ARCTURUS THERAPEUTICS

Building the Next Generation of RNA Medicines

May 2020

FORWARD LOOKING STATEMENTS

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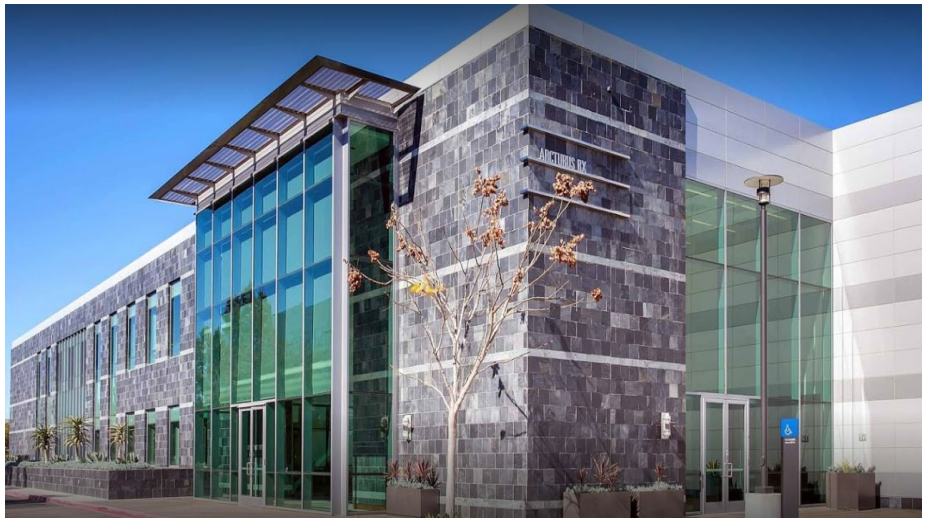
Company Highlights

Arcturus is a Clinical-Stage mRNA Medicines and Vaccines Company

Publicly Traded (NASDAQ:ARCT)

- Headquarters: San Diego, CA
- Number of Employees: 90
- Founded: 2013



Strong Intellectual Property Technology Portfolio

- 187 Patents & Patent Applications
- LUNAR® Delivery Technology
- STARR™ RNA Manufacturing Process
- Drug Product (LUNAR® + STARR™) Manufacturing Process

Arcturus Technologies Validated by Multiple Strategic Partners

Johnson & Johnson · Takeda · ultragenyx pharmaceutical · Cystic Fibrosis Foundation · DukeNUS Medical School

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LUNAR® Delivery Technology

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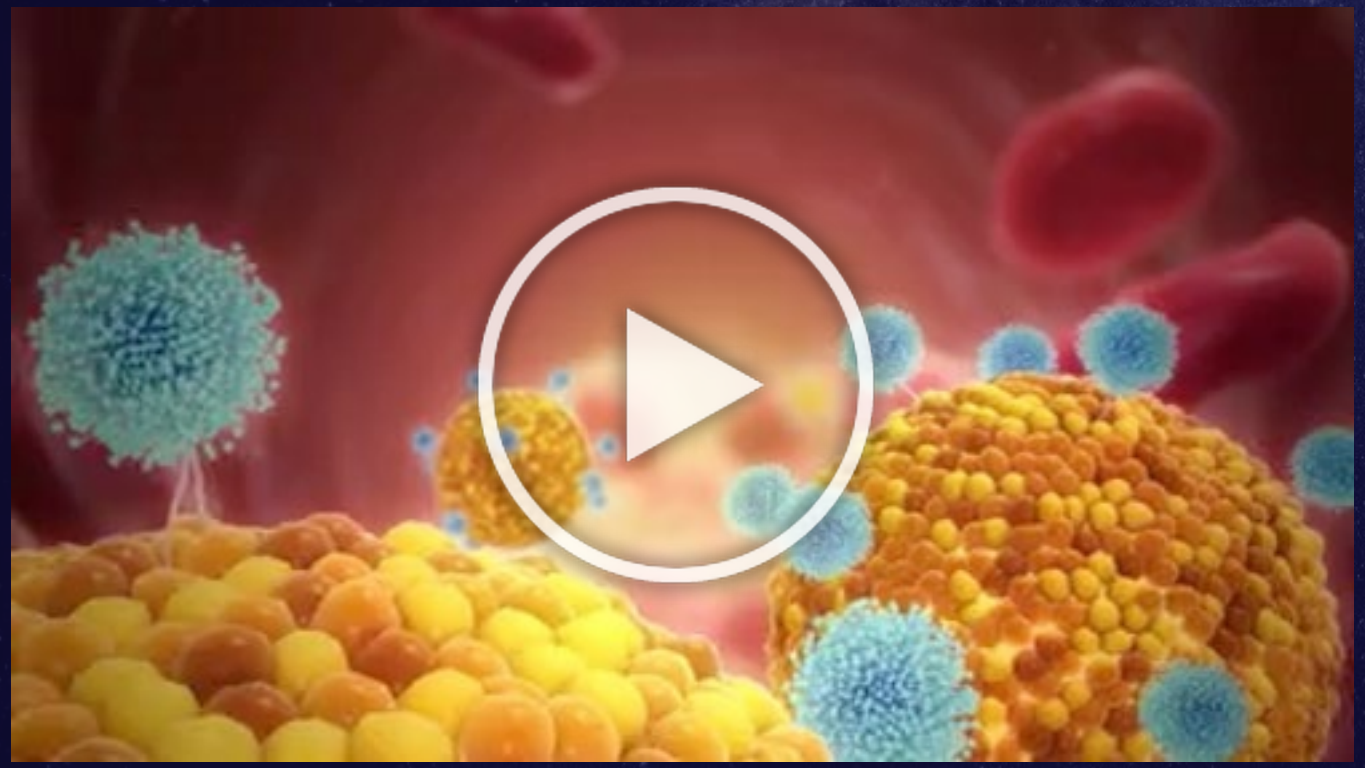
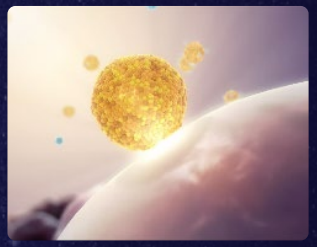
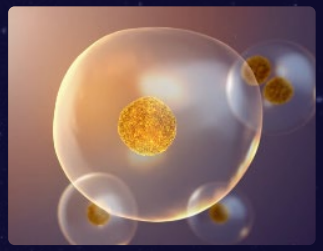


LUNAR Associates with Cell Membrane



Enters Cell
Via Endocytosis

Lipid Particle in Endosome

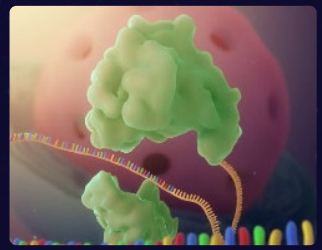


Increased Acidity as
Endosome Ages

pH-Mediated Disruption

Rapid Biodegradation
of Vehicle

RNA in Cytosol



RNA Processing
and Translation

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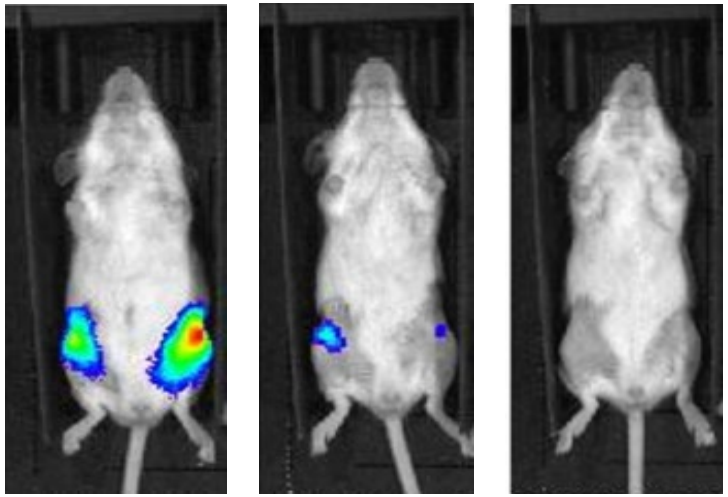
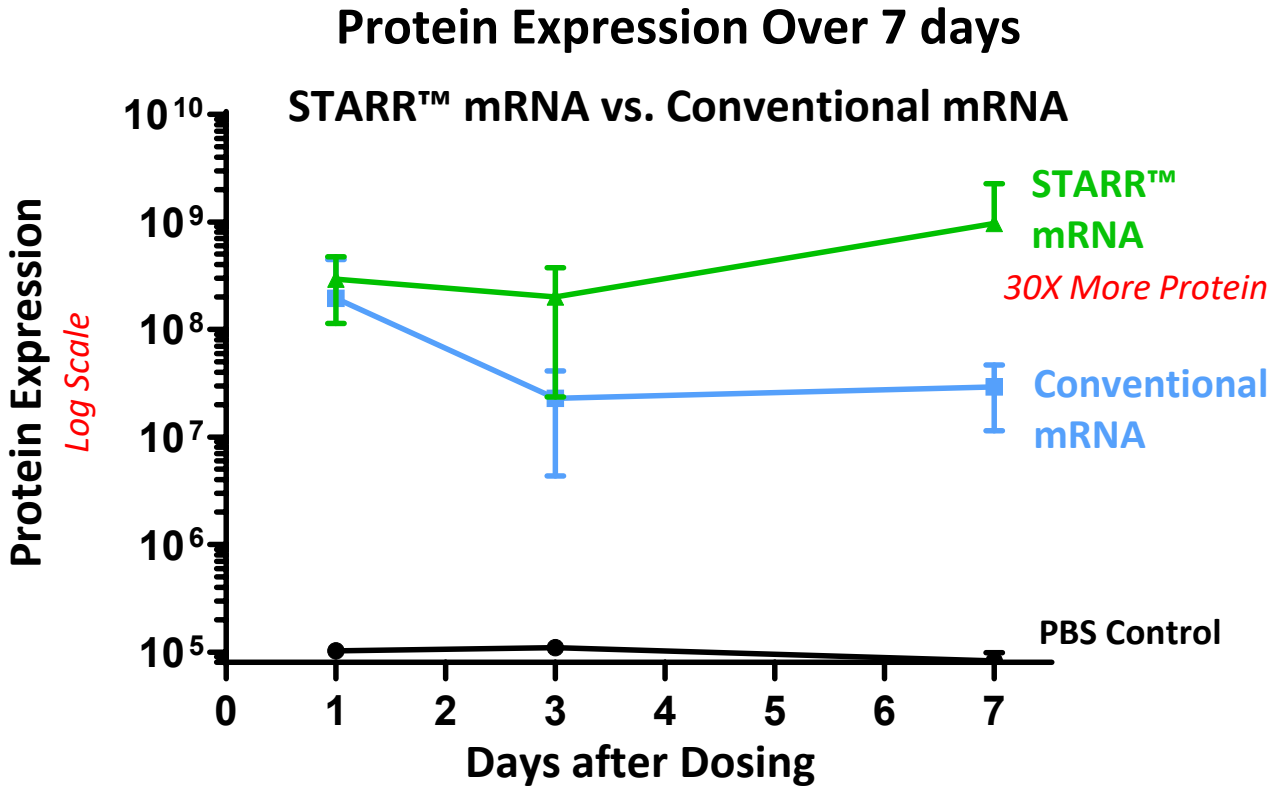
STARR™ mRNA Superior to Conventional mRNA

Self-Transcribing and Replicating mRNA (STARR) delivered with LUNAR® provides higher protein expression and potentially longer-lasting duration of protein expression in mouse

STARR™ Technology
30-Fold Higher Protein Expression



STARR™ Technology | Conventional mRNA | PBS Control



Single Dose of STARR™ mRNA with LUNAR® Delivery Provides Enhanced Protein Expression

Arcturus Pipeline of mRNA Medicines

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Name	Indication	IND/CTA Estimated Timing	Clinical Stage	Route of Administration	Target Organ	Target Cells	Prevalence Worldwide
LUNAR-OTC (ARCT-810)	Ornithine Transcarbamylase (OTC) Deficiency	IND & CTA: Trials Allowed to Proceed	U.S. Phase 1b N.Z. Phase 1	Intravenous (i.v.)	Liver	Hepatocytes	> 10,000
LUNAR-COV19	COVID-19 Vaccine	CTA Summer 2020	Preclinical	Intramuscular (i.m.)	Muscle	Myocytes Dendritic Cells	Global
LUNAR-CF	Cystic Fibrosis	IND 2021	Preclinical	Inhaled Aerosol	Lung	Bronchial Epithelial Cells	> 70,000
LUNAR-CV	Rare Cardiovascular Disease	IND 2022	Preclinical	Intravenous (i.v.)	Liver	Hepatocytes	Undisclosed
LUNAR-MD	Rare Metabolic Disease	IND 2022	Preclinical	Intravenous (i.v.)	Liver	Hepatocytes	Undisclosed

- **LUNAR-OTC (ARCT-810): Phase 1b & Phase 1 Clinical Trials Allowed to Proceed Under IND & CTA, Respectively**
- **LUNAR-COV19: CTA Filing Target Summer 2020**
- **LUNAR-CF: IND Application Filing Target 2021**

Arcturus Platform: Enabling Genetic Medicines

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Program	Partner	Indication	Arcturus Chemistry	Arcturus Delivery	Program Status
LUNAR-GSD3	ultragenyx pharmaceutical	Glycogen Storage Disease Type III	mRNA	LUNAR® Hepatocytes	Target IND 2021
LUNAR-RARE	ultragenyx pharmaceutical	Undisclosed Rare Disease	mRNA	LUNAR® Hepatocytes	Preclinical
LUNAR-HBV	Johnson & Johnson	Hepatitis B	RNA	LUNAR® Hepatocytes	Preclinical
LUNAR-NASH	Takeda	NASH	RNA	LUNAR® Stellate Cells	Preclinical
LUNAR-RPL	**Large Pharma**	Infectious Disease Prophylactic Vaccines	SGI's Replicon RNA	LUNAR®	Preclinical
LUNAR-AH	**Large Animal Health Pharma**	Infectious Disease Prophylactic Vaccines	SGI's Replicon RNA	LUNAR®	Preclinical

- **Greater than $1 Billion in Potential Milestones & Royalties**
- **Enabling Different Types of RNA – Messenger RNA, Gene Editing RNA, Replicon RNA**
- **Multiple Cell Types Targeted**
- **LUNAR-GSD3 (UX053) partnered with Ultragenyx – IND Target 2021**

Arcturus Developing COVID-19 Vaccine with Duke-NUS

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Arcturus Duke-NUS Partnership Initiated March 4, 2020

- Duke-NUS Medical School is an academic world leader in coronaviruses and infectious diseases
- Funded, up to $10M

DukeNUS
Medical School

Arcturus COVID-19 Vaccine Benefits From Duke-NUS Genetic Correlation System

- Helps Arcturus learn more quickly about the LUNAR-COV19 efficacy and safety profile
- Specific gene changes correlate with efficacy and safety
  - Level of neutralizing antibody titers
  - Safety-related adverse events (headache, fever)
- Gene expression changes can be measured within the first 5 days following vaccination

The data generated from the Duke-NUS system gives Arcturus the ability to more efficiently select the dose and streamline the vaccine development program, and potentially accelerate timeline

Arcturus COVID-19 Vaccine has Significant Advantages

Very Low Dose

- Result of combining Arcturus' LUNAR® and STARR™ technologies
- Means potentially more people vaccinated per manufactured batch

Potential Single Shot

- Small, single intramuscular injection
- Simpler logistics for vaccinating large populations

Utilizes STARR™ mRNA

- STARR™ mRNA produces 30X more protein than conventional mRNA
- Lasts longer, booster shot may be unnecessary

Contains No Viruses or Viral Material

- No dead viruses, no attenuated viruses, no virus or viral vectors (AAVs) used to deliver the RNA vaccine
- LUNAR® Delivery Technology is Non-Viral

Readily Manufactured

- Arcturus Proprietary Processes
- Proven; Scalable; High yields; High purities

OTC Deficiency Market Opportunity

Ornithine Transcarbamylase (OTC) Deficiency: The most common urea cycle disorder

- The urea cycle converts neurotoxic ammonia to water-soluble urea that can be excreted in urine
- Deficiency in OTC causes elevated blood ammonia, which can lead to neurological damage, coma, and death
- 10,000 worldwide prevalence

Unmet Medical Need

- Present standard of care involves a strict diet (low protein, high fluid intake) plus ammonia scavengers (sodium phenylbutyrate)
- Present standard of care does not effectively prevent life-threatening spikes of ammonia
- Severe OTC Deficiency patients are typically referred for liver transplant, currently the only cure

LUNAR-OTC Aims to Restore Enzyme Function

- Expression of OTC enzyme in liver has potential to restore normal urea cycle activity to detoxify ammonia, preventing neurological damage and removing need for liver transplantation

LUNAR-OTC (ARCT-810) Clinical Plan

Two Single Dose Studies to Initiate in 2020

- Phase 1b clinical trial in up to 12 stable OTC-deficient patients – IND allowed to proceed in the U.S.
- Phase 1 clinical trial in up to 30 healthy volunteers – Clinical Trial Application (CTA) approved in New Zealand

Primary Goal: Identify safest doses to take forward into multiple dose clinical trials
Primary Endpoints: Safety and tolerability
Exploratory Endpoints: Biomarkers include ureagenesis, plasma ammonia levels and OTC enzyme activity, urine orotic acid levels

Dosing

- Single ascending dose (SAD) studies; randomized, placebo controlled and blinded
- Healthy volunteer study – up to 5 dose levels; Patient study – up to 3 dose levels
- All doses are within the anticipated range for therapeutic biological effect

Timing of Enrollment *(dependent on COVID19 status)*

- Healthy volunteers in New Zealand – Soon
- OTC-deficient patients in the U.S. – Q3/Q4 2020

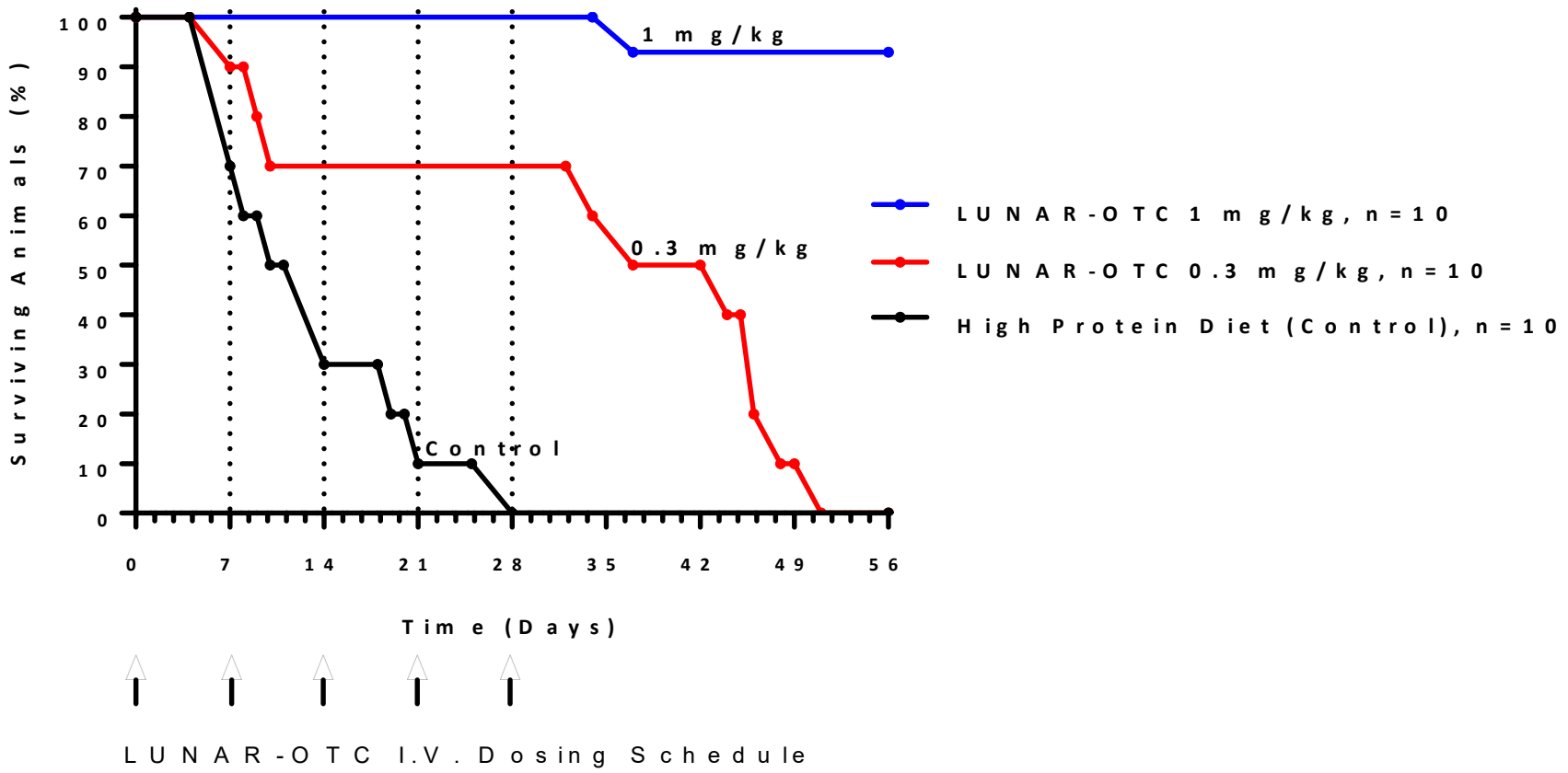
Timing of Human Data *(dependent on COVID19 status)*

- Phase 1 healthy volunteer study
  - First results will be safety – within ~6 weeks after dosing each cohort
  - Study targeted to complete in Q4 2020
- Phase 1b patient study
  - Longer to recruit – typical for rare disease studies
  - Estimated timing of data to be provided when study initiates

LUNAR®-OTC

Disease Normalization Following Single and Repeat Dosing in OTC Mouse Model

Survival of OTC-deficient Mice on High Protein Diet - Weekly LUNAR-OTC Treatment



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LUNAR-OTC

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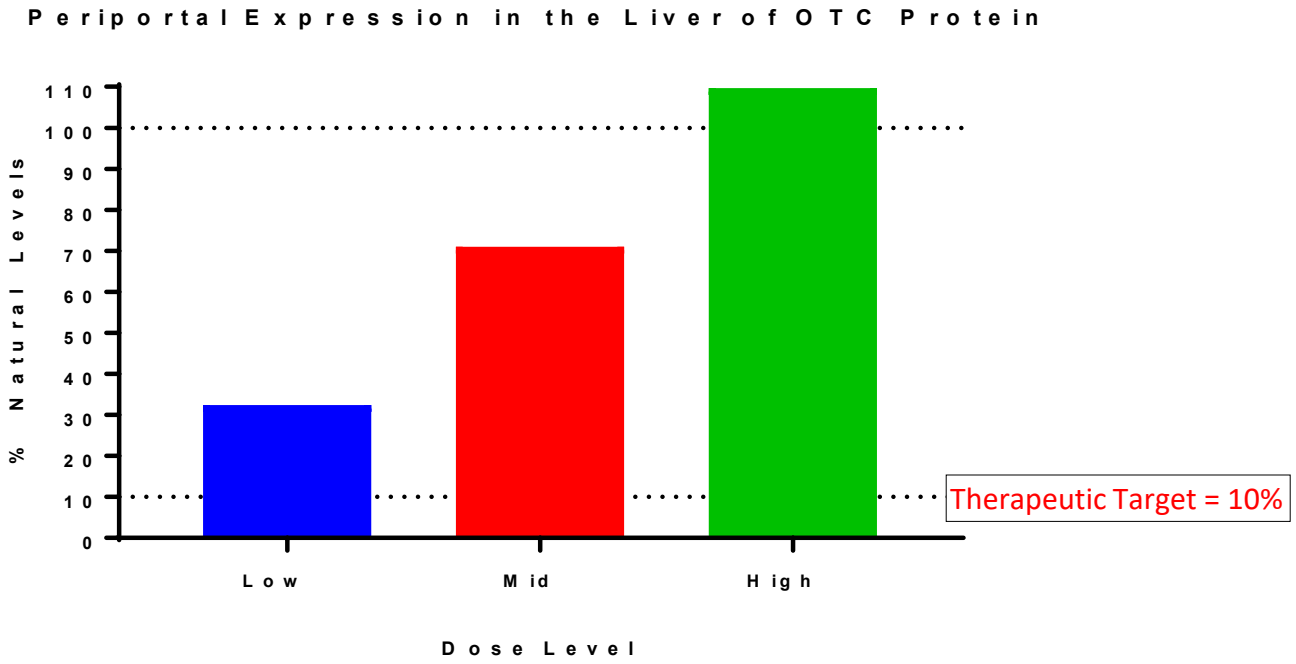
Exceeds Therapeutic Target of 10% Enzyme Replacement at all Doses in OTC-Deficient Mouse Model

- OTCD impacts ureagenesis (ammonia detoxification)
- The main site of ureagenesis is the periportal region of the liver*
- Establishing 10% of natural enzyme levels is expected to be therapeutically significant



*Li, L. et al. PGC-1α Promotes Ureagenesis in Mouse Periportal Hepatocytes through SIRT3 and SIRT5 in Response to Glucagon. Scientific Reports. 6:24156 | DOI: 10.1038/srep24156, April 2016
*Lamers, W.H., Hakvoort, T.B.M., and Köhler, E.S. 'Molecular Pathology of Liver Diseases' in Monga S.P.S. (ed.), *MOLECULAR PATHOLOGY LIBRARY SERIES*, Springer Publishing, New York, pp. 125-132 | DOI: 10.1007/978-1-4419-7107-4

LUNAR-OTC treatment increases OTC expression in mouse periportal hepatocytes (main site of ureagenesis)

Cystic Fibrosis Market Opportunity

Cystic Fibrosis: The most common rare disease in the United States

- Caused by genetic mutations in the CFTR gene, resulting in aberrant flux of ions in and out of cells, causing thick mucus buildup in lung airways
- Chronic airway obstruction leads to infection and inflammation, which causes permanent tissue scarring and respiratory failure
- 70,000 worldwide prevalence

Unmet Medical Need

- No CFTR functional corrector is approved for treatment of all patients
- Present standard of care does not effectively prevent long-term effects of mucus accumulation. CF patients with late-stage loss of respiratory function require lung transplant

LUNAR-CF Aims to Restore CFTR Function

- An mRNA replacement therapy has the potential to deliver a new copy of CFTR into the lungs of CF patients, independent of any genotype
- A functional CFTR protein can restore chloride channel efflux in the airways, reducing mucus accumulation, tissue scarring and minimizing the progressive respiratory dysfunction observed in CF patients

LUNAR® Delivery of mRNA to Lung (Mouse)

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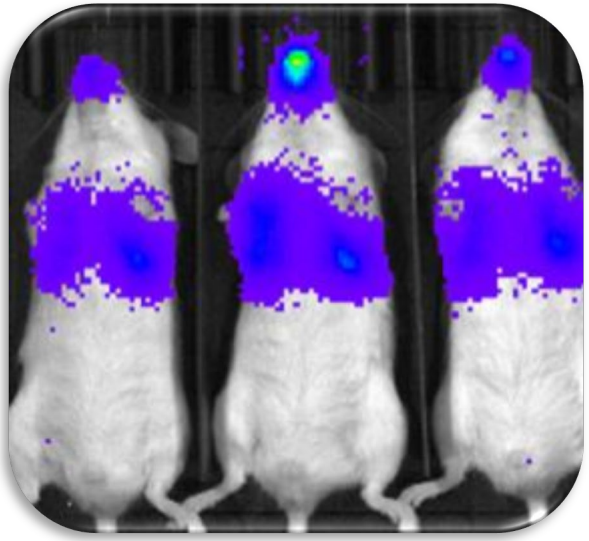
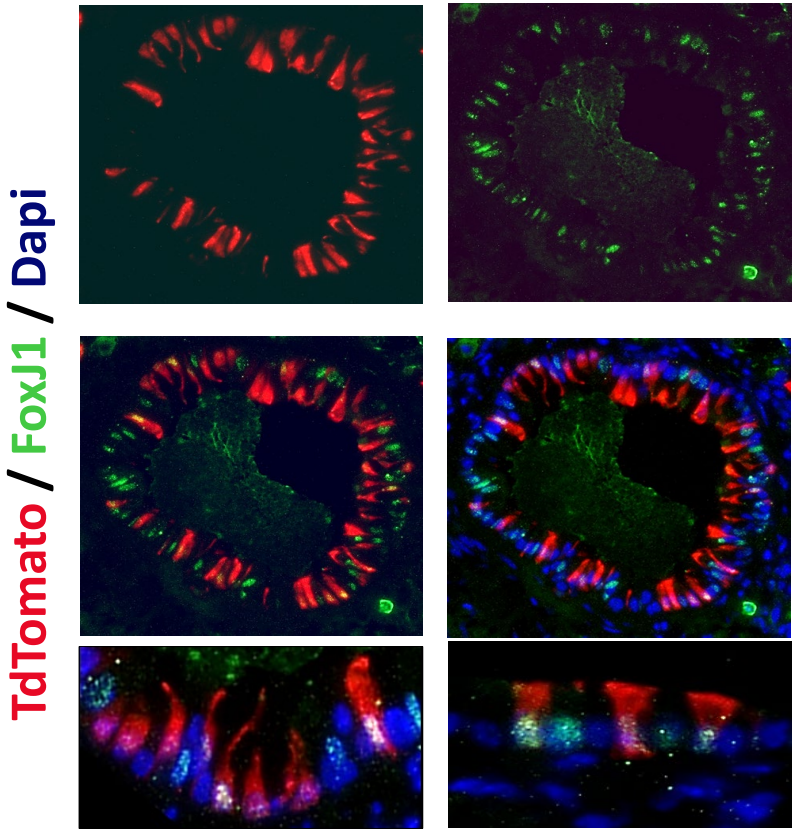
Nebulization



LUNAR® + Luciferase mRNA

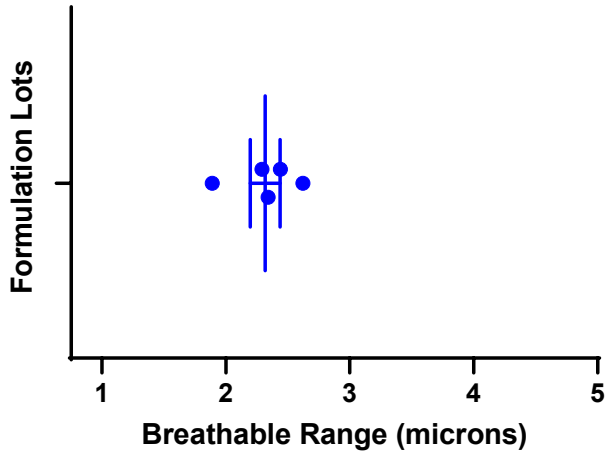
LUNAR® Delivery of mRNA into Bronchial Epithelial Cells (BECs)



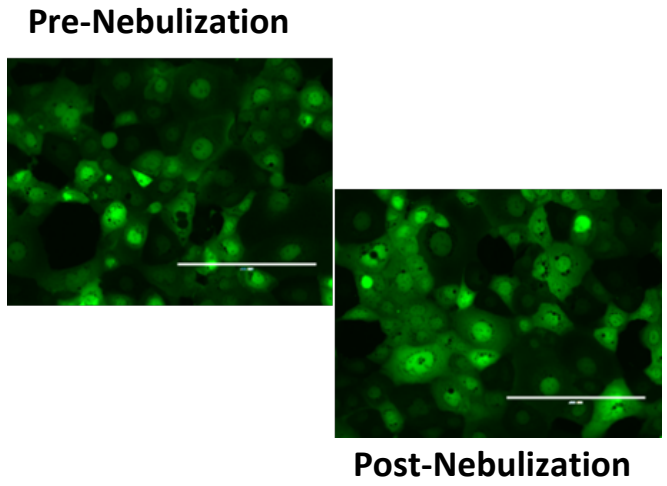
Functional Nebulized Delivery of LUNAR®+ mRNA into Lung Epithelial Cells

Aerosolized LUNAR®

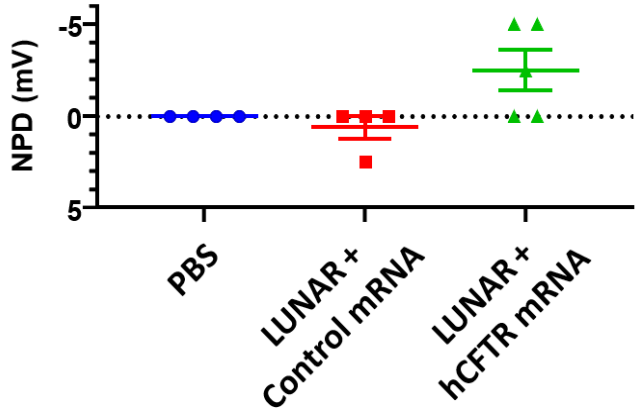
**Aerosolized LUNAR®
Particles are Breathable**



**Aerosolized LUNAR® -mRNA (GFP)
maintains activity**



**Aerosolized LUNAR®-CF
is functional *in vivo* (mouse)**



Aerosolized LUNAR® Droplets are in the Optimal Breathable Range (2-3 microns)
Aerosolized LUNAR® Maintains Function as Measured by GFP Protein Expression & Nasal Potential Difference (NPD)

Arcturus Safety Profile

External Validation

- Multiple strategic partnerships over many years confirms the positive safety profile of Arcturus LUNAR® and mRNA

Arcturus is committed to developing safe mRNA products

- 15 studies over several years with strategic partners

Top Safety Concern for RNA Medicines is Delivery

Arcturus LUNAR® Delivery Technology is well tolerated in non-human primates (NHPs)

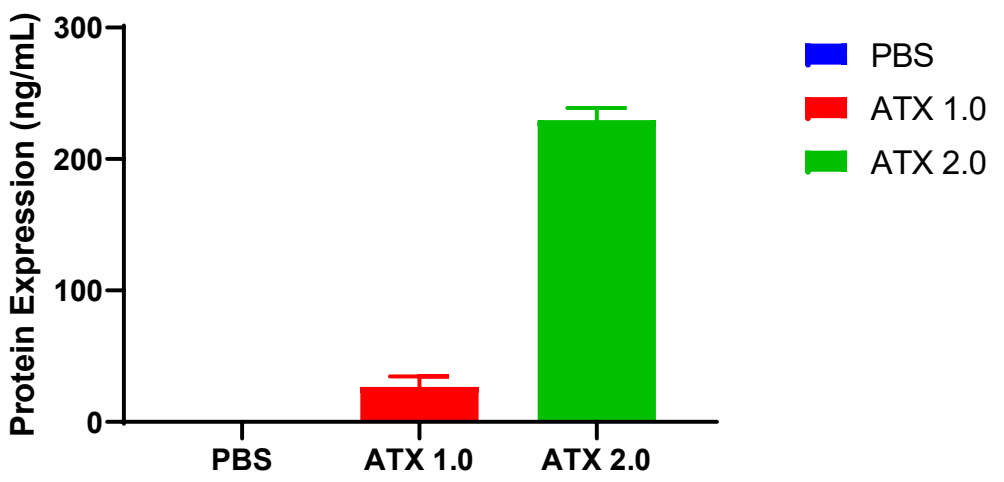
- ✓ **@ 15 mg/kg single dose of non-coding siRNA**
- ✓ **@ 3 mg/kg x eight (8) weekly doses of non-coding siRNA (total of 24 mg/kg over 2 months)**

Arcturus mRNA chemistry shows promising efficacy and tolerability data

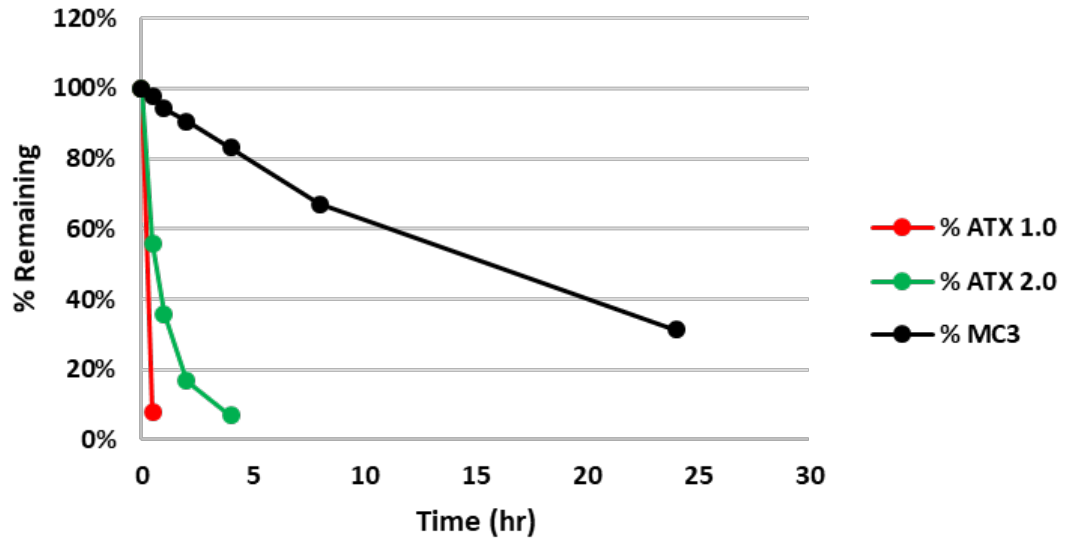
- Efficacy of OTC mRNA in mouse model @ 0.1 – 1 mg/kg

ATX Lipids are Effective and Degrade Rapidly

Protein Expression (*in vivo*)



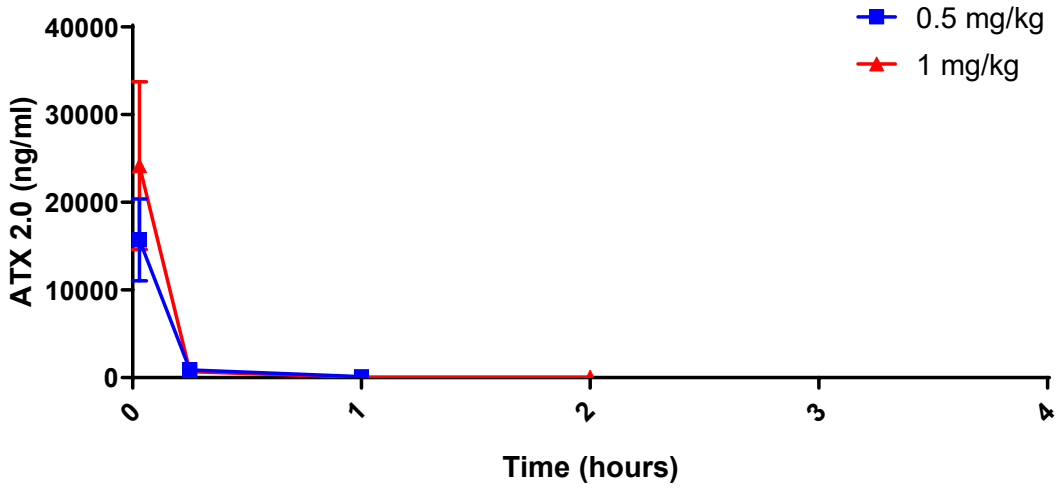
Esterase Catalyzed Degradation (*in vitro*)



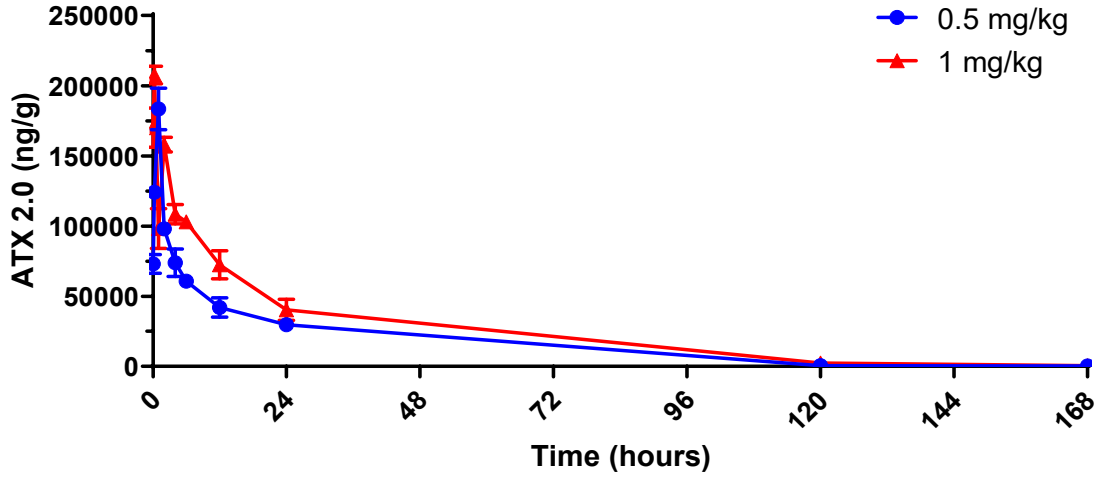
Next Generation ATX Lipids Retain Degradability & Improve Delivery Efficiency

ATX 2.0 Lipid Rapidly Clears *in vivo*

Plasma



Liver



- ATX Lipid (the major component in LUNAR® technology) is rapidly degraded *in vivo*
- ATX Lipid Half-Life in the Liver is Approximately 20 hours

Drug Substance: mRNA Design

Arcturus' proprietary mRNA optimization platform

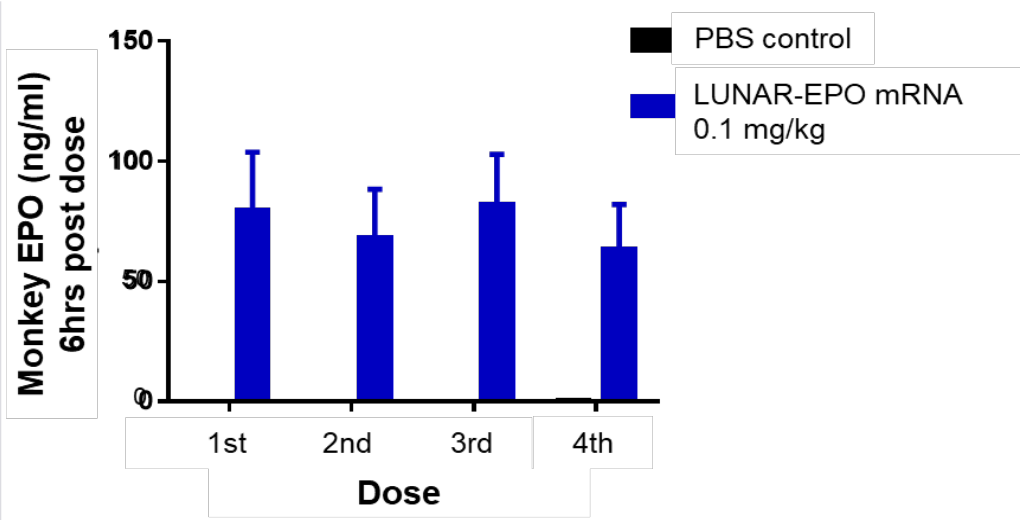
Optimize
mRNA sequence
Chemistry
Process

⇨

Improve
Protein Expression
Duration
Functional Activity

Sustained hEPO activity in NHPs upon repeat dosing

Weekly Dosing in Non-Human Primates (NHPs)



Proprietary mRNA Optimization Platform Demonstrates Sustained Activity Upon Repeat Dosing in NHPs

Drug Substance (mRNA) Manufacturing

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Features	Benefits
Optimized IVT Method	Reduced Cost; Higher Purity
Improved Capping Reaction	Reduced Cost of Goods
Proprietary Purification Process	Higher Purity in a Shorter Time
Efficient	Entire Process Less Than One Week
Scalable to > 1Kg	Access Large Patient Populations
Adaptable	Can Utilize a Variety of Modifications

Arcturus Internal non-GMP mRNA Production Capabilities: Up to 30 g in Less Than One Week

Drug Substance (mRNA) Manufacturing

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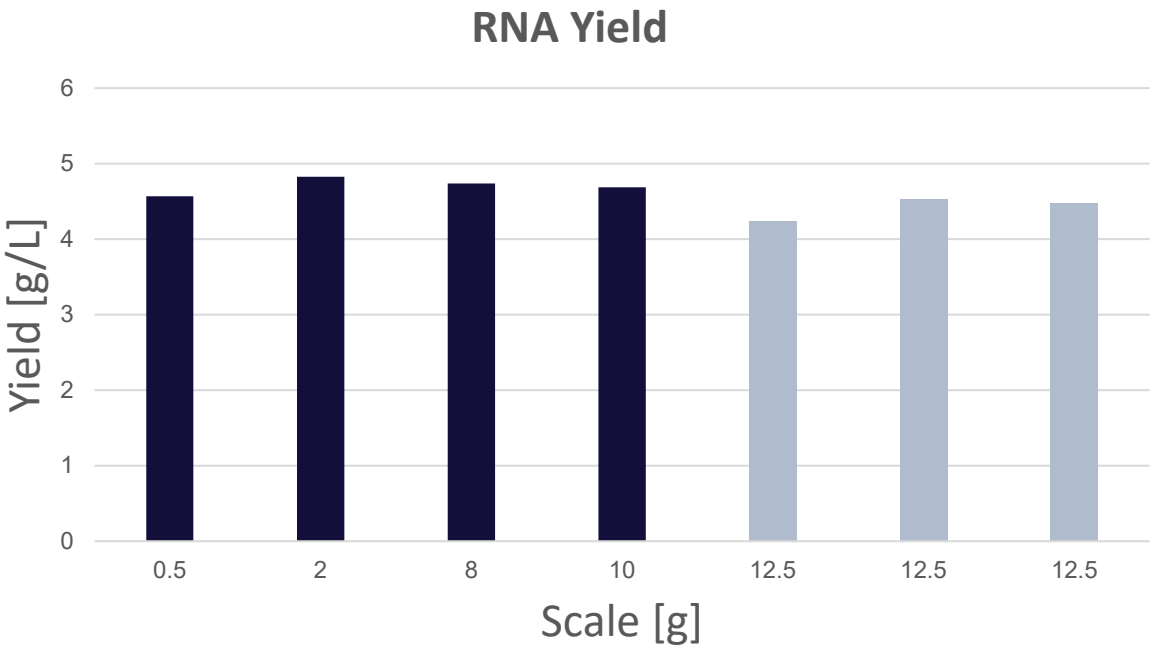

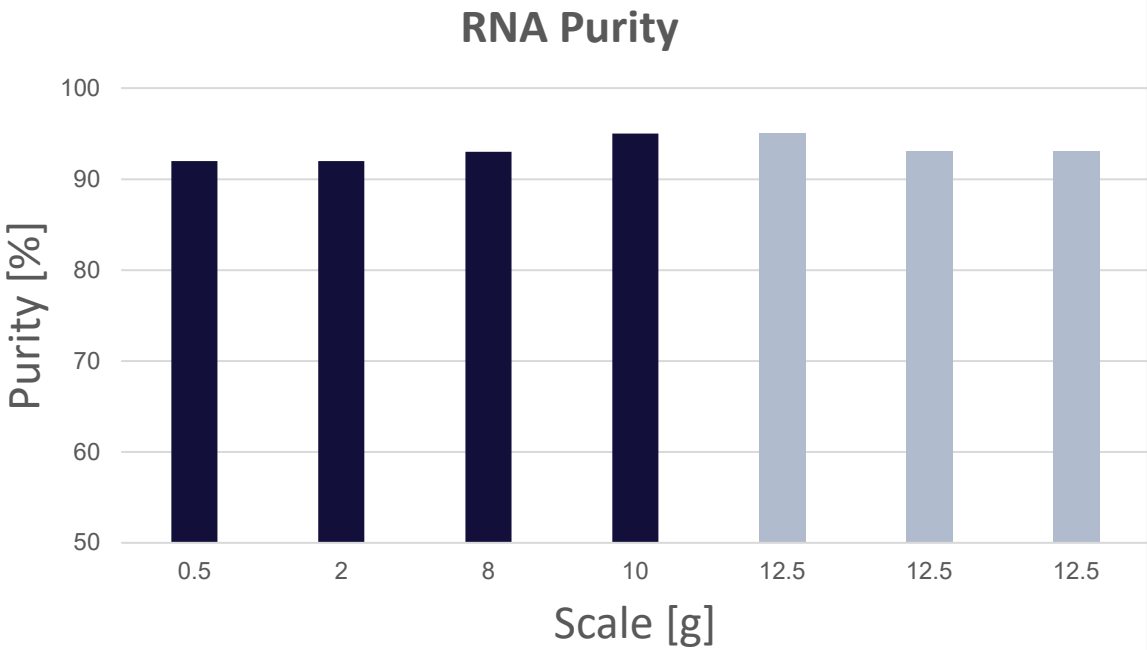

Non-GMP Lots Produced at Arcturus

GMP Lots Produced at CMO as part of recent GMP campaign

Three 12.5 g lots produced in recent GMP campaign are of equivalent quality and yield

Drug Product (LUNAR® + mRNA) Manufacturing

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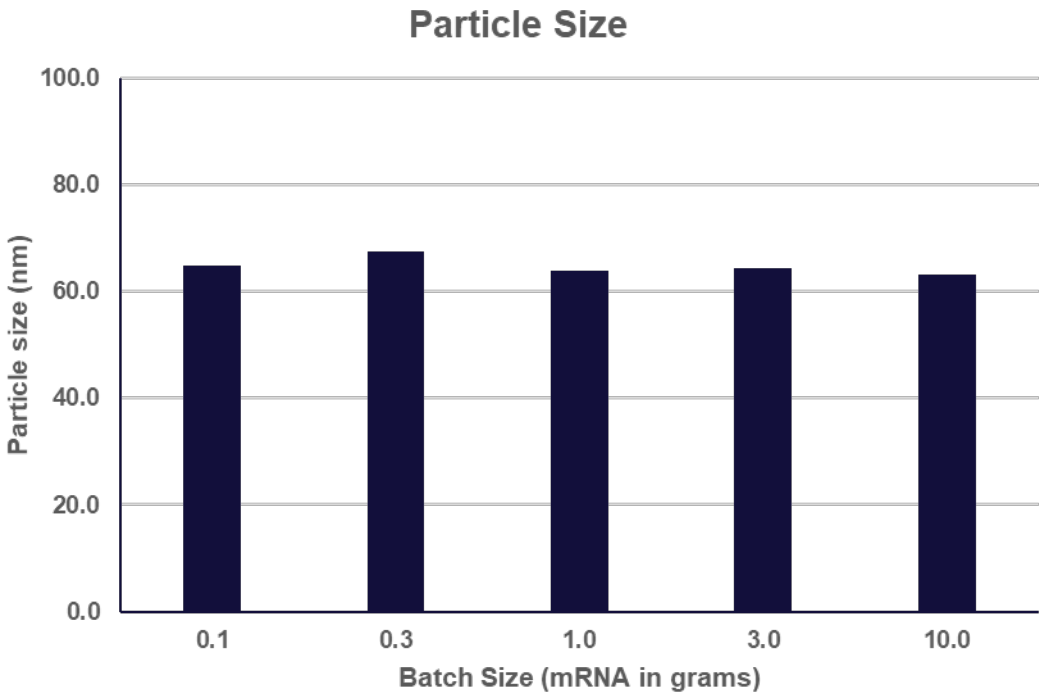

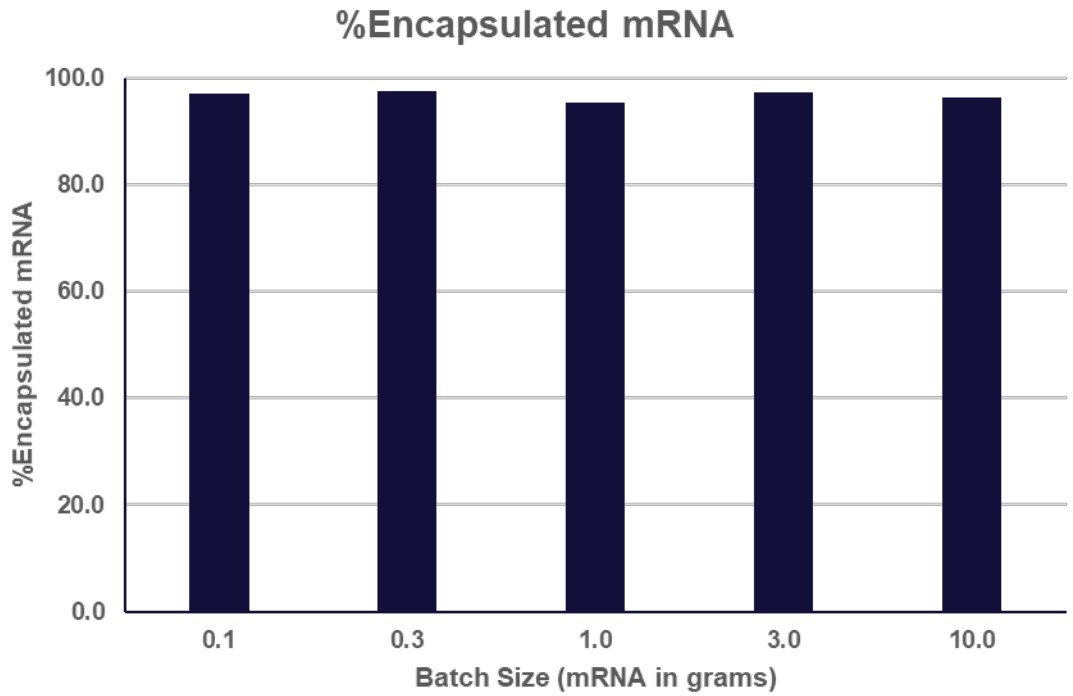

- **Manufacturing of Drug Product Demonstrated up to Multigram Scale with Yields ≥ 85%**
- **GMP Batch of LUNAR®-OTC (ARCT-810) Drug Product Manufactured and Released**

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