Acuitas Therapeutics

Non-Confidential Presentation



Overview

WHO WE WORK WITH

We work with a variety of

Cutting edge

biotechnology

companies

pharmaceutical &

organizations, including:



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 have currently enable two commercial products:



COMIRNATY (COVID-19 Vaccine. mRNA)



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Foundations & NGOs

Leading academics in

universities & institutes

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

Z)

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



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.



Gene Modulation Expression of an epigenic

editor to modify gene expression without changing the genetic code.

Antibodies

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





Our Partners' Success

Products in Clinic

1 1 1 1 1	1 1 1 1 1	
1 1 1 1	1 1 1 1	
2 2	2 2	
3	3	
	Clini	
onpattrov		
Alnylam	P fizer BIC	
First Clinically Approved RNA interference-	First Clinically mRNA-basec	
based Medicine	First Widely A	

18 in Phase 1

in Phase 2

in Phase 3

ical Firsts

RNATY[.] -19 Vaccine, mRNA) **NTECH**

Approved d Medicine

Approved Vaccine

Children's Hospital

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



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: Arbor Biotechnologies to Present Preclinical Data for ABO-101 in PH1 at the American Society of Gene and Cell Therapy (ASGCT)

Beam

Mar 2025: Beam Therapeutics Announces Positive Initial Data for BEAM-302 in the Phase 1/2 Trial in Alpha-1 Antitrypsin Deficiency (AATD), Demonstrating First Ever Clinical Genetic Correction of a Disease-causing Mutation



PRECISION Feb 2025: Precision BioSciences Announces Initial Safety and BIOSCIENCES Antiviral Activity of PBGENE-HBV in the ELIMINATE-B Clinical Trial

THERAPEUTICS

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



OMEGA Nov 2024: <u>Omega Therapeutics Announces Successful</u> 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

Structured Activity Relationship (SAR)

Preclinical Characterization (Potency & Safety) Our Discovery Engine

> Formulation Development

Lipid Design & Synthesis





Our Approach to Innovation



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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





Continuous Innovation: Targeted LNP Delivery

100

90

80

70

60

40

0

0.2

0.4

Dose (mg/kg)

0.6

0.8

T-lymphocytes

8 50 8 50

₽ ⁴⁰ 30

Q 20

× 10

LNP Binding / Uptake

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

Long circulating LNP further increases binding / uptake and expression.



Reporter Gene Expression



Reporter Gene Expression



Ratio = 4 Dose = 0.5 mpk



Continuous Innovation: Multivalent & Cancer Vaccines

MULTIVALENT VACCINES

Multivalent vaccines can be developed and delivered with equivalent titers to monovalent versions with our technology.



Our LNPs demonstrate better cellular response vs. lipoplex at a lower dose.



Frequency of Ag-specific and Multifunctional CD8 T-cells





Continuous Innovation: Pre-Formed Vesicles (PFV)



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

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		J J

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

Fast and costeffective



Rare Genetic Disease Therapeutics

Modular, flexible, platform approach



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Accelerating Clinical Entry



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

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Why Acuitas?

Best-in-class <u>and</u> First-in-class

We have unparalleled technology with:

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

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.

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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 •	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	The NEW ENGLAND JOURNAL of MEDICINE C Cell C Cell	Patient-Specific In Vivo Gene Editing to Treat a Rare Genetic Disease (2025) Mosaic sarbecovirus nanoparticles elicit cross-reactive responses in pre-vaccinated animals (2024) Physiologically based modeling of LNP-mediated delivery of mRNA in the vascular system (2024)
E S	Vaccine Improvements	Science nature c ² Cell	A multivalent mRNA-LNP vaccine protects against Clostridioides difficile infection Development of a nucleoside-modified mRNA vaccine against clade 2.3.4.4b H5 highly pathogenic avian influenza virus (2024) Mutation-guided vaccine design: A process for developing boosting immunogens for HIV broadly neutralizing antibody induction (2024)
	Extra-hepatic Application	 Celi 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)

For a current list of publications, please visit our website <u>here</u>.





formulation.



*: Shipping costs may apply.



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