Acuitas Therapeutics

Non-Confidential Presentation

ACUITAS
THERAPEUTICS

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.**

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Applying Our LNP Technology

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

Gene Modulation
Expression of an **epigenic editor** to modify gene expression **without changing the genetic code.**

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

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

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

Therapeutic Protein Delivery
Expression of a **human protein** to treat disease.

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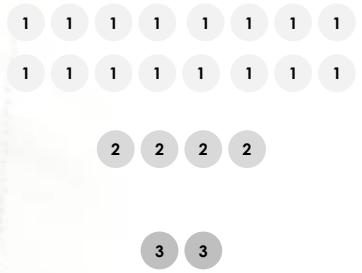
Our Partners' Success

Products in Clinic



16 in Phase 1

4 in Phase 2

2 in Phase 3

Clinical Firsts

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

First Clinically Approved **mRNA-based Medicine**

First Widely Approved **COVID-19 Vaccine**

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

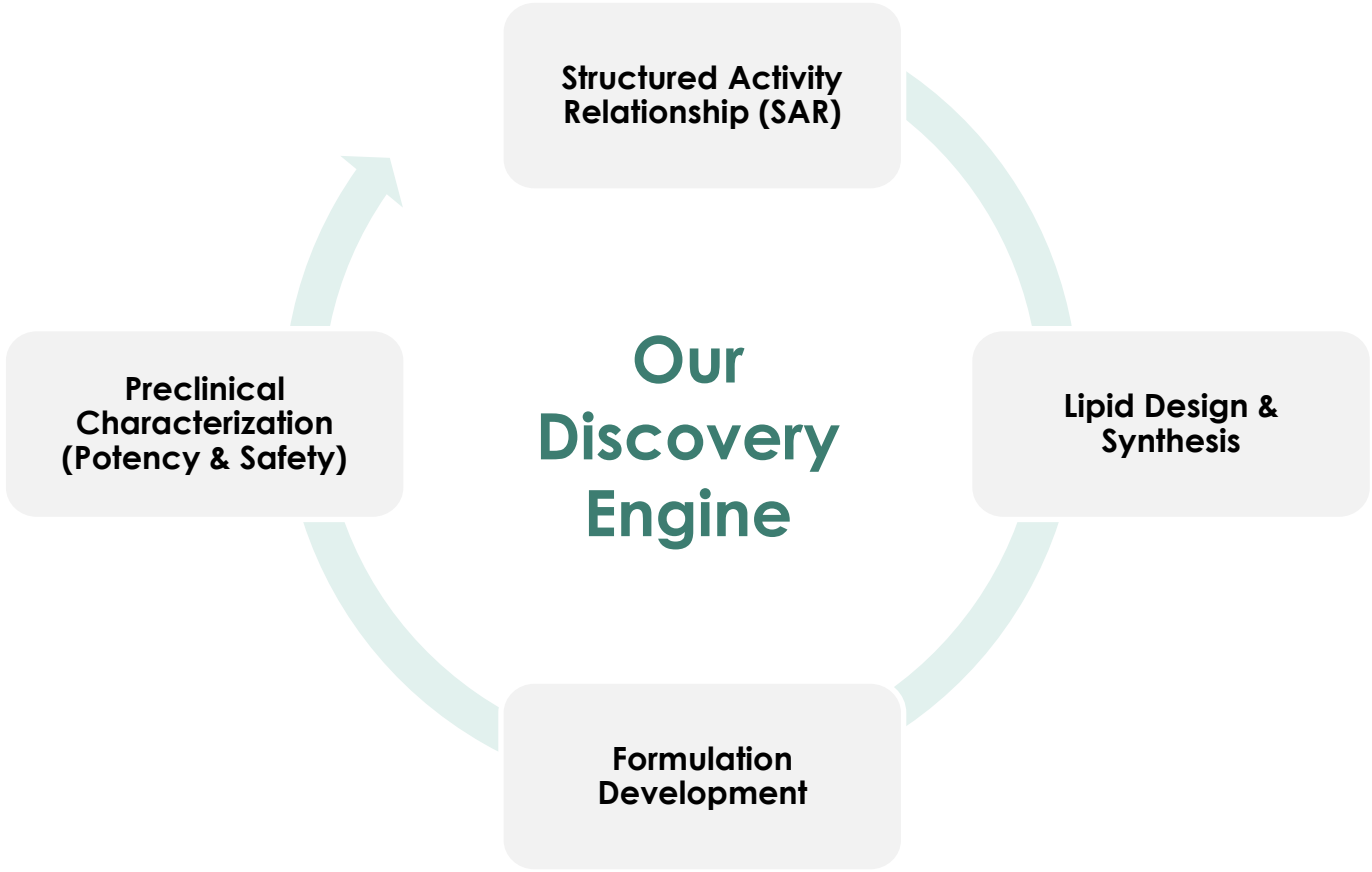
Dec 2024: Tune Therapeutics Moves into Clinical Spotlight with TUNE-401: A First-in-Class Epigenetic Silencer for Hepatitis B

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



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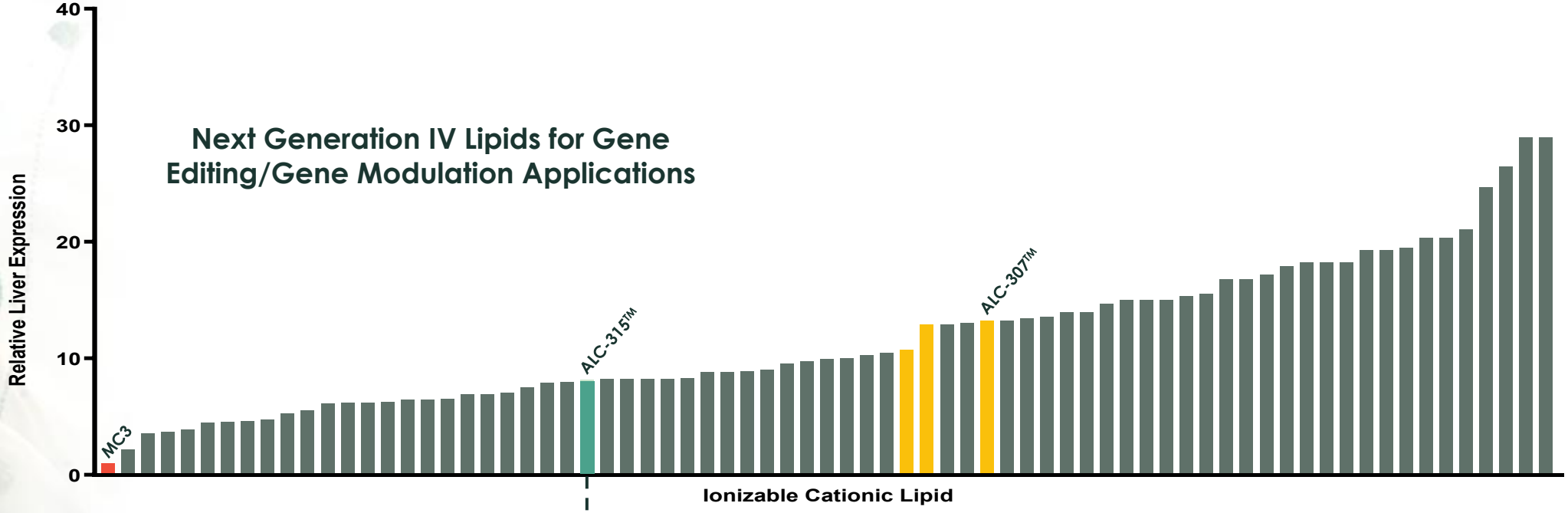
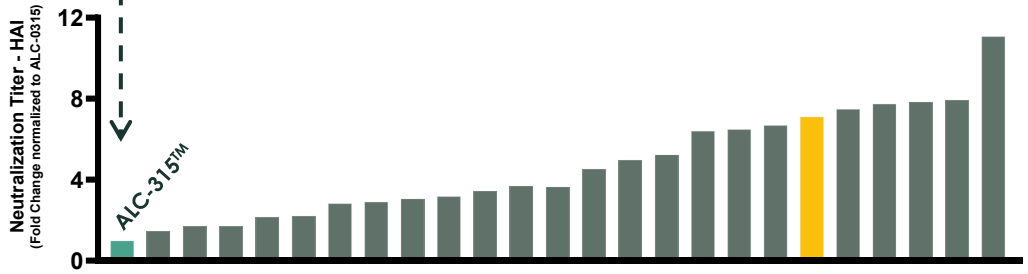
Our Approach to Innovation

Next Generation IV Lipids for Gene Editing/Gene Modulation Applications



Next Generation IM Lipids for Vaccine Applications



LEGEND cGMP ready + in clinic

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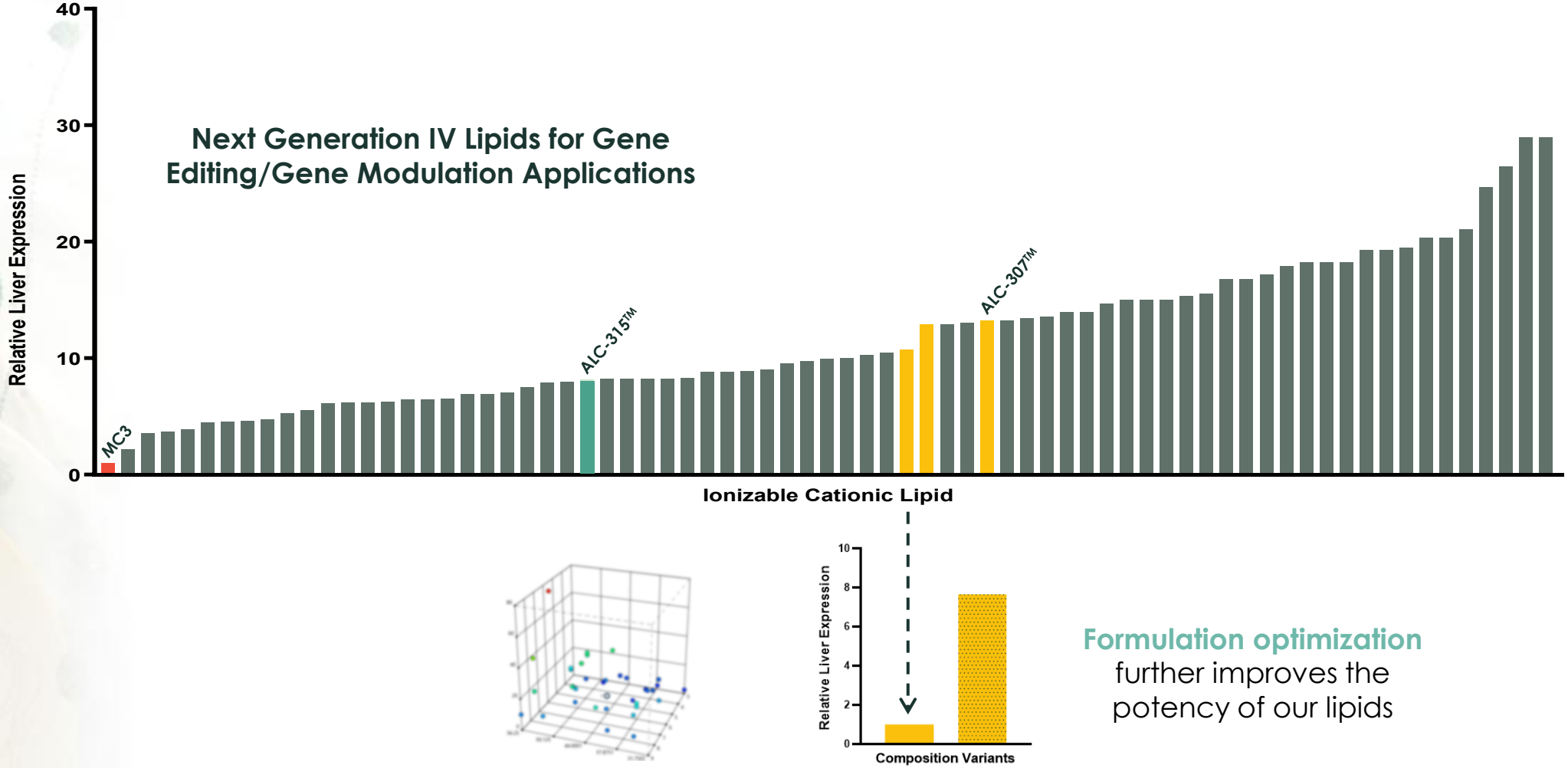
Continuous Innovation: Formulation Optimization



Formulation optimization further improves the potency of our lipids

LEGEND: cGMP ready + in clinic; cGMP ready with improved formulation

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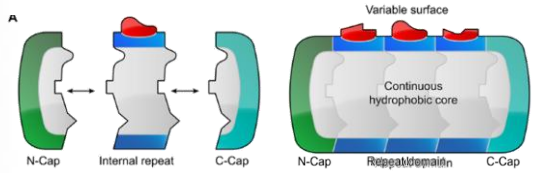
Continuous Innovation: Extrahepatic Targets

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 (DARPins)** to target any tissue and / or organ with high specificity.



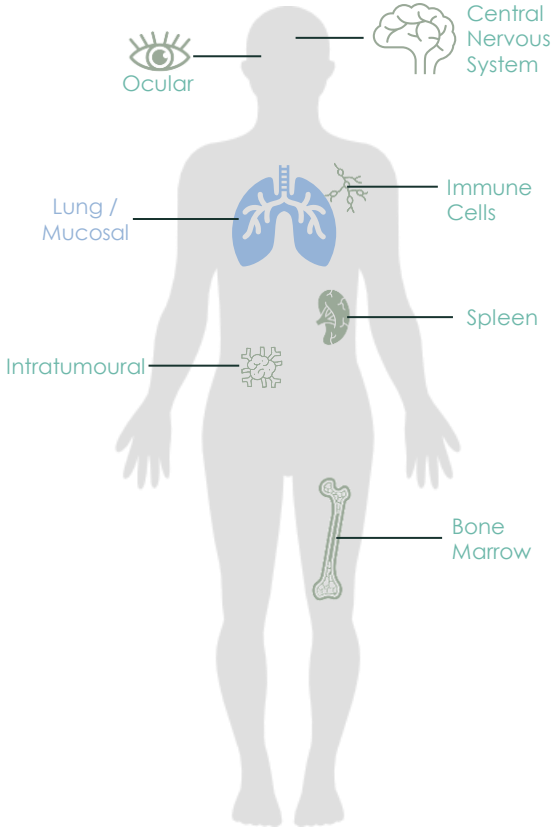
AREAS WE CAN TARGET

<u>Diseases Affecting the Lung</u>
(i.e., CF, Cancer, Infectious Disease etc.)

Mucosal (via aerosolization)

<u>Epigenetic Regulation and Gene Modification</u>

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



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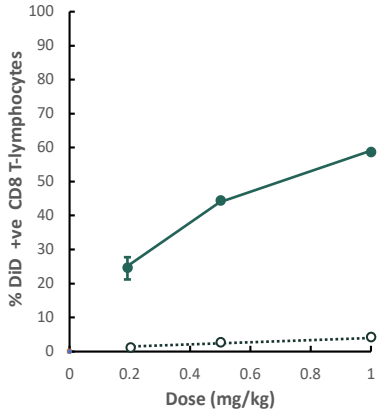
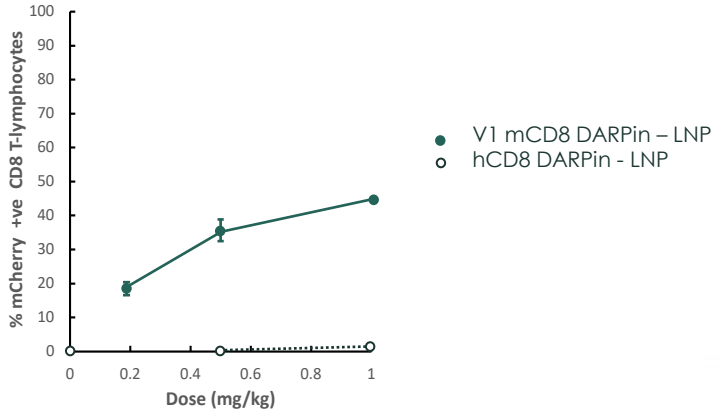
Continuous Innovation: Targeted LNP Delivery

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

LNP Binding / Uptake



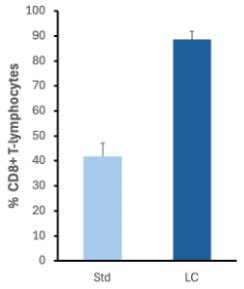
Reporter Gene Expression



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

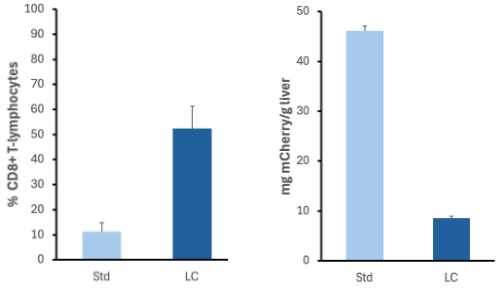
Expression in liver is ~5x lower vs. standard LNP.

LNP Binding / Uptake



CD8+ T-cell

Reporter Gene Expression



CD8+ T-cell *Liver Protein Expression*

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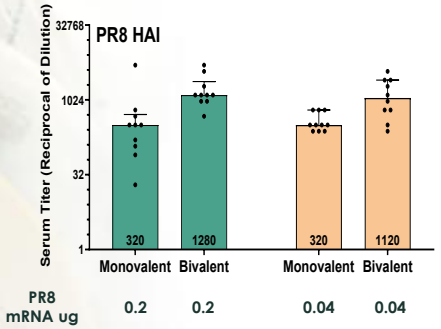
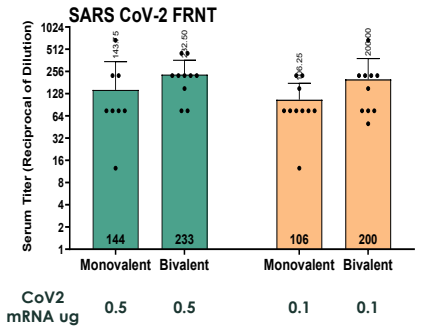
Continuous Innovation: Multivalent & Cancer Vaccines

MULTIVALENT VACCINES

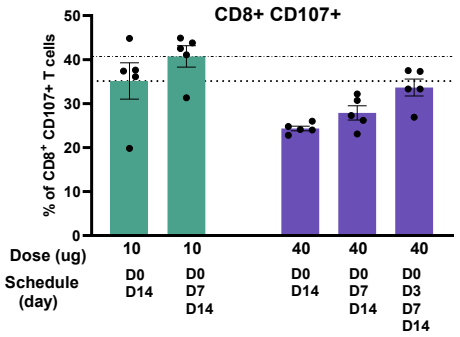
Vaccines can be developed and delivered using **5-fold lower dose vs. ALC-315™ with equivalent titers** with our newer technology.

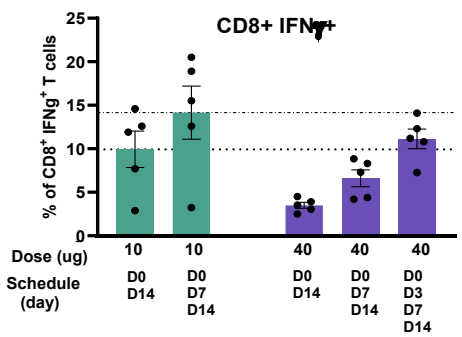




CANCER VACCINES

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

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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

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Accelerating Clinical Entry

Our Development Lifecycle

Chemistry	Formulation	Analytical	Preclinical
Lipid Design	LNP Formulation & Nucleic Acid Loading	(Bio)analytical Methods Development & Transfer	Pharmacology
Lipid Synthesis	Product	Biophysical	PK / ADME
SAR Analysis	Formulation Optimization		Toxicology
			Immunology

CMC	Regulatory
Tech Transfer	(Pre) IND / CTA Review
Manufacturing Scale Up	LNP Positioning to Regulators
	Clinical Safety Profile

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

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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.

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Our Scientific Leadership

Mechanism of Action	nature	• Spatial profiling of gene editing by in situ sequencing in mice and macaques (2025)
	nature	• Lipid nanoparticles (LNP) induce activation and maturation of antigen presenting cells in young and aged individuals (2023)
	nature	• Molecular fate-mapping of serum antibody responses to repeat immunization (2023)
Therapeutic Areas	The New England Journal of Medicine	• Patient-Specific In Vivo Gene Editing to Treat a Rare Genetic Disease (2025)
	nature	• Treatment of a metabolic liver disease in mice with a transient prime editing approach (2025)
	nature	• A potent epigenetic editor targeting human PCSK9 for durable reduction of low-density lipoprotein cholesterol levels (2025)
	Cell	• Physiologically based modeling of LNP-mediated delivery of mRNA in the vascular system (2024)
Extra-hepatic Application	Cell	• Targeting lipid nanoparticles to the blood-brain barrier to ameliorate acute ischemic stroke (2024)
	Advanced Healthcare Materials	• Exploring Mechanisms of Lipid Nanoparticle-Mucus Interactions in Healthy and Cystic Fibrosis Conditions (2024)
	Science	• In vivo modification of hematopoietic stem cells by targeted lipid nanoparticles delivering mRNA (2023)
	Science	• CAR T cells produced in vivo to treat cardiac injury (2022)
Vaccine Improvements	Science	• Nonstabilized SARS-CoV-2 spike mRNA vaccination induces broadly neutralizing antibodies in nonhuman primates (2025)
	Science	• An IL-12 mRNA-LNP adjuvant enhances mRNA vaccine–induced CD8 T cell responses (2025)
	Science	• A multivalent mRNA-LNP vaccine protects against Clostridioides difficile infection (2024)
	nature	• 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.

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Our Business Development Process

1. Initial Conversation / CDA

2. Technology Evaluation Agreement *(Optional)**

An opportunity to evaluate our technology

3. Term Sheet

4. Development & Option Agreement

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

5. License

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

*: Shipping costs may apply.

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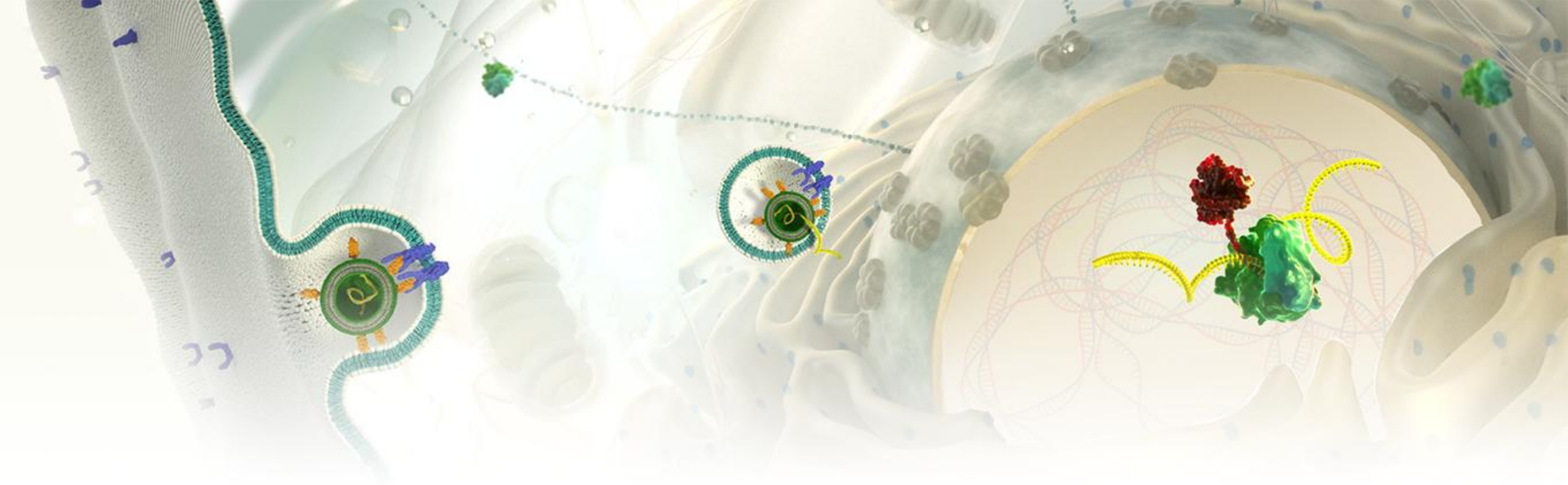

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