology

A panel of LNP formulations were quickly and easily formulated on the NanoAssemblr Spark. Lead candidate formulations were selected based on particle characteristics, the intensity of gene expression and preservation of iPSC-neuron viability.

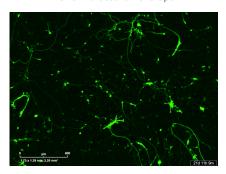
### **Outcomes**

The Spark enabled an accelerated screening workflow to identify mRNA LNP formulations optimal for gene delivery to neurons.

# Panel of LNP formulations Characteristics Lead Candidate Transfection Characteristics LNP encapsulating GFP mRNA Reporter Gene Expression

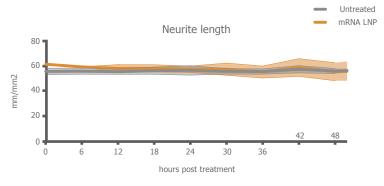
### Efficient gene delivery, robust gene expression

Human iPSC-neurons strongly express the GFP mRNA delivered using mRNA LNPs formulated on the Spark.



### Safe solutions for sensitive cells

Lead mRNA LNP formulations minimally impact neuron health and viability.



The NanoAssemblr Spark accelerates screening workflows for the identification of novel LNPs for biological applications. Lead candidates were rapidly identified as effective nucleic acid delivery systems while preserving the viability of human neurons.

### See the full story online:

www.precisionnanosystems.com/spark

# **Ordering Information**

INSTRUMENT AND CARTRIDGES		PRODUCT CODE	INCLUDES	DESCRIPTION
	NanoAssemblr® Spark™	NIS0001	1 NanoAssemblr® Spark™ Instrument 1 Power supply (worldwide) 1 One-year warranty	Electrical: 100-240 VAC, 0.58 A (Max.) Dimension (WxDxH): 16.5 x 19.5 x 22.5 (cm) Weight: 3.6 kg
	Spark™ Cartridge	NIS0009 NIS0013	20 pack 80 pack	Spark <sup>™</sup> Cartridge is equipped with NxGen <sup>™</sup> microfluidic mixing technology. No cleaning or cleaning validation required: Cartridges are single-use and gamma irradiated for sterility.
REAGENTS		PRODUCT CODE	INCLUDES	DESCRIPTION
	GenVoy-ILM™ T Cell Kit for mRNA	1000701	1 kit	Ionizable lipid mix optimized for the delivery of messenger RNA (mRNA) into activated human primary T cells using LNPs
C MASSINGS	GenVoy-ILM™ T Cell Kit for mRNA with Spark Cartridges	1000683	1 kit 5 cartridges	

If you have any additional questions, please reach out to your regional PNI representative, or email us at <a href="mailto:info@precision-nano.com">info@precision-nano.com</a>

Or go to our website: www.precisionnanosystems.com

## **About Precision NanoSystems**

Precision NanoSystems is a global leader of innovative solutions for the discovery, development, and manufacture of genomic medicine based gene and cell therapies, small molecule and protein-based drugs. Precision NanoSystems ULC is a wholly owned subsidiary of Pall Corporation.

Contact info@precision-nano.com 1-888-618-0031

Headquarters: 50 - 655 West Kent Ave. N., Vancouver, BC, V6P 6T7 Canada

Precision Nanosystems USA: 395 Oyster Point Boulevard, Suite 145 South San Francisco, CA 94080 USA Precision Nanosystems Europe: Two Snowhill Road Birmingham, UK. B4 6WR

Precision Nanosystems APAC: 10 Anson Road #12-14 International Plaza Singapore 079903 Document ID: naspark-BR-0322

For Research Use or Further Manufacturing. Not for direct administration into humans. Copyright © Precision NanoSystems ULC. 2022 All rights reserved. Create Transformative Medicines, NanoAssemblr, NxGen, Spark, and GenVoy-ILM are trademarks of Precision NanoSystems ULC.