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.**

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Applying Our LNP Technology

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

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).

Antibody and Therapeutic Protein Delivery
Expression of **proteins including prophylactic** or **therapeutic antibodies** to treat current and emerging diseases.

In Vivo CAR-T
Expression of a Chimeric Antigen Receptor for **in vivo production of CAR-T cells** to treat cancer and autoimmune disease.

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Our Partners' Success

Products in Clinic

| 1 | 1 | 1 | 1 | 1 | 1 | 1 | 1 |
| 1 | 1 | 1 | 1 | 1 | 1 | 1 | |

17 in Phase 1

| | 2 | 2 | 2 | 2 |

4 in Phase 2

| | | 3 | 3 |

2 in Phase 3

Clinical Firsts

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

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

COMIRNATY (COVID-19 Vaccine, mRNA)
Pfizer BIONTECH

First Clinically Approved **mRNA Vaccine**

Children's Hospital of Philadelphia

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

CREATE MEDICINES — November 2025: CREATE Medicines Announces Positive First-in-Human Results for MT-302

PRECISION BIOSCIENCES — November 2025: Precision BioSciences Presents Late-Breaking Phase 1 PBGENE-HBV Data at AASLD The Liver Meeting® Showing Safety, Tolerability and Cumulative, Dose-Dependent Antiviral Activity in First Three Cohorts

nChromaBio — November 2025: nChroma Bio Presents Preclinical Data Demonstrating Epigenetic Silencing Approach as a Potential Functional Cure for Chronic Hepatitis B at AASLD 2025

Metagenomi — November 2025: Metagenomi Presents New Preclinical Data from MGX-001 Hemophilia A Program Supporting Advancement into Clinical Development

Beam THERAPEUTICS — Aug 2025: Beam Therapeutics Provides Update on BEAM-302 Development Progress in Alpha-1 Antitrypsin Deficiency (AATD)

arbor biotech — 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
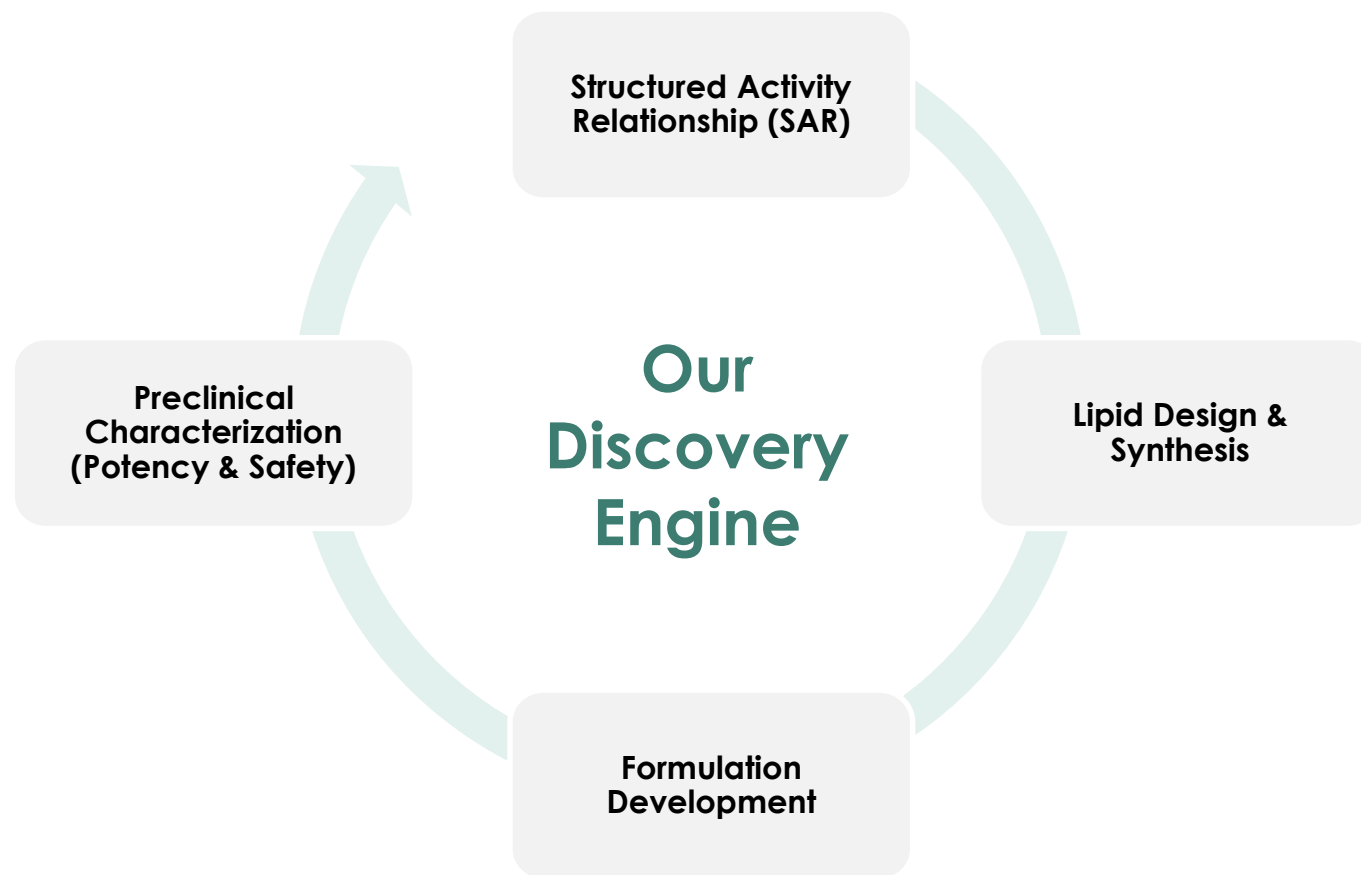
TUNE THERAPEUTICS — Dec 2024: Tune Therapeutics Moves into Clinical Spotlight with TUNE-401: A First-in-Class Epigenetic Silencer for Hepatitis B
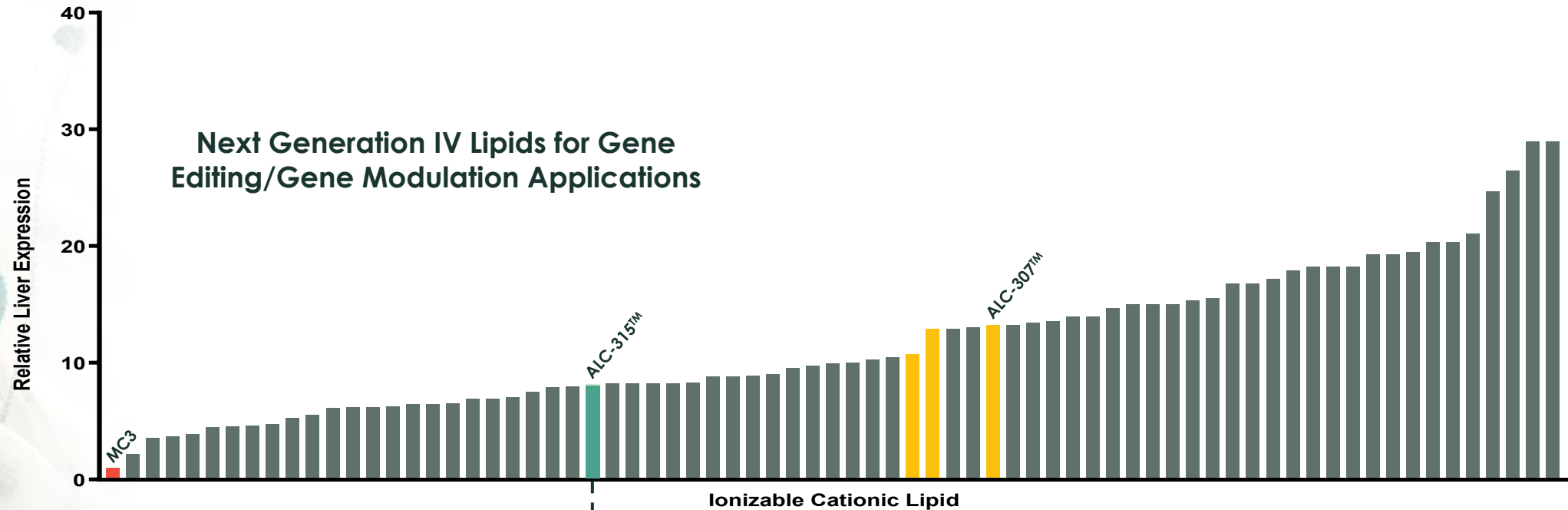
CureVac the RNA people® — Sept 2024: CureVac's CVGBM Cancer Vaccine Induces Promising Immune Responses in Phase 1 Study in Glioblastoma Presented at the ESMO 2024 Congress
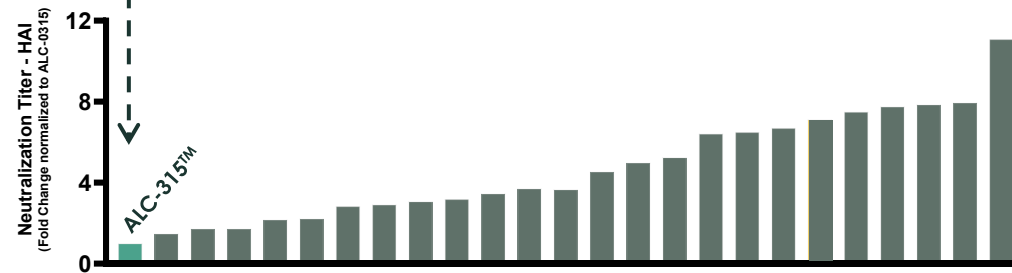Sept 2024: CureVac Partner GSK Announces Positive Phase 2 Data from Seasonal Influenza mRNA Vaccine Program

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Our Approach to Innovation

Structured Activity Relationship (SAR)

Lipid Design & Synthesis

Our Discovery Engine

Formulation Development

Preclinical Characterization (Potency & Safety)
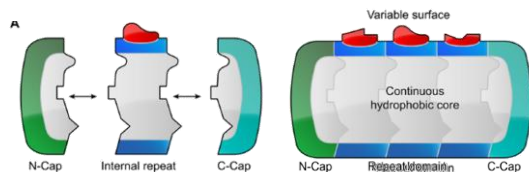
Our Approach to Innovation

Continuous Innovation: Extrahepatic Targets

OUR APPROACH

TARGETED
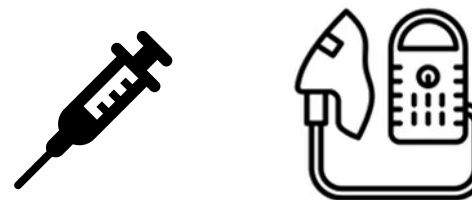
Targeted delivery to immune cells and hematopoietic stem cells.

We use antibody mimetics called **Designed ankyrin repeat proteins (DARPins).**
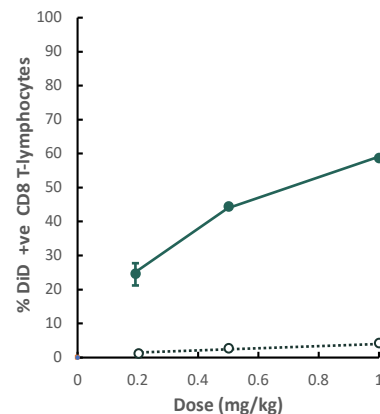

NON-TARGETED

Local delivery to the **eye, lung, and solid tumours.**
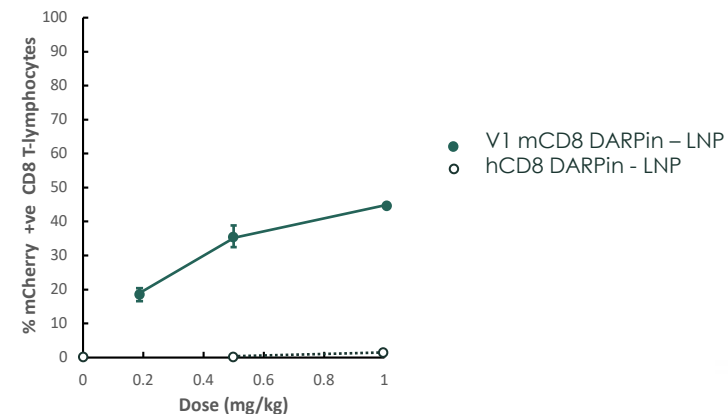
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Continuous Innovation: Targeted LNP Delivery

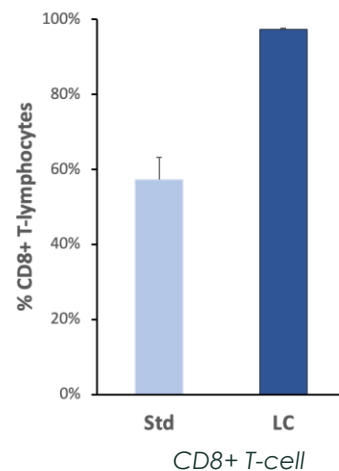CD8 DARPin targeted mRNA LNP show **dose dependent, target specific binding / uptake and transgene expression.**
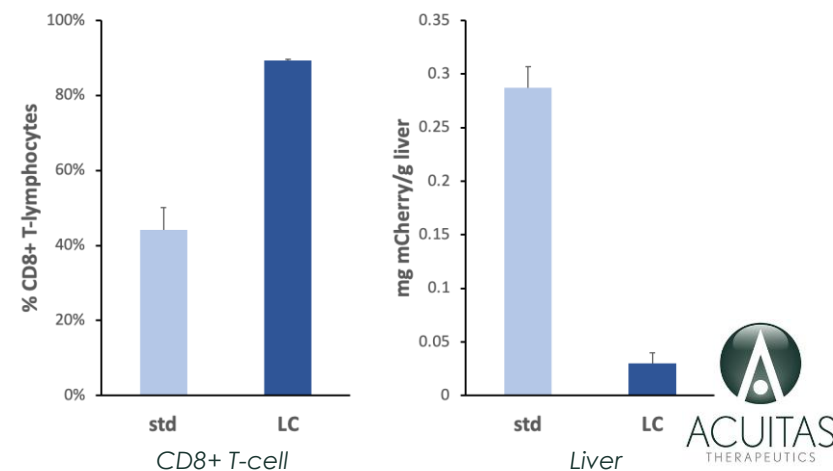
Optimized long circulating (LC) LNP results in **increased binding / uptake and expression.**

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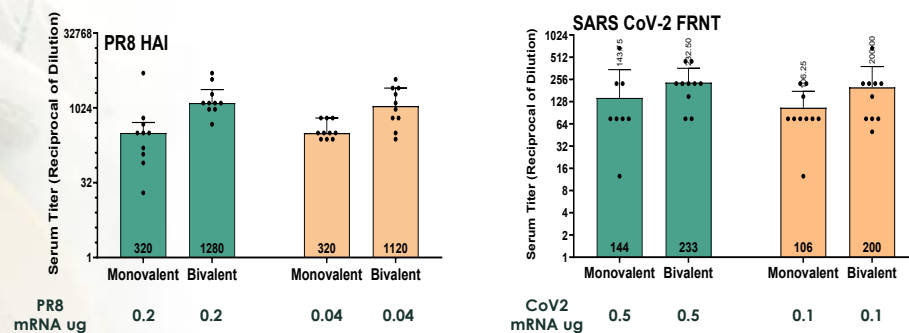

LNP Binding / Uptake

Reporter Gene Expression

● V1 mCD8 DARPin – LNP
○ hCD8 DARPin - LNP

LNP Binding / Uptake

CD8+ T-cell

Reporter Gene Expression

CD8+ T-cell

Liver

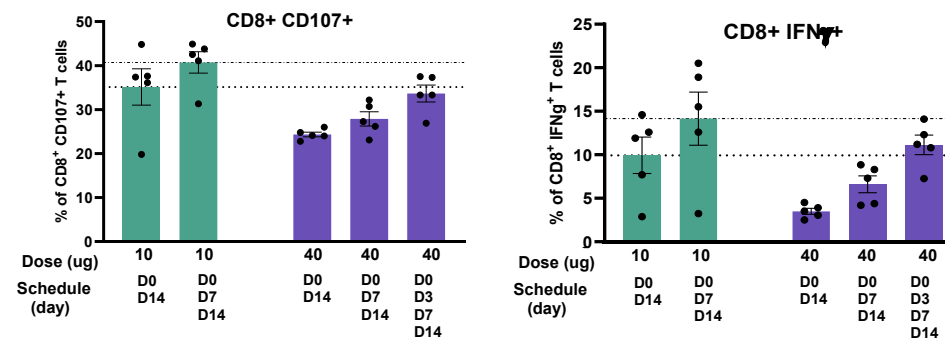# Continuous Innovation: Multivalent & Cancer Vaccines

MULTIVALENT VACCINES

Next Generation vaccine LNP show equivalent activity at **5-fold lower dose vs. ALC-315™.**

CANCER VACCINES

Our LNP demonstrate **better cellular response vs. clinical competitor** at 4-fold lower dose.

LEGEND — ALC-315™ | Next Generation LNP | Clinical Competitor

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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**

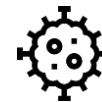**Regional-specific** vaccine formulation

On-demand variant selection

Personalized Cancer Vaccine

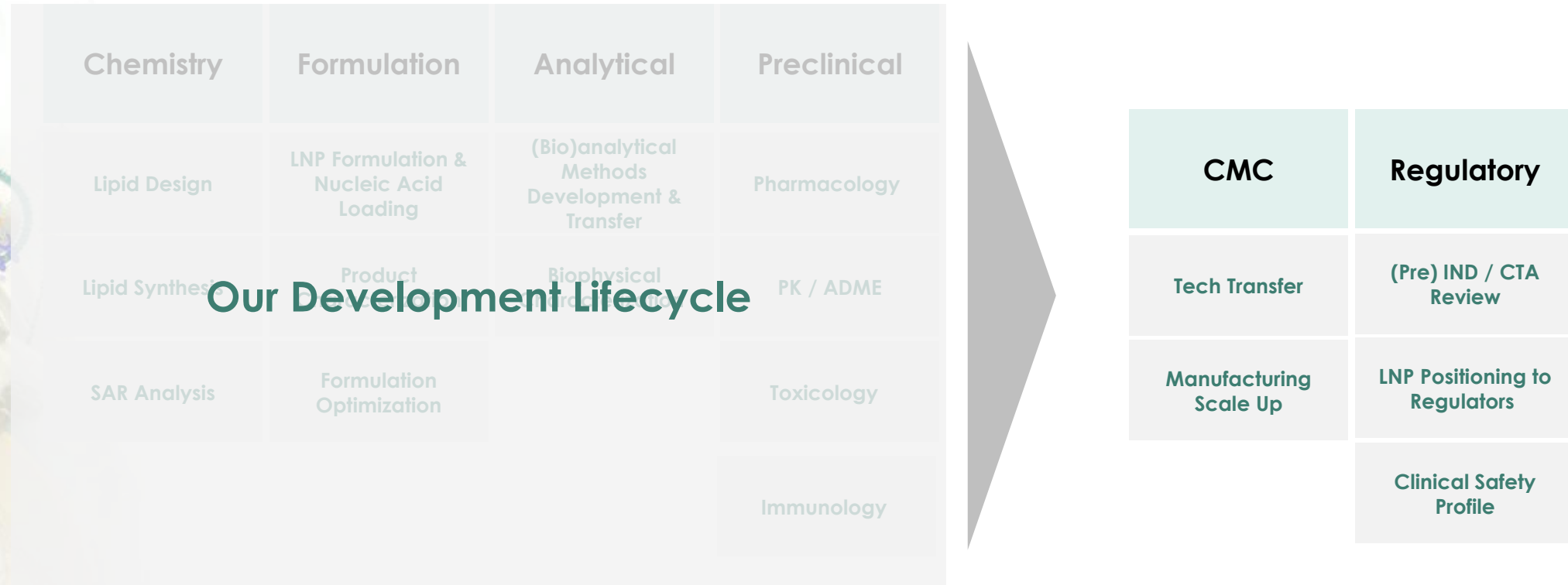Adaptable **neoantigen modification**

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 Characterization	Biophysical Characterization	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 **33 clinical trials** in the last 3 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)](#)

Cell
- [Distinct components of nucleoside-modified messenger RNA vaccines cooperate to instruct efficient germinal center responses (2025)](#)

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)](#)

Science Translational Medicine
- [Computationally designed mRNA-launched protein nanoparticle immunogens as an integrated vaccine platform (2025)](#)

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

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How We Collaborate

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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