delivery platform to turn

nucleic acids into therapeutic solutions

RJH Biosciences Inc.
www.rjhbiosciences.com

RJH BIOSCIENCES

DELIVERY PLATFORM TO TURN
NUCLEIC ACIDS INTO THERAPEUTIC SOLUTIONS

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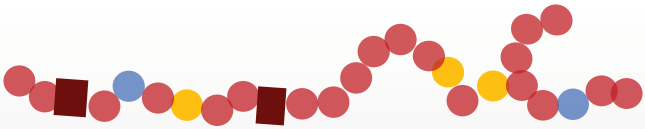
Our platform

lipopolymers for delivering nucleic acids into cells

Combinations of **lipids** and **polymers** for effective delivery of nucleic acids: optimized for size, composition, stability, and architecture.

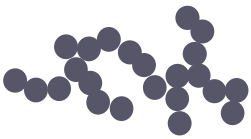


Superior to current delivery vehicles

Viruses

Safer than viruses

Polymers



More effective than polymers

Lipid particles

Easier to manufacture than LNPs

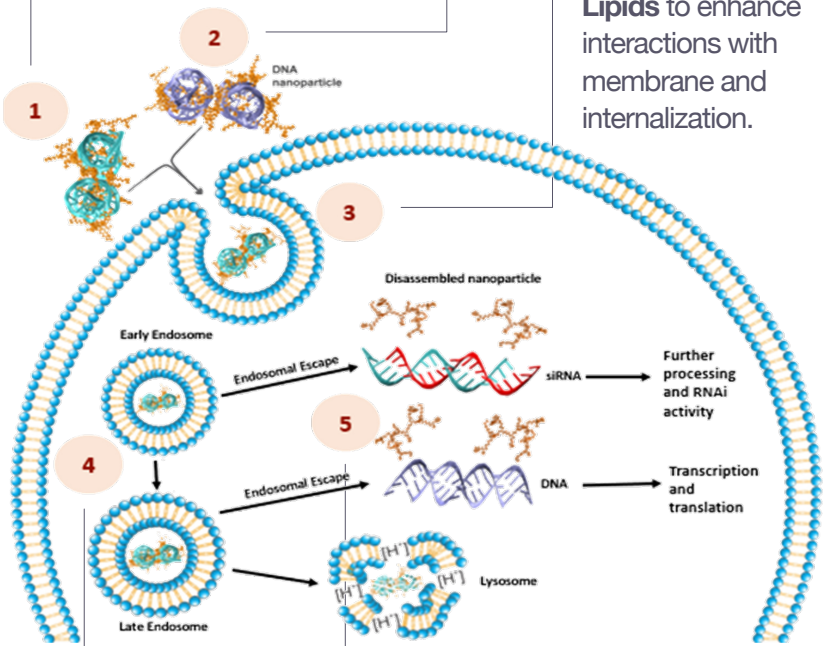
Mechanism of action

for lipopolymer-mediated nucleic acid delivery

Multivalent interactions with nucleic acids for strong binding that withstand disruptive forces during membrane transit.

Synergistic effects of cationic and lipidic binding that coat and protect cargo against nucleases.

Lipids to enhance interactions with membrane and internalization.

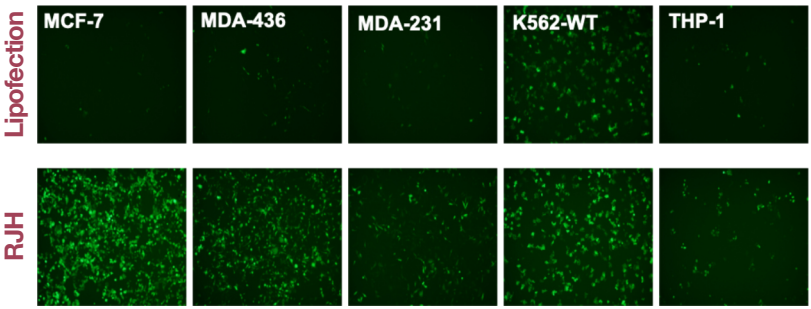


pH buffering that facilitate escape from endosomes.

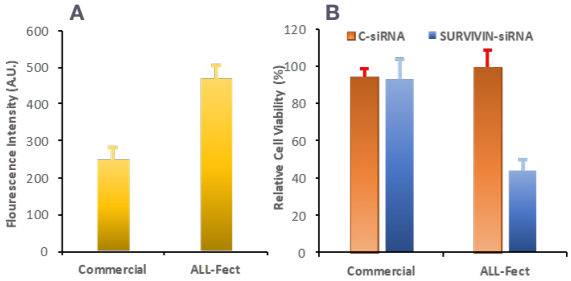
Biodegradable formulations to free nucleic acids in cytoplasm.

Formulations
for different applications

I. Different cell types



II. Different nucleic acids



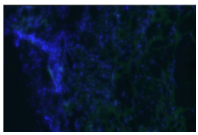
Transfecting attachment-independent K562 cells with plasmid DNA and siRNA using ALL-Fect and a leading lipofection reagent.

A) GFP expression was induced with plasmid DNA and analyzed by flow cytometry 2 days after transfection.

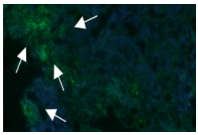
B) Survivin silencing and resultant inhibition of cell growth with specific siRNA 3 days after transfection.

Efficacious delivery
in animal models

I. Expression of reporter GFP gene in implants with pDNA



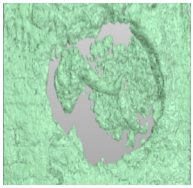
pDNA alone



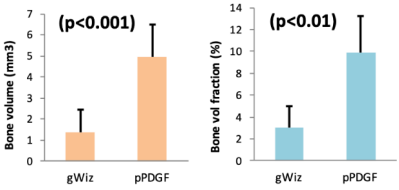
pDNA with RJH Reagent

II. Repair of bone (skull) defects with implantation of pDNA

Control implant

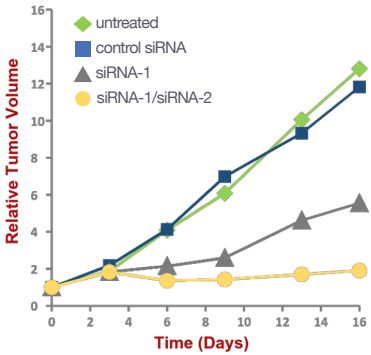


pPDGF implant

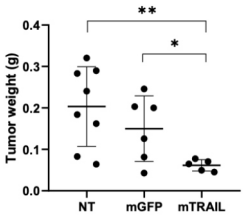


µCT Analysis of Implants (n=5)

III. Inhibition of tumor growth with siRNA injections



IV. Inhibition of tumor growth with local mRNA injections

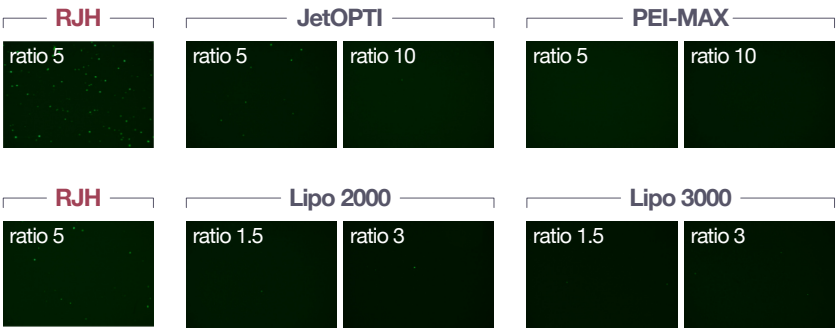


Weight of explanted breast cancer tumors (day 17) for non-treated (NT), GFP and TRAIL expressing mRNAs. * and ** indicate significant differences between TRAIL mRNA and mGFP and NT groups.

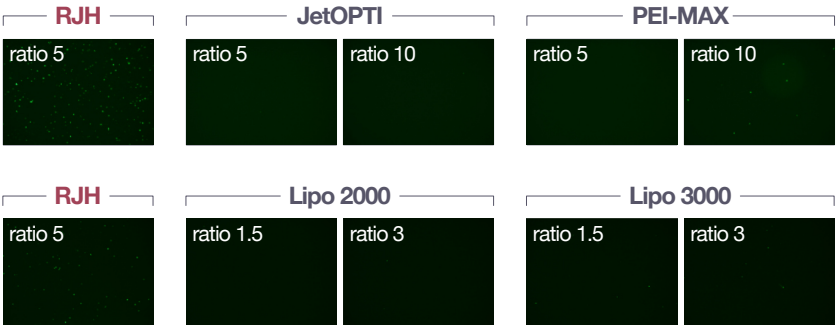
Superior delivery

of mRNA and pDNA in suspension cells

I. pDNA (GFP) delivery



II. mRNA (GFP) delivery

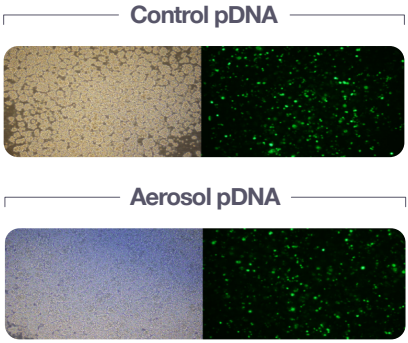


Effective Modification of Human PBMC with pDNA and mRNA

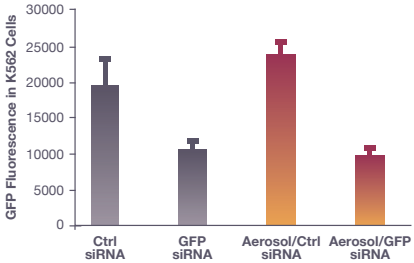
(numbers indicate optimal reagent : nucleic acid w/w ratio)

Formulations
for aerosolized transfection

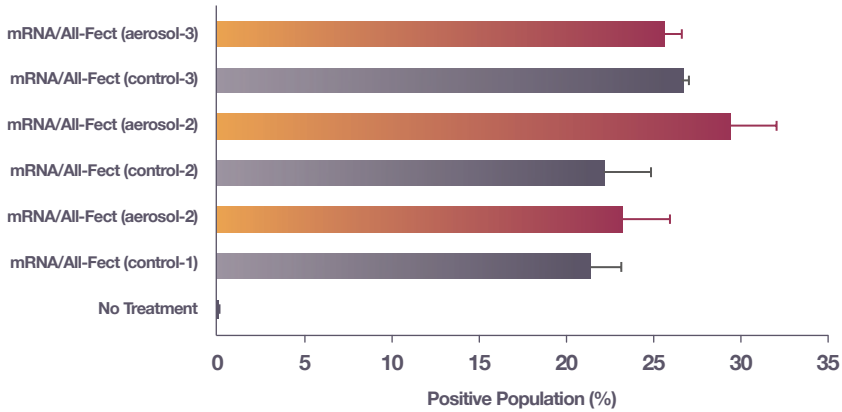
GFP Expression with Aerosolized pDNA Particles



GFP Silencing with Aerosolized siRNA Particles

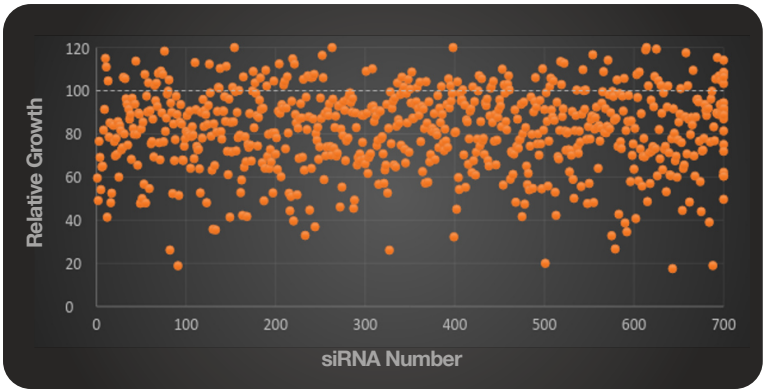


mRNA Delivery with Aerosolized mRNA Particles

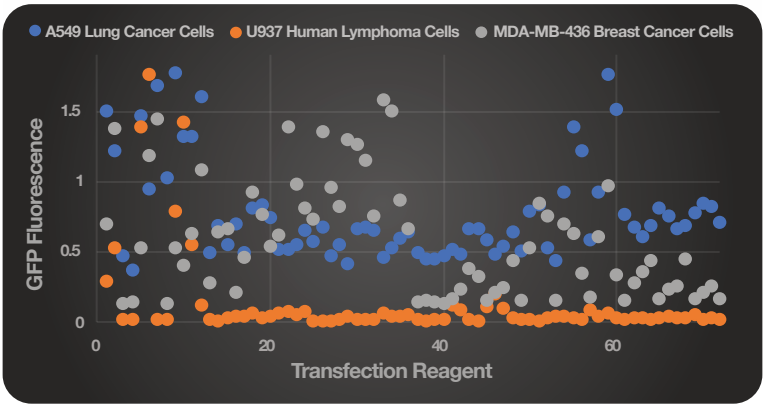


Optimization and development
of reagents through screening services

Screening siRNA libraries with RJH reagents for biomarker discovery

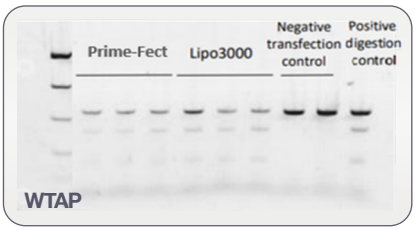


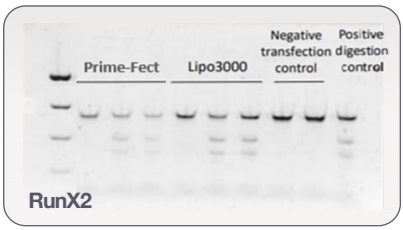
Screening for optimal delivery vehicles in different cell types



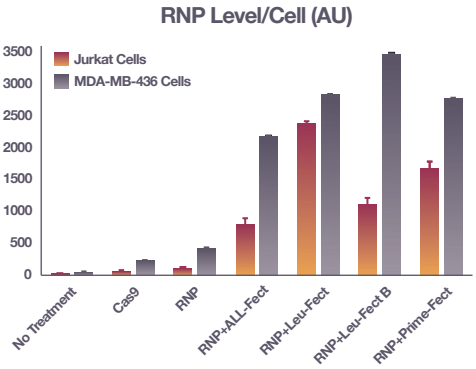
CAS9 delivery
for CRISPR activity

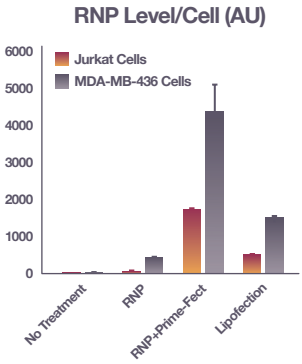
I. Transfection for efficient pDNA-based editing of target genes



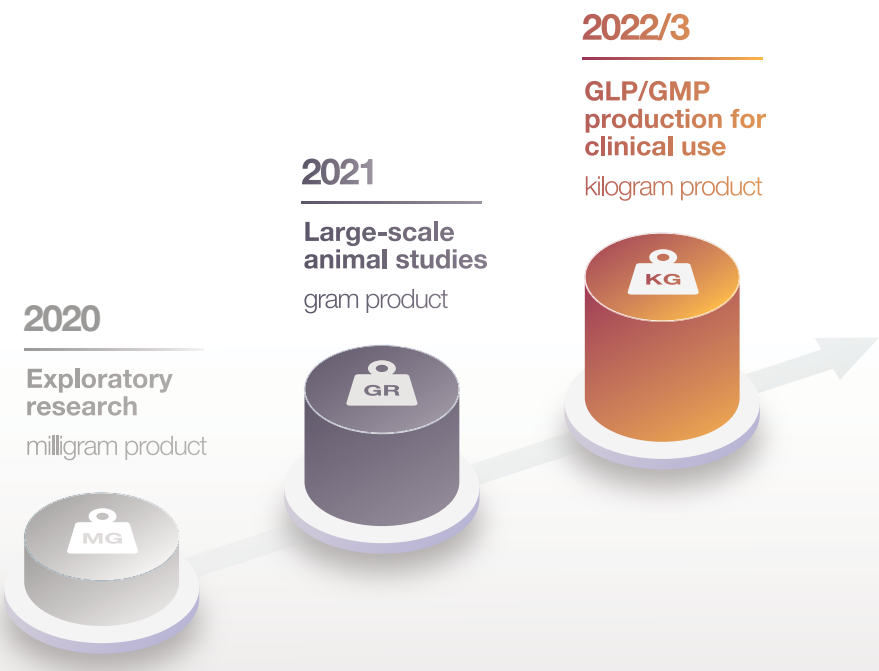


II. Improved delivery of Cas9/sgRNA RNP superior to leading lipofection reagent





RJH lipopolymers
are suitable for large scale production

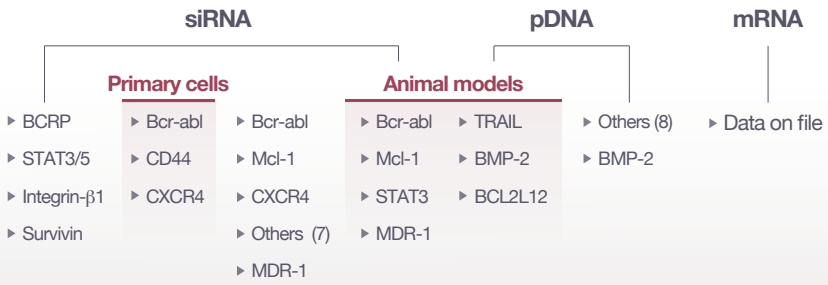


Our products are now produced at a scale suitable for animal studies

Custom transfection reagents produced on demand

Spectrum
of applications

Publication topics with RJH reagents



Cell lines

- MDCK epithelial cells
- 293T kidney fibroblasts
- MDA-231/436, SUM-149PT, MCF-7 breast cancer cells
- AML THP-1, KG1/A, HL60 cells
- ALL NALM-6, MOLM-13 cells
- U937 lymphoma cells
- A549 lung cancer cells
- HCT-116 colon cancer cells
- Immortalized myoblasts

Primary cells

- Mononuclear cells from peripheral blood
- Bone marrow stromal cells
- Skin fibroblasts
- Vascular smooth muscle cells
- Endothelial cells
- Cord blood derived mesenchymal stem cells
- Rat primary sympathetic neurons

Animal models

- Systemic injection of siRNA
- Local injection of siRNA
- Local injection and implantation of pDNA expression vectors
- Local injection of mRNA
- Combinational delivery of multiple nucleic acids

Novel delivery platform

Wide applicability for different nucleic acids

Tailored for specific types of cells, with superb efficacy with blood and immune cells

Seamless Translation to preclinical animal models

v1.1

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