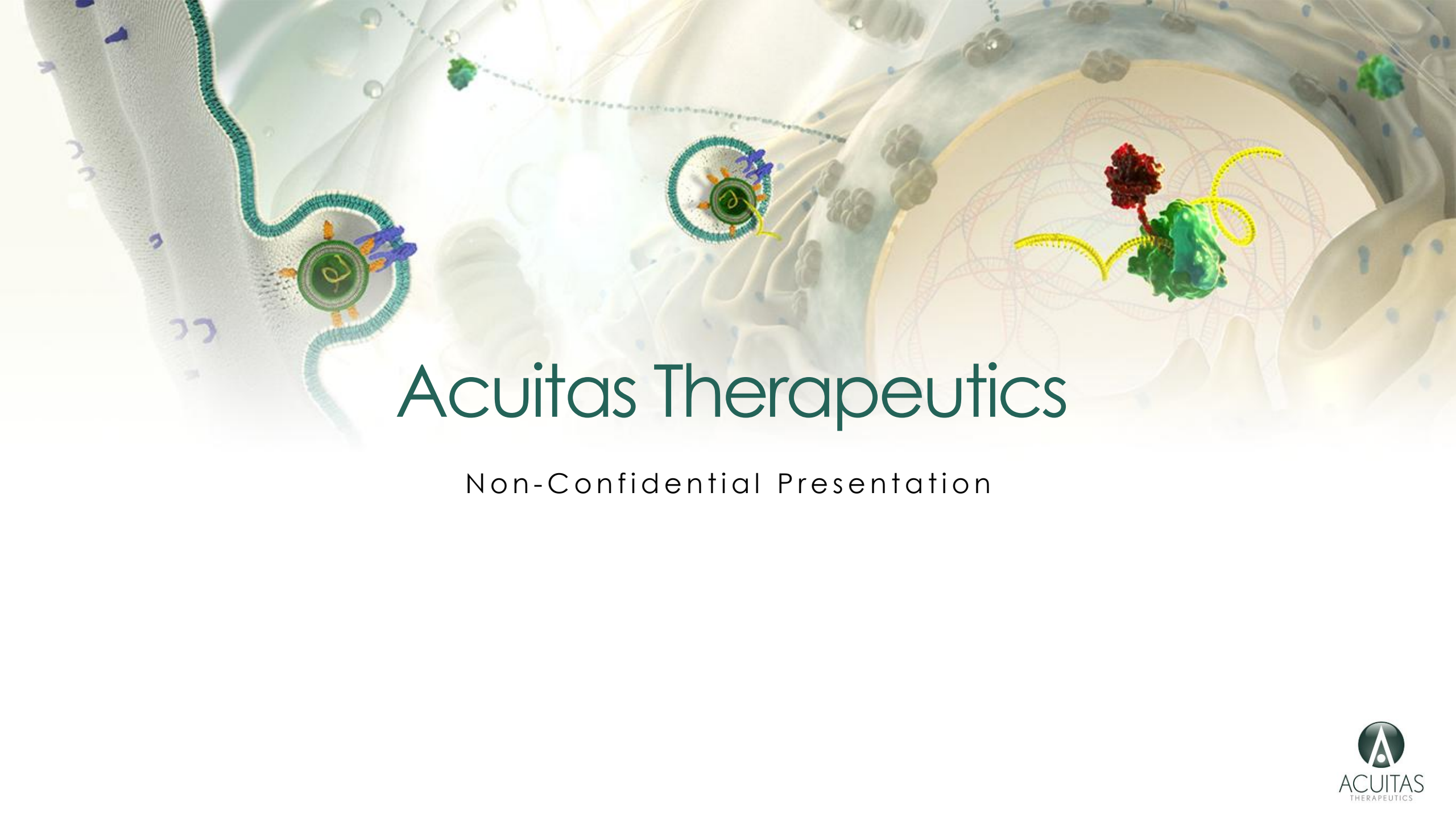




Acuitas Therapeutics

Non-Confidential Presentation

Overview

WHO WE ARE

Acuitas is a globally recognized biotechnology company specializing in the **development of delivery systems for nucleic acid therapeutics based on lipid nanoparticles (LNP).**

Our LNP currently enable two commercial products:

onpattro
(patisiran)
lipid complex injection
10 mg/5 mL

COMIRNATY
(COVID-19 Vaccine, mRNA)

WHO WE WORK WITH

We work with a variety of organizations, including:



Cutting edge **pharmaceutical & biotechnology** companies



Leading **academics in universities & institutes**



Foundations & NGOs

HOW WE WORK

As a technology platform provider, we exclusively work in collaboration with partners.

We do not have our own drug development programs – **we are focused on supporting our partners to bring their drug products to patients.**



Applying Our LNP Technology

Gene Modulation

Expression of an **epigenetic editor** to modify gene expression **without changing the genetic code**.

Antibodies

Expression of **prophylactic or therapeutic antibodies** to treat current and emerging diseases.

Gene Editing

Expression of a **genome editing** protein to modify gene expression.

Vaccines

Expression of viral or bacterial proteins to generate a protective **immune response**.

Expression of **tumour antigens** (including personalized cancer vaccines).

Therapeutic Protein Delivery

Expression of a **human protein** to treat disease.



Our Partners' Success

Products in Clinic

1 1 1 1 1 1 1 1
1 1 1 1 1 1 1 1

16 in Phase 1

2 2 2 2

4 in Phase 2

3 3

2 in Phase 3

Clinical Firsts

onpattro
(patisiran)

Alnylam

First Clinically
Approved **RNA
interference-
based Medicine**

COMIRNATY
(COVID-19 Vaccine, mRNA)

Pfizer BIONTECH

First Clinically Approved
mRNA-based Medicine

First Widely Approved
COVID-19 Vaccine

**Children's Hospital
of Philadelphia**



First **LNP enabled
personalized
CRISPR gene
editing therapy**



Sept 2025: [Precision BioSciences Presents Data from the Phase 1 ELIMINATE-B Trial of PBGENE-HBV at the 6th International Coalition to Eliminate HBV Cure Symposium](#)



Aug 2025: [Beam Therapeutics Provides Update on BEAM-302 Development Progress in Alpha-1 Antitrypsin Deficiency \(AATD\)](#)



July 2025: [Arbor Biotechnologies Announces First Patient Dosed at Mayo Clinic in the redePHine Phase 1/2 Study of ABO-101, an Investigational Gene Editing Treatment for Primary Hyperoxaluria Type 1](#)



May 2025: [Myeloid Therapeutics Unveils First-in-Human In Vivo mRNA CAR Data, Marking a Breakthrough in RNA-Based Immuno-Oncology at the 2025 ASCO](#)



May 2025: [CRMA-1001 an epigenetic editor for the treatment of chronic hepatitis B](#)



Dec 2024: [Tune Therapeutics Moves into Clinical Spotlight with TUNE-401: A First-in-Class Epigenetic Silencer for Hepatitis B](#)



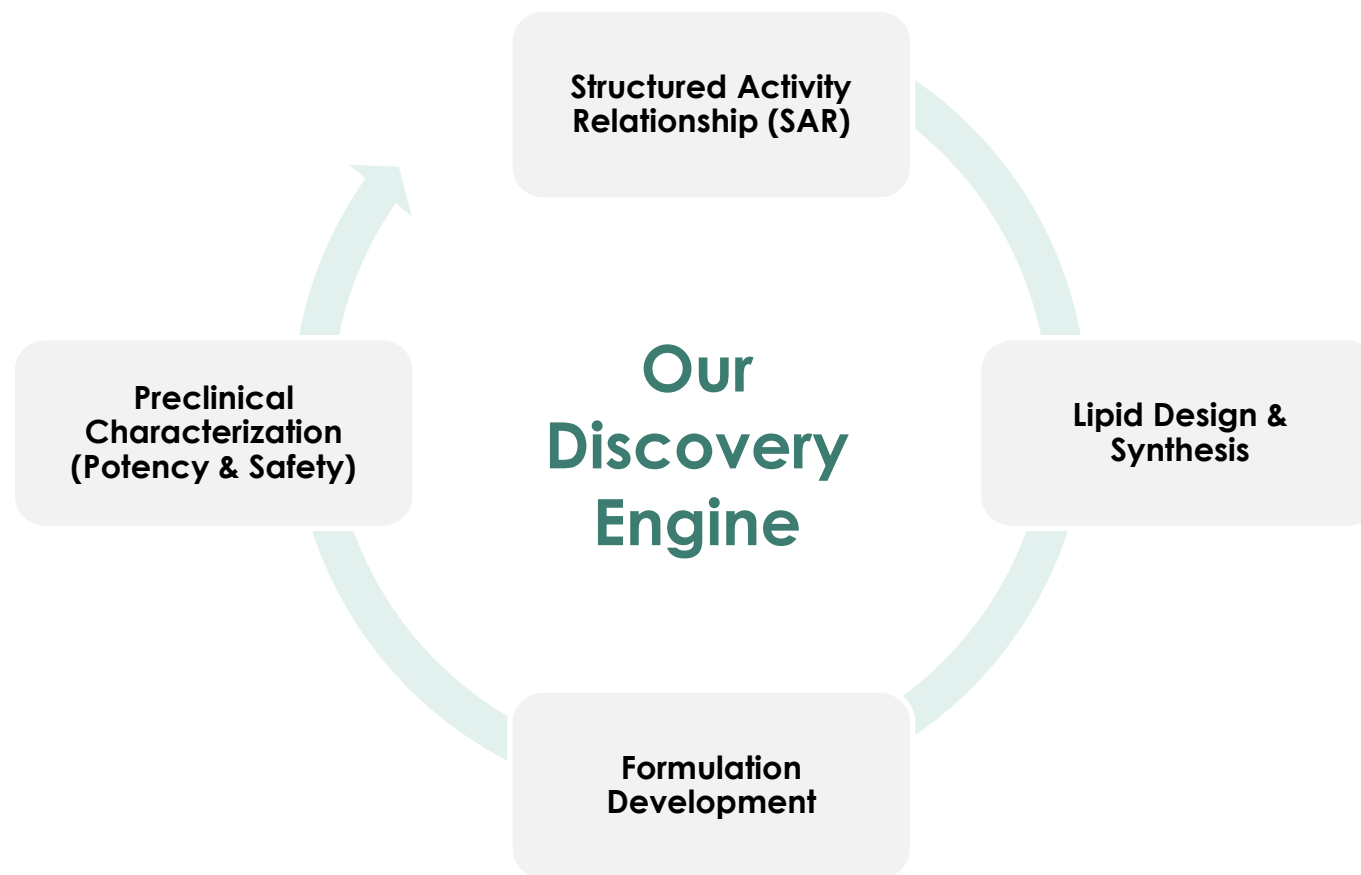
Nov 2024: [Omega Therapeutics Announces Successful Completion of Phase 1 Trial for Novel Epigenomic Controller](#)



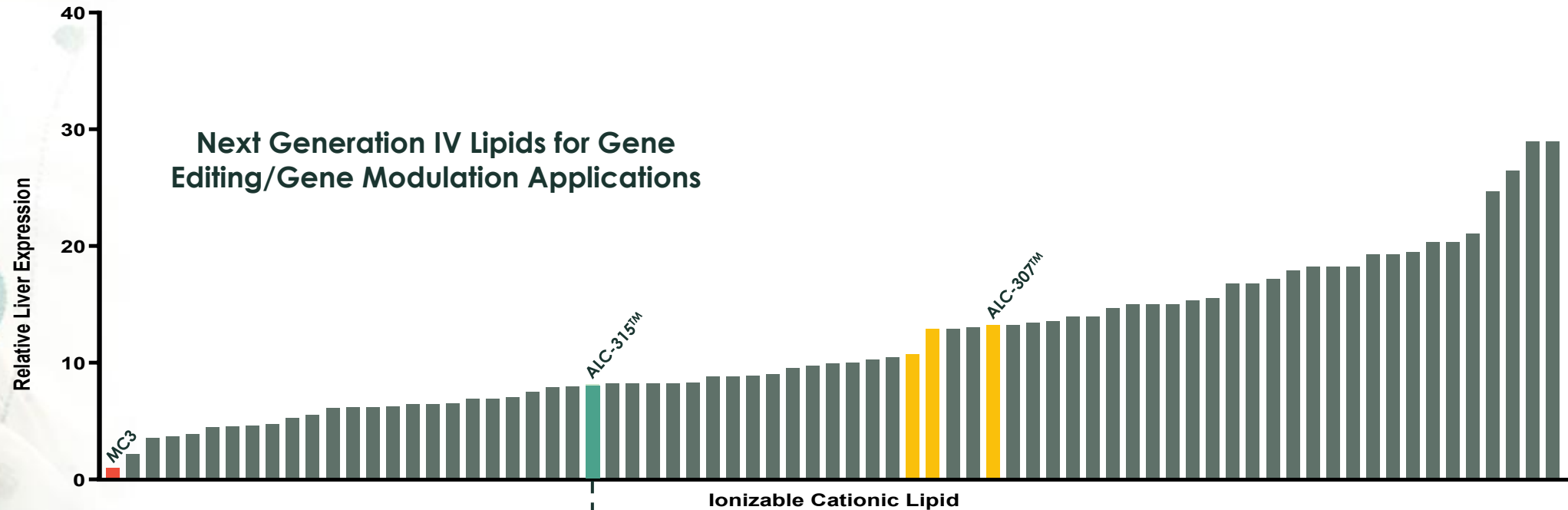
Sept 2024: [CureVac's CVGBM Cancer Vaccine Induces Promising Immune Responses in Phase 1 Study in Glioblastoma Presented at the ESMO 2024 Congress](#)
Sept 2024: [CureVac Partner GSK Announces Positive Phase 2 Data from Seasonal Influenza mRNA Vaccine Program](#)



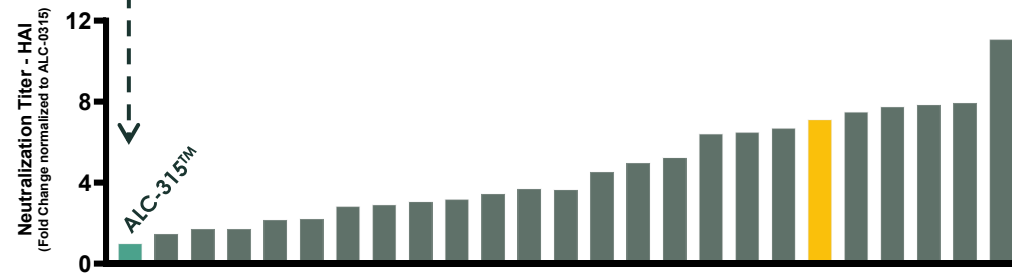
Our Approach to Innovation



Our Approach to Innovation



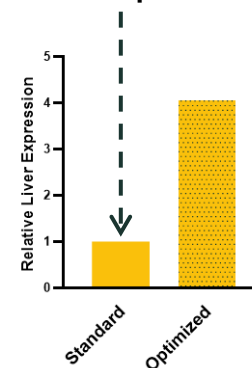
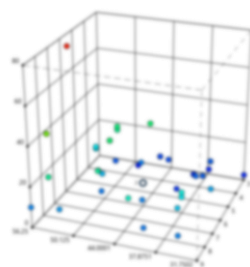
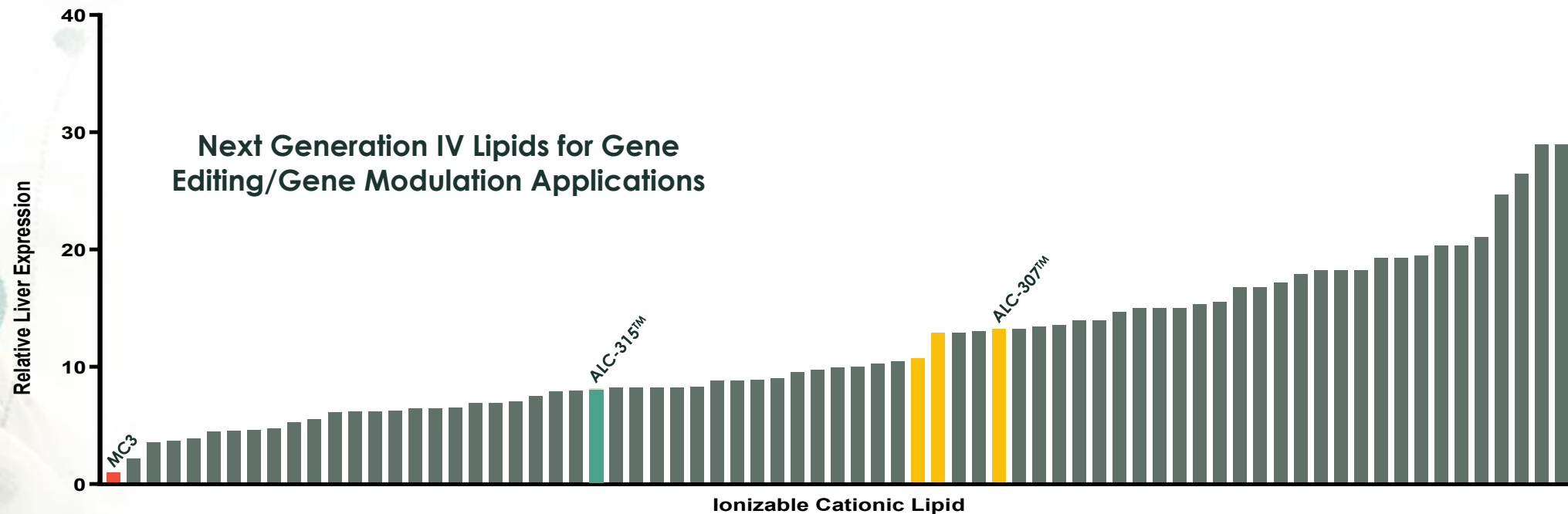
Next Generation IM Lipids for Vaccine Applications



LEGEND

 cGMP ready + in clinic

Continuous Innovation: Formulation Optimization



Formulation optimization
further improves the
potency of our lipids

LEGEND

■ cGMP ready
+ in clinic

■ cGMP ready with
improved formulation

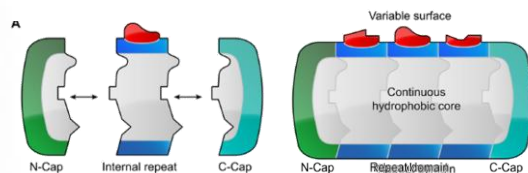
Continuous Innovation: Extrahepatic Targets

OUR APPROACH

Our extrahepatic program is focused on delivery via **cell targets that are directly accessible in the blood compartment or local administration.**

HOW WE DO IT

We use antibody mimetics called **Designed ankyrin repeat proteins (DARPs)** to target any tissue and / or organ with high specificity.



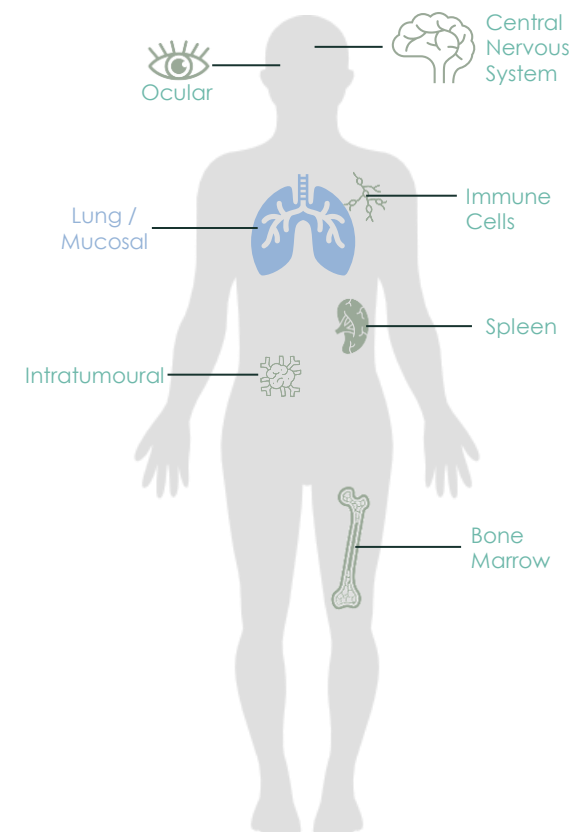
AREAS WE CAN TARGET

Diseases Affecting the Lung
(i.e., CF, Cancer, Infectious Disease etc.)

Mucosal (via aerosolization)

Epigenetic Regulation and Gene Modification

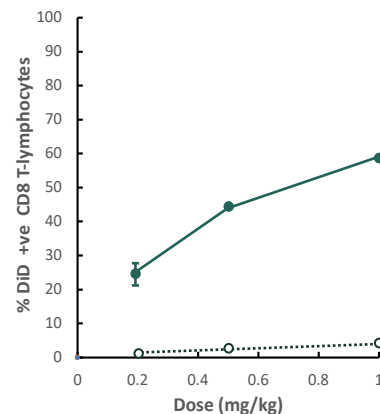
Spleen, Bone Marrow, Ocular, Intratumoural, Immune Cells, Central Nervous System (CNS)



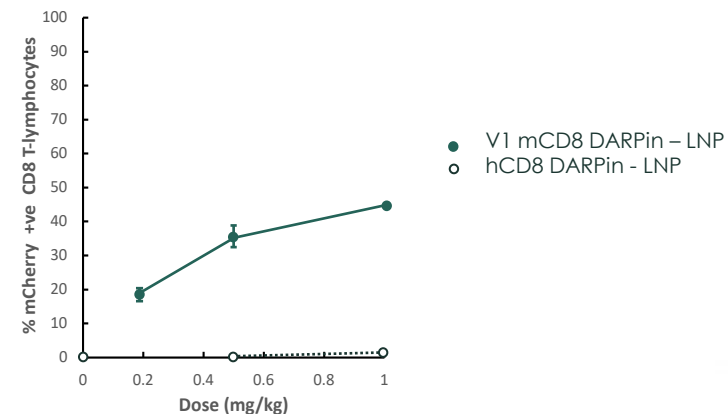
Continuous Innovation: Targeted LNP Delivery

CD8 DARPin targeted mRNA LNP show **dose dependent, target specific binding / uptake and transgene expression.**

LNP Binding / Uptake



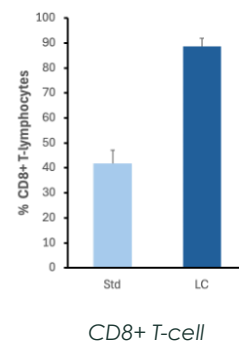
Reporter Gene Expression



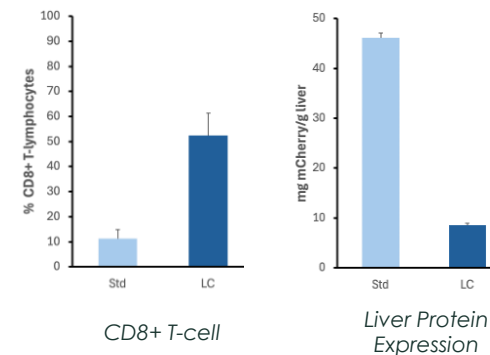
Optimized long circulating LNP results in **increased in binding / uptake and expression.**

Expression in liver is ~5x lower vs. standard LNP.

LNP Binding / Uptake



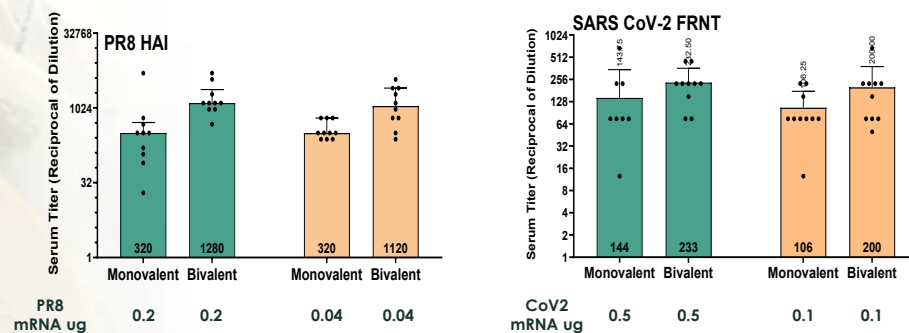
Reporter Gene Expression



Continuous Innovation: Multivalent & Cancer Vaccines

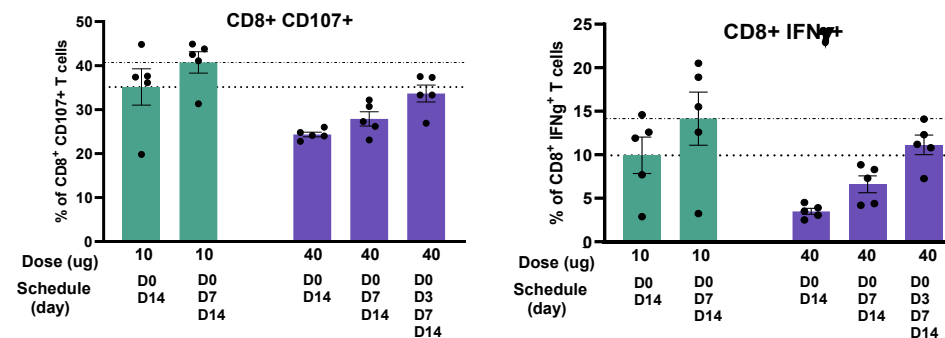
MULTIVALENT VACCINES

Vaccines can be developed and delivered using **5-fold lower dose vs. ALC-315™** with **equivalent titers** with our newer technology.



CANCER VACCINES

Our LNP demonstrate **better cellular response vs. industry clinical candidate** at 4-fold lower dose and at least 1 less administration.



LEGEND

ALC-315™

Next Generation Lipid

Industry Clinical Candidate

Continuous Innovation: Pre-Formed Vesicles (PFV)



Refrigerated (2-8°C) (and potential for room temperature) long term **storage & distribution**



Flexible, small-scale manufacturing capability

Infectious Disease Vaccine



Improve **accessibility and distribution**

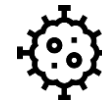


Regional-specific vaccine formulation



On-demand variant selection

Personalized Cancer Vaccine



Adaptable **neoantigen modification**

Rare Genetic Disease Therapeutics

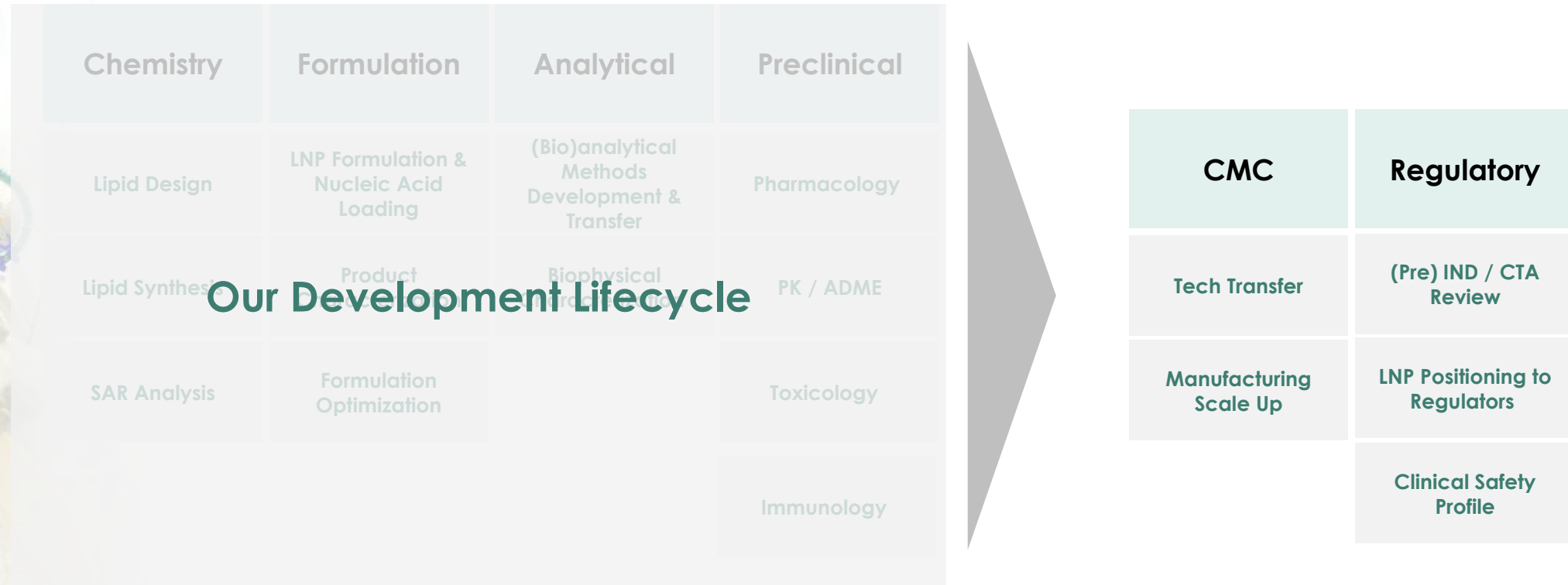


Fast and cost-effective

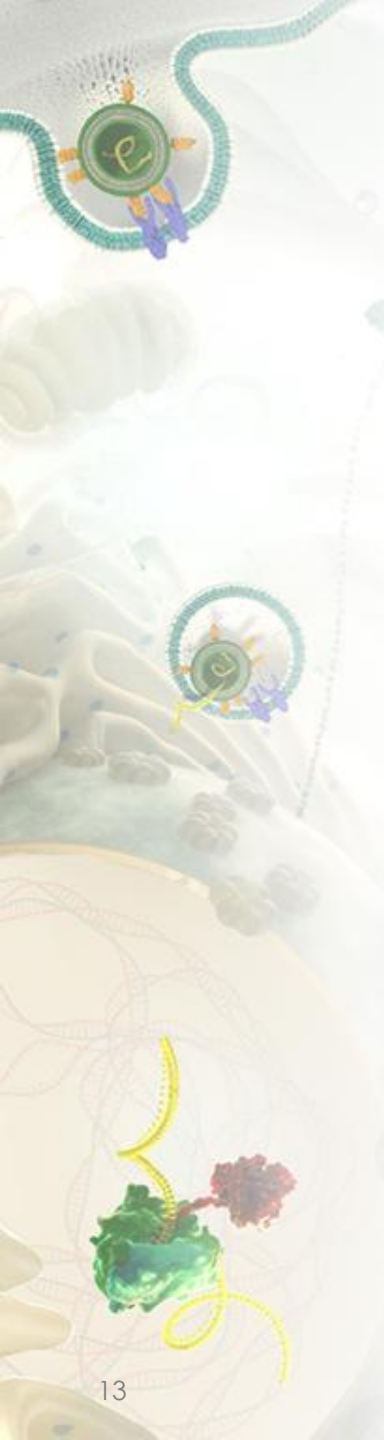


Modular, flexible, platform approach

Accelerating Clinical Entry



Our breadth and depth of capabilities enables end-to-end drug development support.



Why Acuitas?

1 **Best-in-class and First-in-class**

We have unparalleled technology with:

- **First-in-class and best-in-class** drug products commercialized, including Onpattro® and Comirnaty®.
- A broad and **comprehensive IP portfolio**.

2 **Accelerated Clinical Entry**

We understand the importance of early clinical entry.

We provide access to **cGMP-grade lipids**.

Our **expertise in tech transfer and product scale up** de-risks your development program, saving you time and money.

Our partners have initiated **26 clinical trials** in the last 2 years.

3 **Unparalleled Scientific Leadership & Experience**

Working with academic scientists and key opinion leaders we **publish regularly in the top scientific journals**.

Our team is at the cutting edge of scientific discovery.

Our Scientific Leadership

Mechanism of Action

nature
nature
nature

- Spatial profiling of gene editing by in situ sequencing in mice and macaques (2025)
- Lipid nanoparticles (LNP) induce activation and maturation of antigen presenting cells in young and aged individuals (2023)
- Molecular fate-mapping of serum antibody responses to repeat immunization (2023)

Therapeutic Areas



nature
nature



- Patient-Specific In Vivo Gene Editing to Treat a Rare Genetic Disease (2025)
- Treatment of a metabolic liver disease in mice with a transient prime editing approach (2025)
- A potent epigenetic editor targeting human PCSK9 for durable reduction of low-density lipoprotein cholesterol levels (2025)
- Physiologically based modeling of LNP-mediated delivery of mRNA in the vascular system (2024)

Extra-hepatic Application



ADVANCED HEALTHCARE
MATERIALS

Science

Science

- Targeting lipid nanoparticles to the blood-brain barrier to ameliorate acute ischemic stroke (2024)
- Exploring Mechanisms of Lipid Nanoparticle-Mucus Interactions in Healthy and Cystic Fibrosis Conditions (2024)
- In vivo modification of hematopoietic stem cells by targeted lipid nanoparticles delivering mRNA (2023)
- CAR T cells produced in vivo to treat cardiac injury (2022)

Vaccine Improvements

Science

Science

Science

nature

- Nonstabilized SARS-CoV-2 spike mRNA vaccination induces broadly neutralizing antibodies in nonhuman primates (2025)
- An IL-12 mRNA-LNP adjuvant enhances mRNA vaccine-induced CD8 T cell responses (2025)
- A multivalent mRNA-LNP vaccine protects against Clostridioides difficile infection (2024)
- Development of a nucleoside-modified mRNA vaccine against clade 2.3.4.4b H5 highly pathogenic avian influenza virus (2024)

For a current list of publications, please visit our website [here](#).

Our Business Development Process

**1. Initial
Conversation /
CDA**

**2.
Technology
Evaluation
Agreement
(Optional)***

**3. Term
Sheet**

**4.
Development
& Option
Agreement**

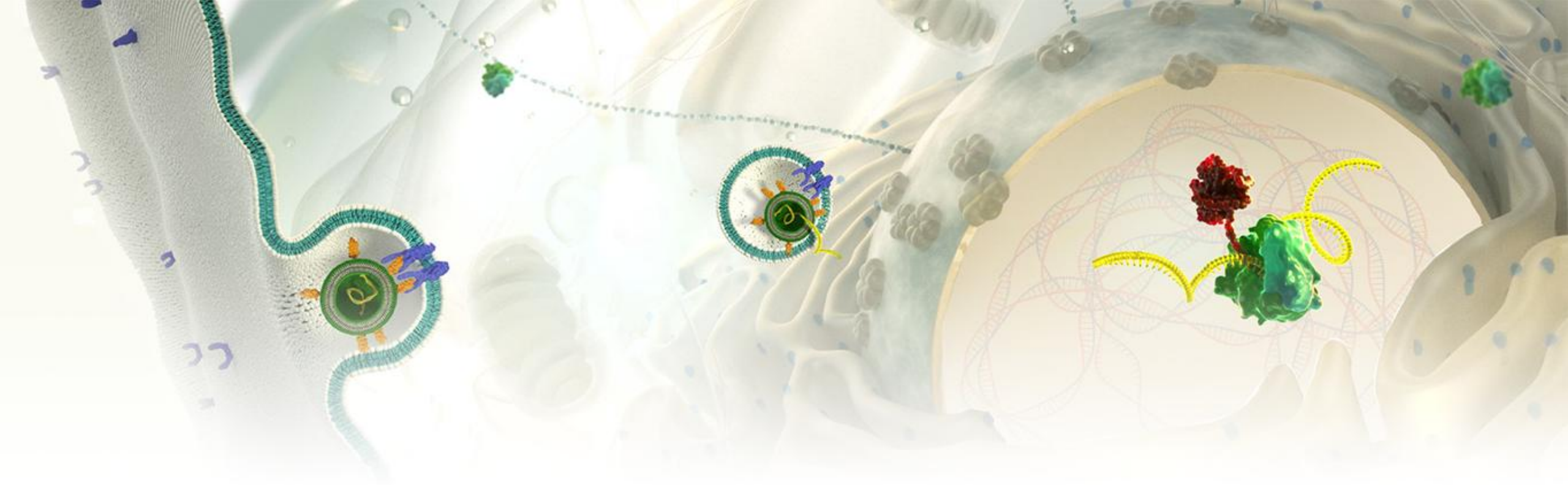
5. License

*An opportunity to
evaluate our
technology*

*A collaboration to
optimize your clinical
candidate and
select the ideal LNP
formulation.*

*A license to
enable clinical trials and
commercialization of your
product.*

*: Shipping costs may apply.



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