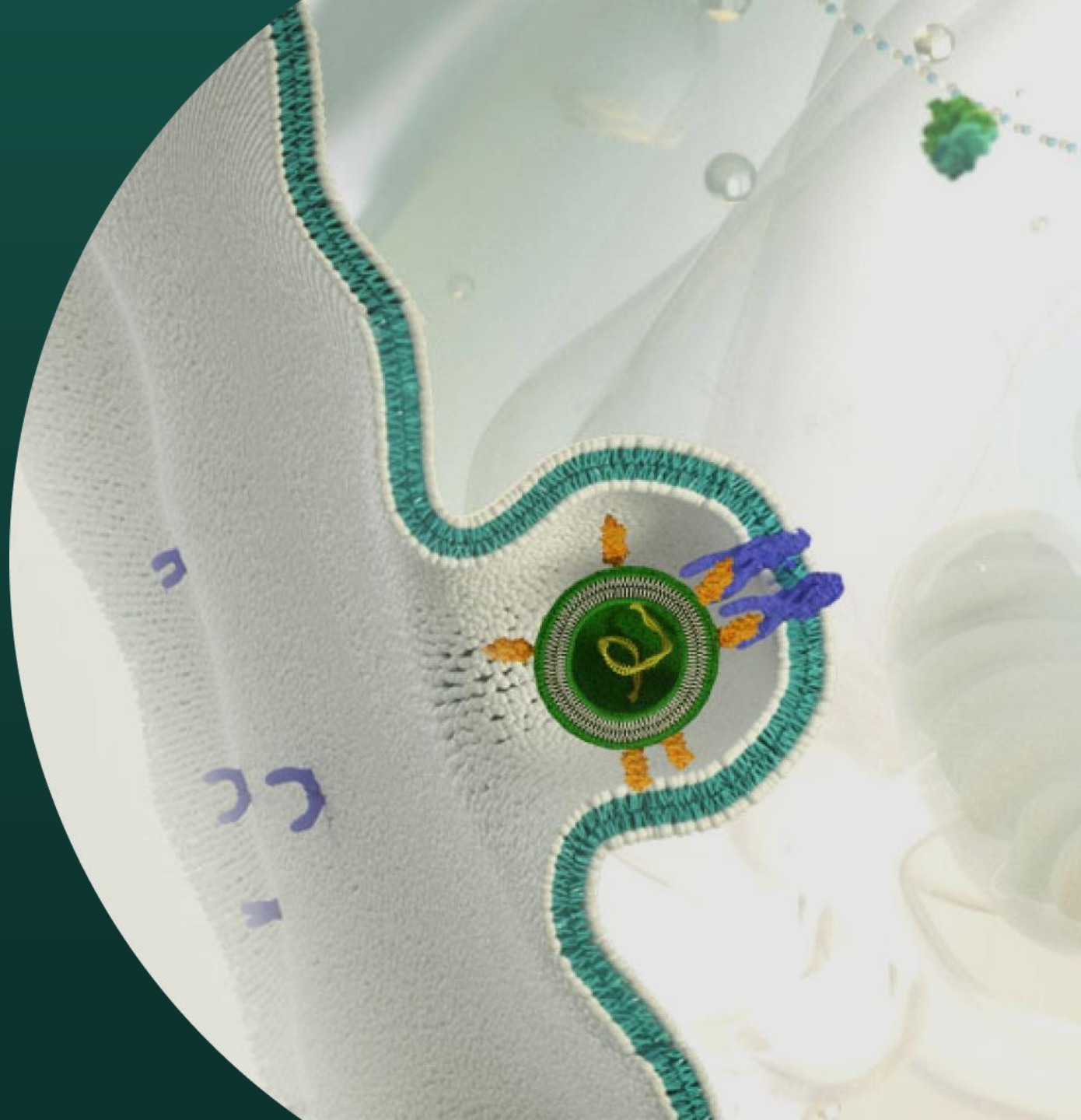
ACUITAS THERAPEUTICS

# Acuitas Therapeutics Inc.

Non-Confidential Presentation
November 2026



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# 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 has enabled **three world firsts**: Onpattro®, Comirnaty®, and the first personalized CRISPR gene therapy.



## WHO WE WORK WITH

- 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).

**Antibody & 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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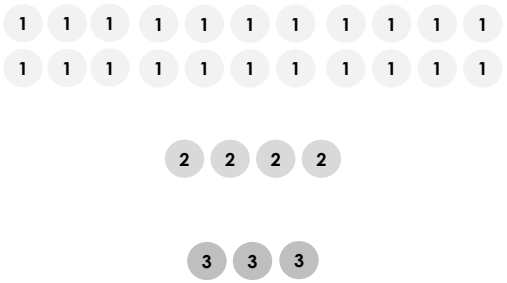
# Our Partners' Success

## Products in Clinic



**22** in Phase 1

**4** in Phase 2

**3** in Phase 3

## Clinical Firsts

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

First Clinically Approved **mRNA Vaccine**

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

Oct 2026: Beam Therapeutics Reports Initial Clinical Data from Phase 1/2 Trial of BEAM-301, Demonstrating Proof of Concept for Genetic Correction in Glycogen Storage Disease Type Ia (GSDIa) at ESGCT 33rd Annual Congress

Sept 2026: Beam Therapeutics Presents Updated Clinical Data from the Phase 1/2 Trial of BEAM-302 in Alpha-1 Antitrypsin Deficiency (AATD) at the European Respiratory Society (ERS) Congress 2026

July 2026: CREATE Medicines Receives Approval to Initiate First-in-Human Phase 1/2 Clinical Trial of In Vivo CAR-T Candidate CRT-402 for Autoimmune Diseases

April 2026: CREATE Medicines Doses First Patient in Phase 1/2 Study of MT-304, a First-in-Class Multi-immune In Vivo CAR Therapy Targeting HER2-Positive Solid Tumors

December 2025: CREATE Medicines Doses First Patient in Frontline HCC Trial Evaluating MT-303, an In Vivo CAR Therapy, in Combination with Standard-of-Care Immunotherapy

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

May 2026: Tune Therapeutics Presents Positive Phase 1b/2a Proof of Concept Data on TUNE-401: a First-in-Class Epigenetic Silencer for Patients with Hepatitis B at EASL 2026

May 2026: Scribe Therapeutics Presents Late-Breaking Preclinical Data Supporting STX-1150, a Clinical-Stage Epigenetic Silencing Therapy Designed to Achieve Years of LDL-C Lowering After a Single Dose

May 2026: Precision BioSciences Announces New and Late-Breaking PBGENE-HBV Clinical Data from the ELIMINATE-B Study at European Association for the Study of the Liver Congress 2026

May 2026: Ukko Announces First Participant Dosed in Phase 1/2a Clinical Study Evaluating UKK-0018 for Peanut Allergy Treatment

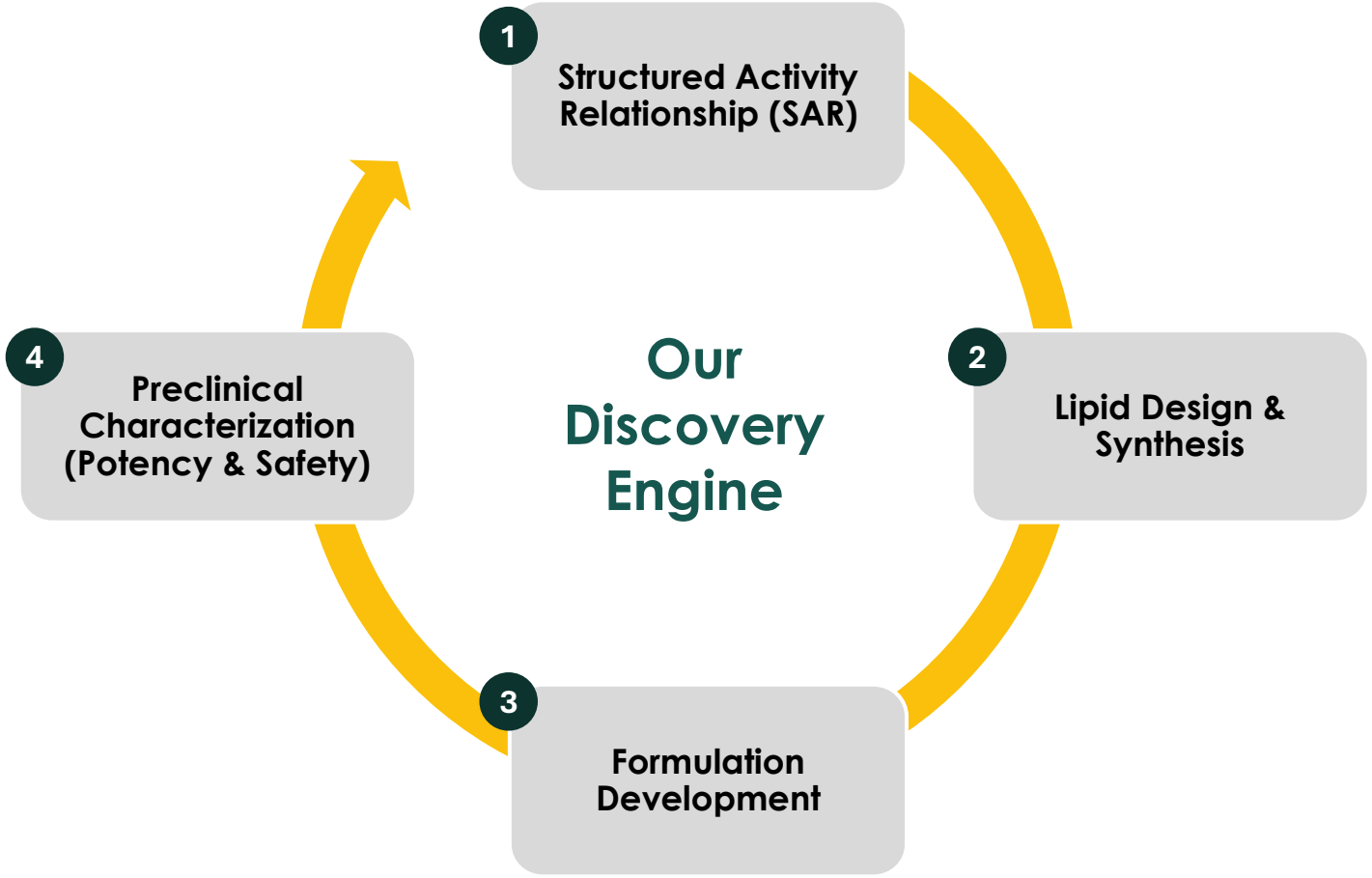
January 2026: Centivax Initiates Phase 1 First-in-Human Clinical Trial of Universal Flu Vaccine

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

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

Sept 2024: CureVac's CVGBM Cancer Vaccine Induces Promising Immune Responses in Phase 1 Study in Glioblastoma Presented at the ESMO 2024 Congress

# Our Approach to Innovation

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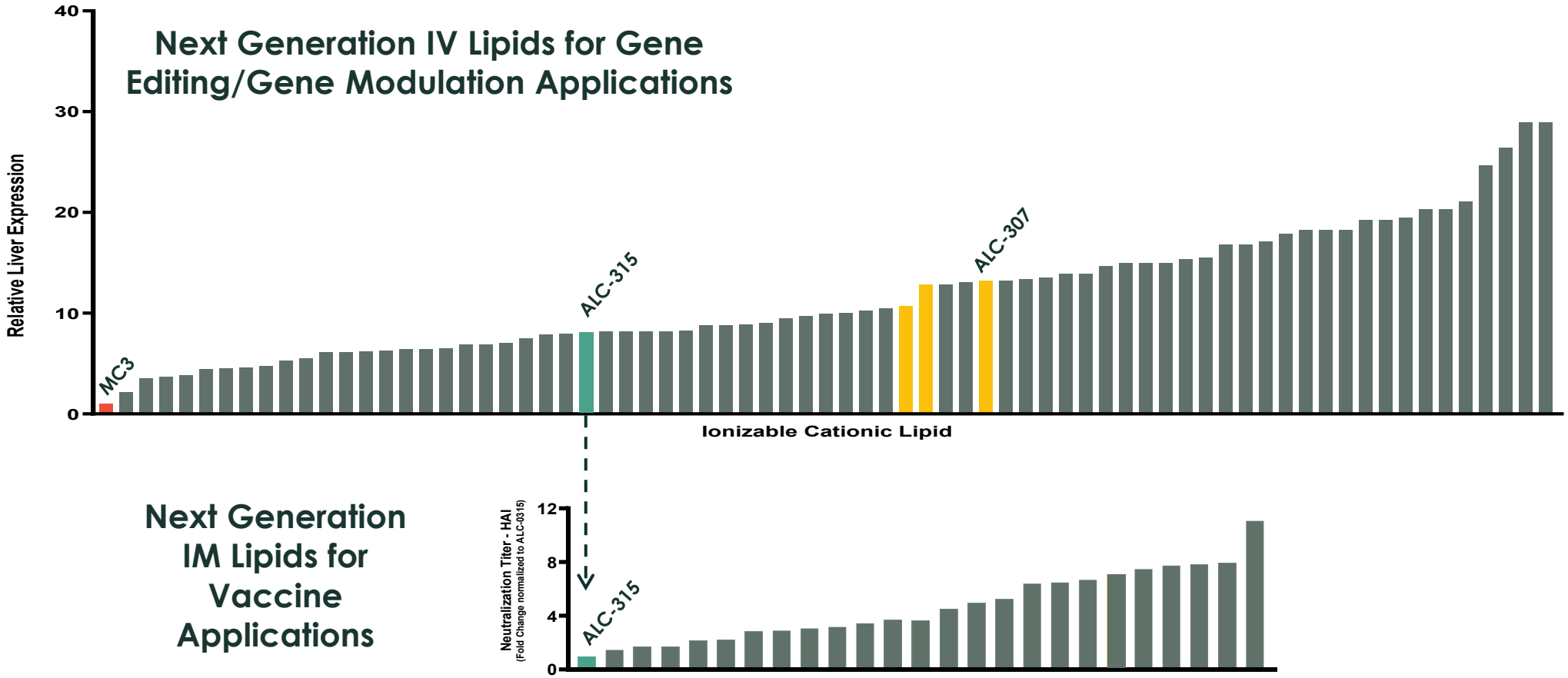
# Our Approach to Innovation




*ALC-315® is a registered trademark of Acuitas Therapeutics, Inc. in the U.S. Patent & Trademark Office.*

**LEGEND** cGMP ready + in clinic

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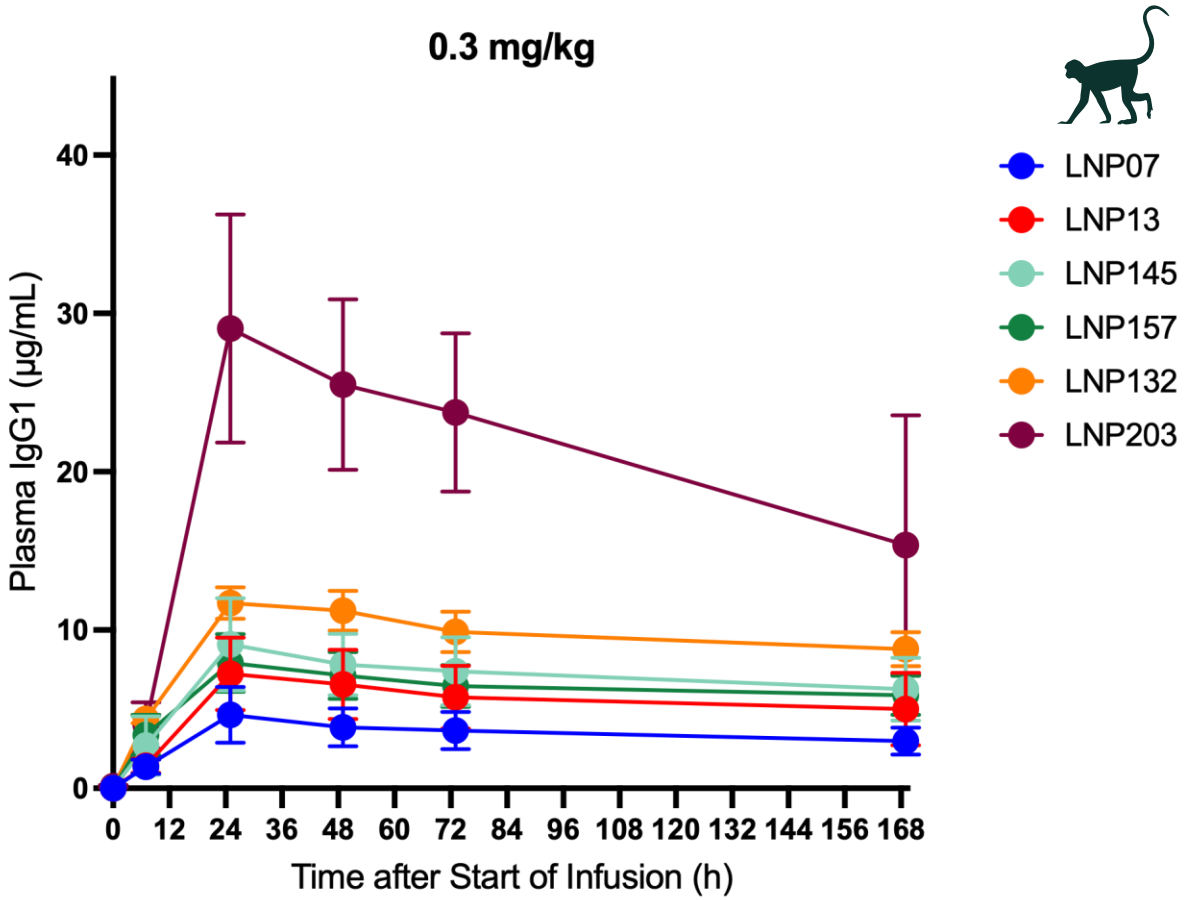
# Our Approach to Innovation

Top novel lipids have **equivalent or better potency at 0.3 mg/kg** compared to benchmark

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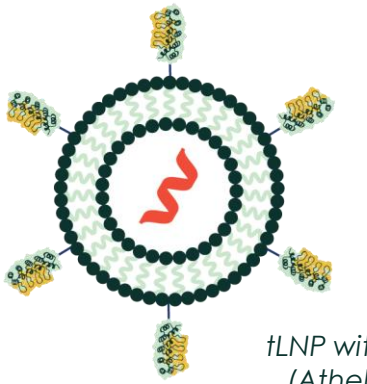
# Our Approach to Extrahepatic Targeting

## TARGETED

Targeted delivery to immune cells and hematopoietic stem cells.

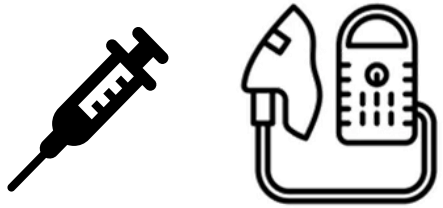
We use antibody mimetics called **Designed ankyrin repeat proteins (DARPins).**



*tLNP with DARPin (Athebody™)*

## NON-TARGETED

Local delivery to the **eye, lung, and solid tumours.**



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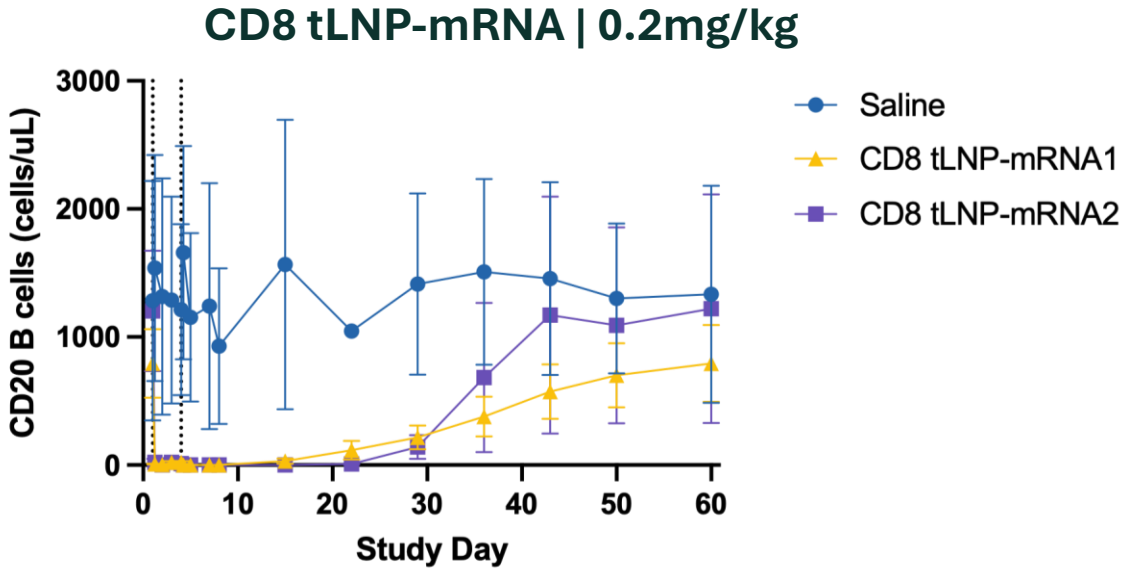
# Complete Reduction of B Cells using CD8-Targeted LNP For 15 Days

B cell recovery begins 20-30 days after last dose of CD8 tLNP

- CD8-targeted LNP containing CD20 CAR mRNA at 0.2 mg/kg in NHP
- Up to **80% of CD8+ T cells** express CD20 CAR T cell receptor
- **Significant and sustained depletion** of peripheral B cells after treatment with CD8-targeted LNP containing CD20 CAR mRNA




**In vivo CAR-T work conducted in collaboration with an RNA therapeutics partner*

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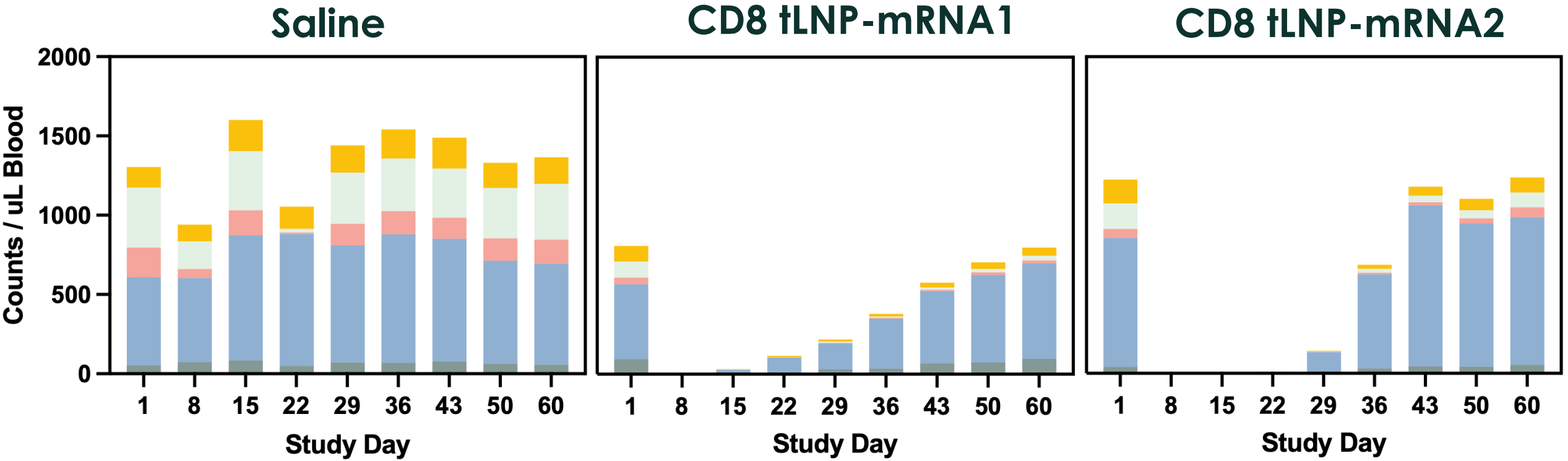
# B Cells Have Predominantly Naïve Phenotype Upon Repopulation




**In vivo CAR-T work conducted in collaboration with an RNA therapeutics partner*

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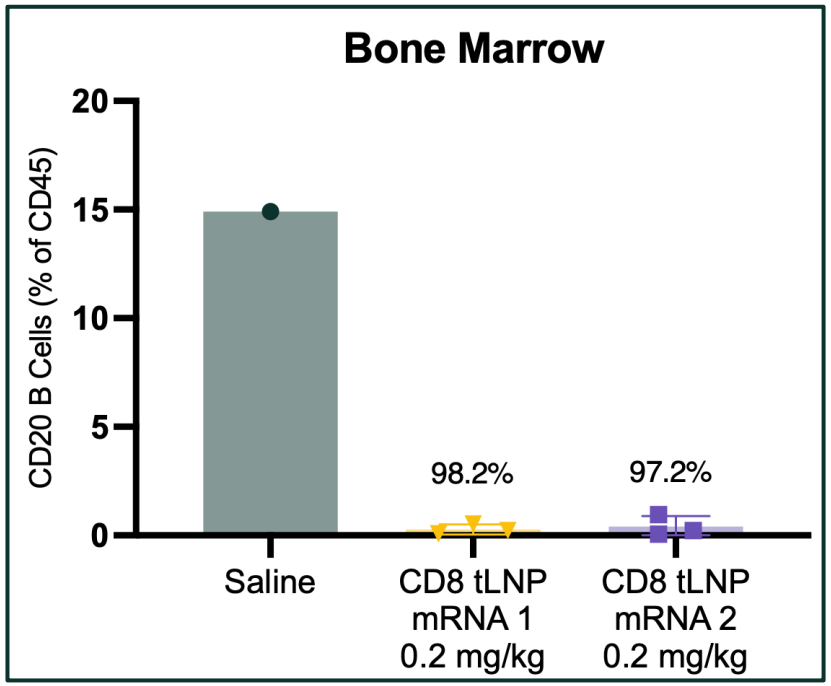
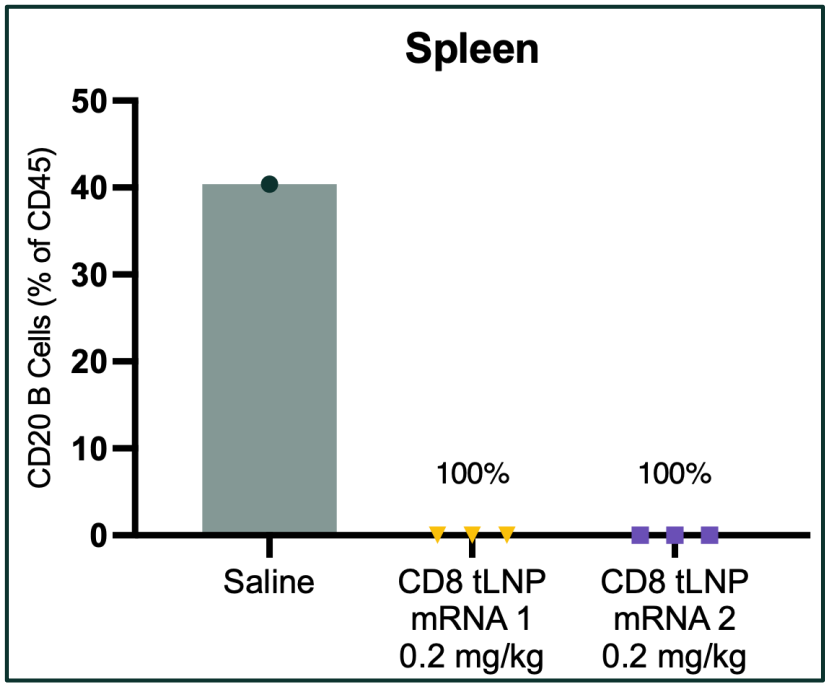
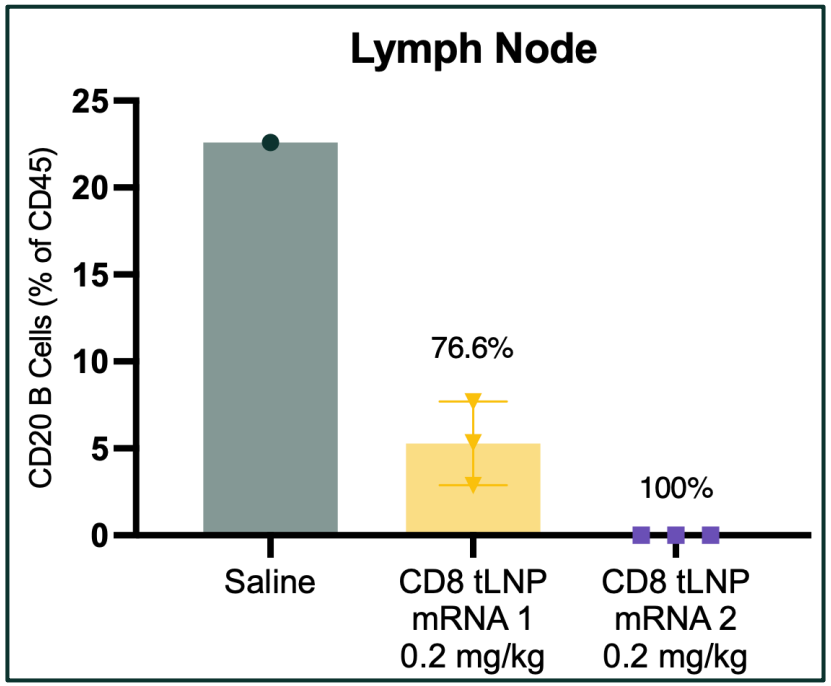
# CD8-Targeted LNP Expressing CD20 CAR mRNA

## Complete Reduction of B Cells in NHP Tissues at 0.2 mg/kg




**In vivo CAR-T work conducted in collaboration with an RNA therapeutics partner*

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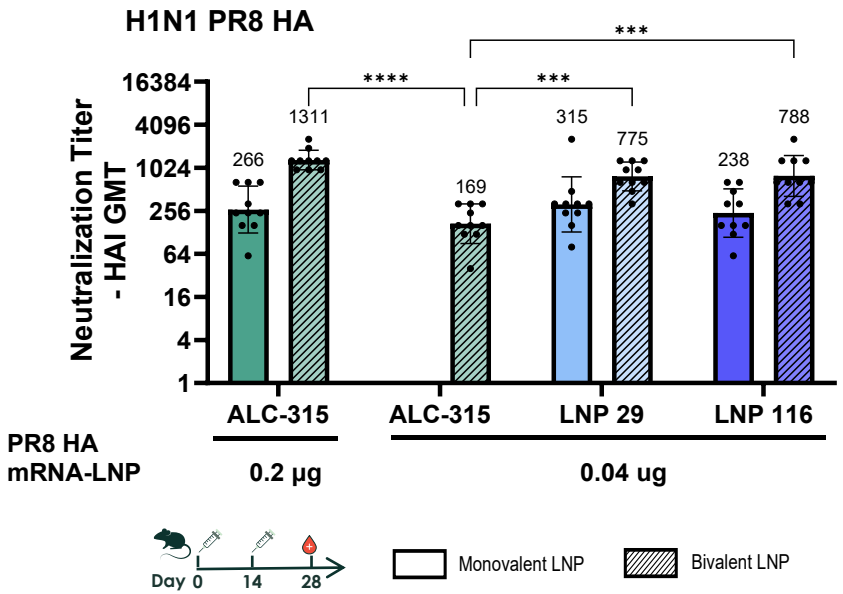
# Best-in-Class Multivalent & Cancer Vaccine Potency

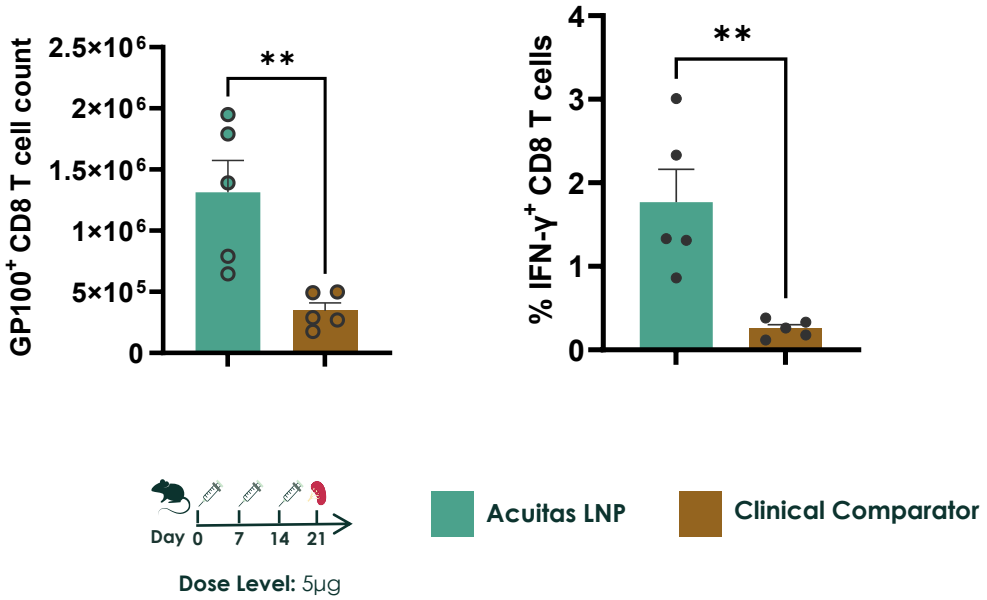
## MULTIVALENT VACCINES

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




## CANCER VACCINES

Acuitas LNP demonstrate **increased abundance & functionality** of antigen-specific CD8 T cells vs. clinical comparator (SM-102).

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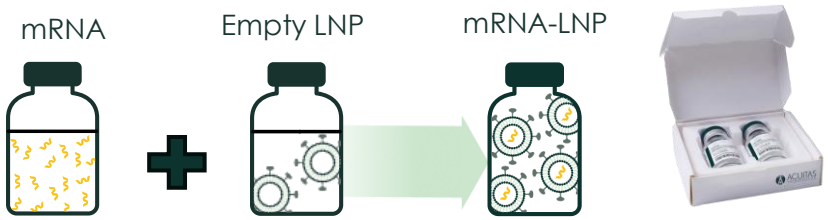
# Simplified Manufacture via Pre-Formed Vesicles (PFV)

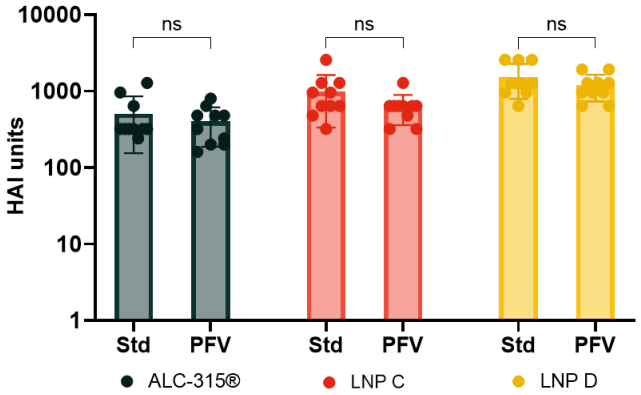
**PFV enables a kit format (2 vials) where mRNA is loaded into empty LNP at point-of-care**



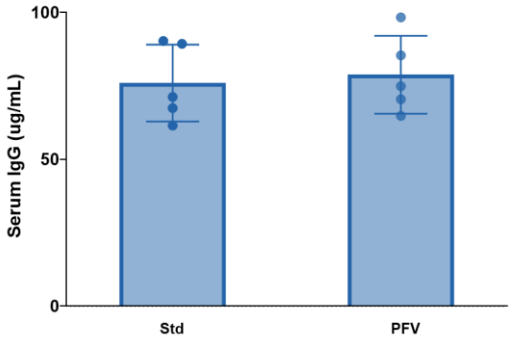

## BENEFITS

- **Refrigerated (2-8°C)** (and potential for room temperature) long term **storage & distribution**
- **Flexible, small-scale manufacturing capability**
- Modular, flexible, platform approach that is **fast and cost effective**

**PFV achieves equivalent potency to conventional LNP manufacturing methods**

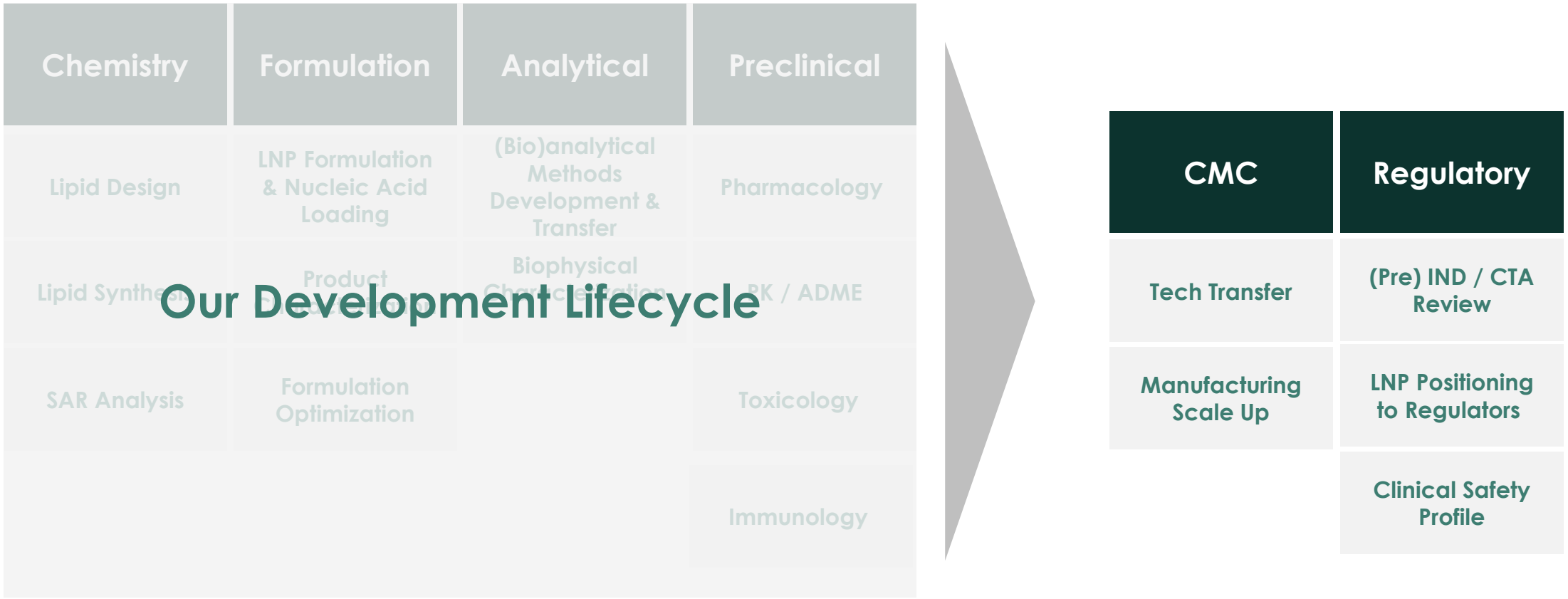



*For Vaccine Applications (i.e., IM)*




*For Systemic Applications (i.e., IV)*

# Accelerating Clinical Entry




**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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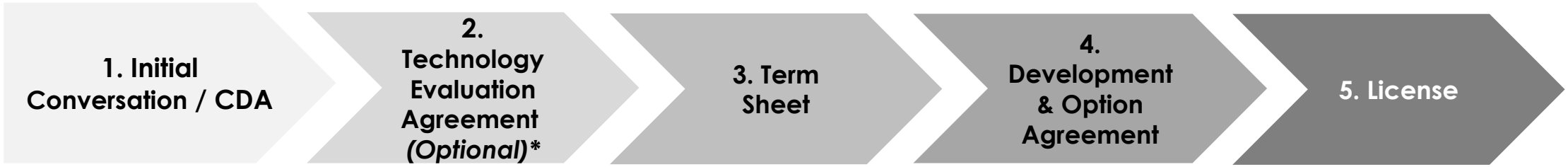
# How We Collaborate




*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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Let's Connect

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https://acuitastx.com/

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