ARCTURUS THERAPEUTICS

Building Next Generation of the RNA Medicines

May 2019

ARCTURUS THERAPEUTICS

BUILDING INNOVATIVE
RNA MEDICINES

FORWARD LOOKING STATEMENTS

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Investment Highlights

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Arcturus is an mRNA Medicines Drug Development Company Focused on Rare Diseases

LUNAR® Delivery Platform Validated by Multiple Strategic Partners

- More than $1 Billion in potential milestones and royalties

Broad and Strong Intellectual Property Portfolio

- 152 Patents & Patent Applications
- LUNAR® Delivery Technology
- RNA Drug Substance & Drug Product Process Manufacturing

HQ: **San Diego**; Founded: **2013**; Nasdaq: **ARCT**
Outstanding Shares: **10.8 M;** Employees: **80;**
Insider Ownership: **24%**

Promising Preclinical Safety Data for LUNAR® Delivery and mRNA Drug Products

Key Value Drivers: mRNA Medicines & Platform

Arcturus LUNAR® Delivery Platform: Enabling Genetic Medicines

***Strategic Partners**: More than $1 Billion in Potential Milestones & Royalties*

Arcturus mRNA Medicines

***LUNAR-OTC (ARCT-810)** to treat Ornithine Transcarbamylase (OTC) Deficiency*

OTC Deficiency market potential $500M annual sales

***LUNAR-CF** to treat Cystic Fibrosis (CF); Funded by the*

Class I CF market potential $900M annual sales

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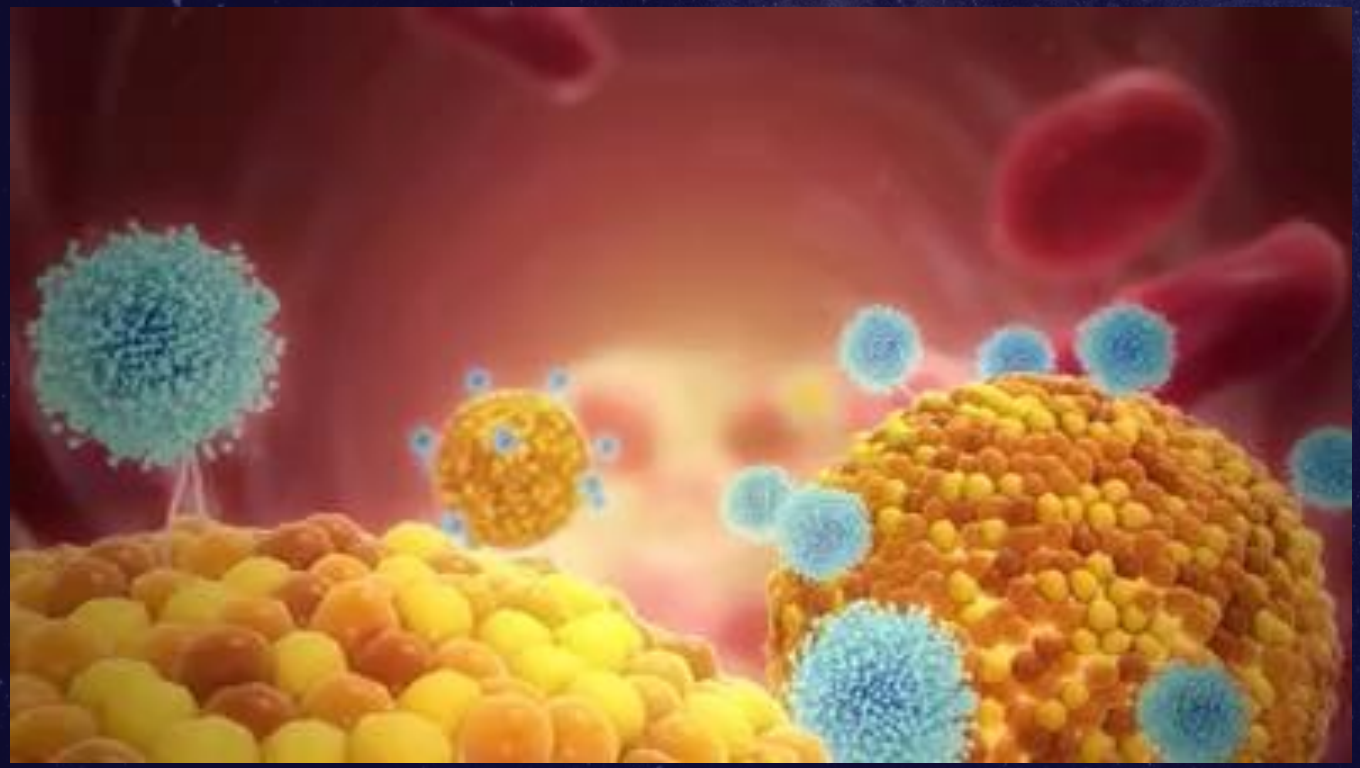
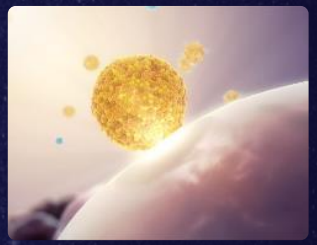
LUNAR® Mechanism of Delivery

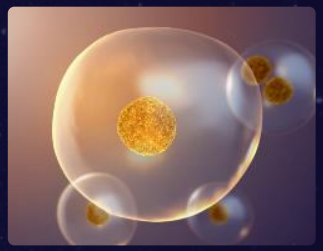


LUNAR Associates with Cell Membrane



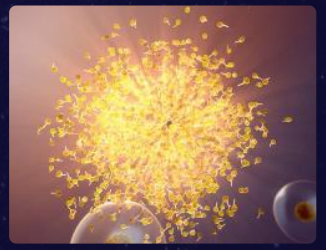
Enters Cell
Via Endocytosis

Lipid Particle in Endosome



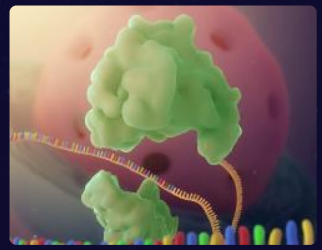
Increased Acidity as
Endosome Ages

pH-Mediated Disruption



Rapid Biodegradation
of Vehicle

RNA in Cytosol



RNA Processing
and Translation

Arcturus Platform: Enabling Genetic Medicines

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Name	Partner	Year of Initiation	Indication	Arcturus Chemistry	Arcturus Delivery	mRNA Process
LUNAR-HBV	Johnson & Johnson	2015	Hepatitis B	RNA	LUNAR® Hepatocytes	ARCT
LUNAR-NASH	Takeda	2017	NASH	RNA	LUNAR® Stellate Cells	ARCT
LUNAR-GSD3	ultragenyx pharmaceutical	2016	Glycogen Storage Disease Type III	mRNA	LUNAR® Hepatocytes	ARCT
LUNAR-RARE	ultragenyx pharmaceutical	2016	Rare Disease	mRNA	LUNAR® Hepatocytes	ARCT
LUNAR-RPL	SYNTHETIC GENOMICS	2017	Vaccines	SGI's Replicon RNA	LUNAR® Intramuscular	SGI

- **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® Platform Substantial Preclinical Proof-of-Concept Demonstrated

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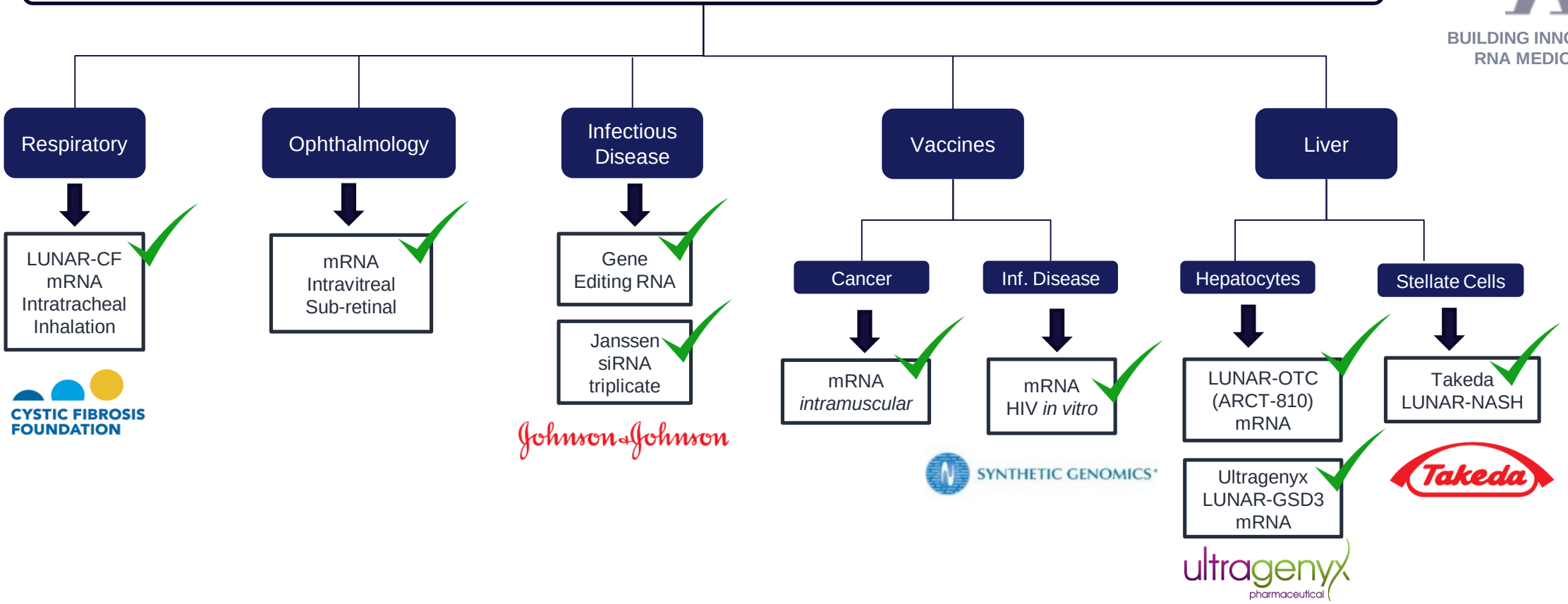
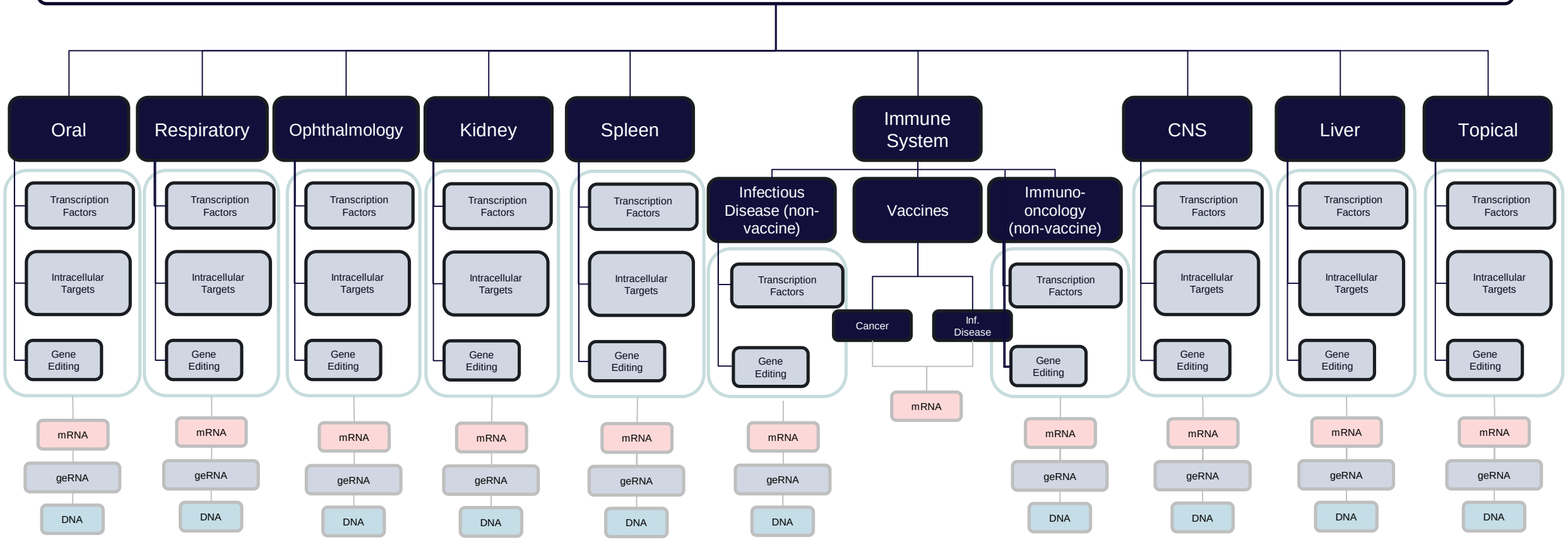


LUNAR® Platform Preclinical Proof-of-Concept Demonstrated in Hepatocytes, Liver Stellate Cells, Bronchial Epithelial Cells (Lung), Subretinal / intravitreal (Eye), Infectious Diseases, Cancer Vaccines

Target Opportunities for LUNAR® Delivery Platform Exceed $100 Billion in Potential Value



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Arcturus Pipeline of mRNA Medicines

Name	Indication	IND Date	Route of Administration	Target Organ	Target Cells	Prevalence Worldwide
LUNAR-OTC (ARCT-810)	Ornithine Transcarbamylase (OTC) Deficiency	Q4 2019	Intravenous (i.v.)	Liver	Hepatocytes	> 10,000
LUNAR-CF	Cystic Fibrosis	H1 2020	Nebulized Aerosol to Lung	Lung	Bronchial Epithelial Cells	> 70,000
LUNAR-2020	Rare Liver Disease	2021	i.v.	Liver	Hepatocytes	
LUNAR-2020	Rare Lung Disease	2021	Nebulized	Lung	Bronchial Epithelial Cells	

- **Arcturus programs focus on messenger RNA (mRNA) drug products for rare diseases**
- **LUNAR-OTC (ARCT-810, intravenous mRNA medicine): Investigational New Drug (IND) Filing Target Q4 2019**
- **LUNAR-CF is funded by the Cystic Fibrosis (CF) Foundation – IND Target H1 2020**
- **If resources are available, we can progress more candidates into the clinic in 2021**

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 spikes of ammonia.
- OTC Deficiency patients are typically referred for liver transplant.

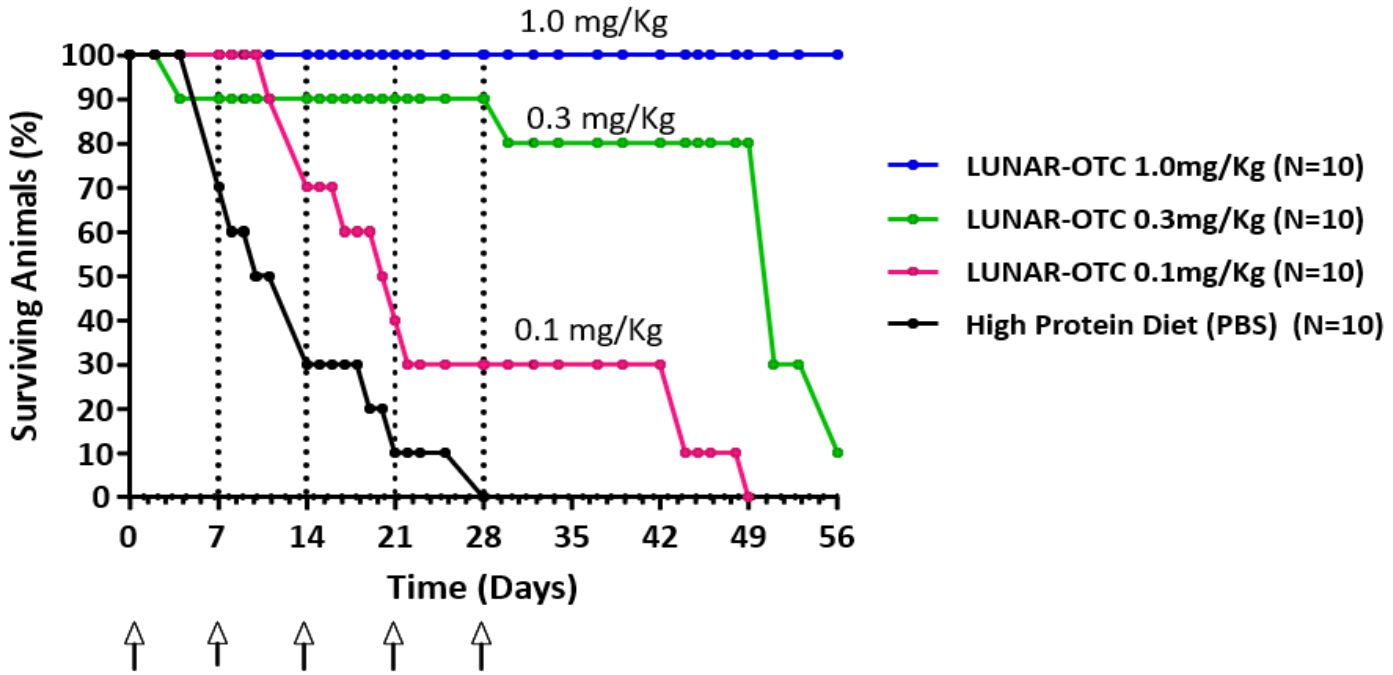
LUNAR-OTC Aims to Restore Enzyme Function

- 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

Disease Normalization Following Single and Repeat Dosing in OTC Mouse Model

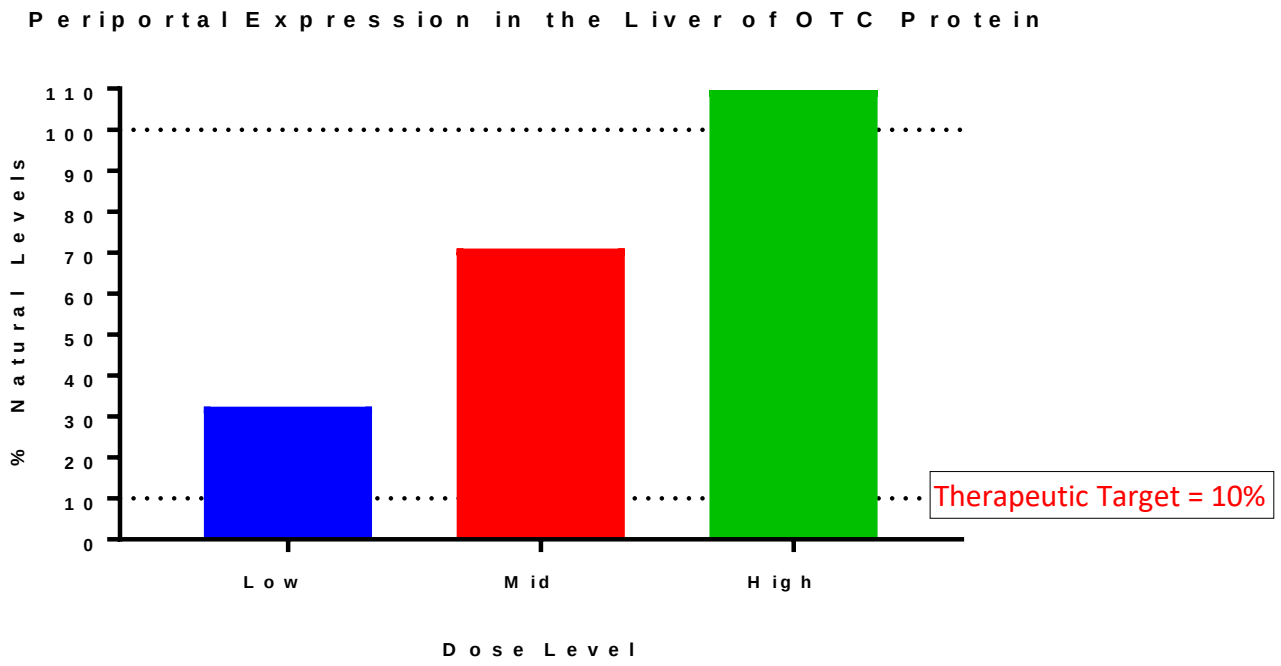
Survival of OTC-deficient Mice on High Protein Diet Weekly LUNAR-OTC Treatment



LUNAR-OTC

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

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

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
- Over $3 Million invested to date

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 RNA
- ✓ @ 3 mg/kg x eight (8) weekly doses of non-coding RNA (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
- Well tolerated in mouse @ 7 mg/kg single dose

IND-enabling toxicology studies at higher doses will provide Maximum Tolerated Dose (MTD)

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

NEBULIZATION AND DELIVERY TO LUNG

LUNAR® Targeting Lung

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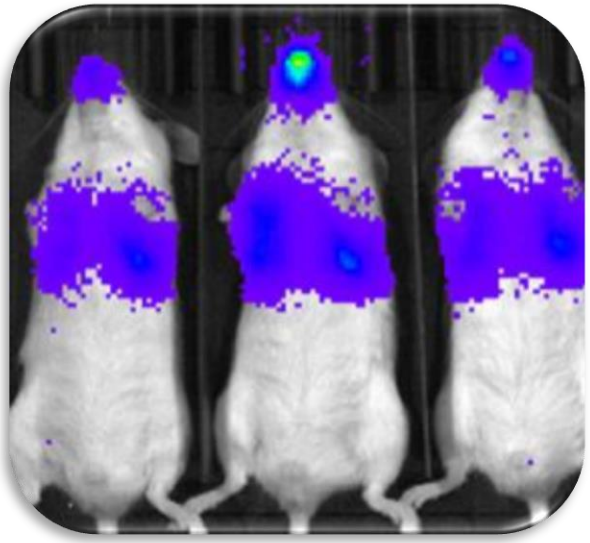
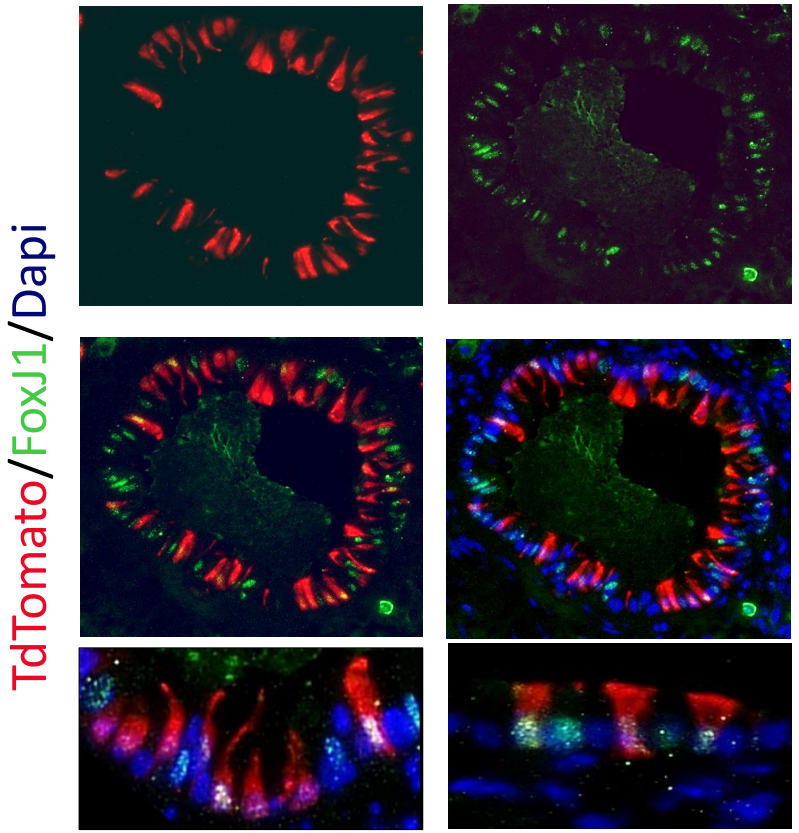
Nebulization



LUNAR-Luc mRNA

LUNAR® Delivery into Bronchial Epithelial Cells (BECs)



Functional Delivery of LUNAR®-mRNA into Lung Epithelial Cells

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Drug Substance: mRNA Design

Arcturus' proprietary mRNA optimization platform

Optimized conditions

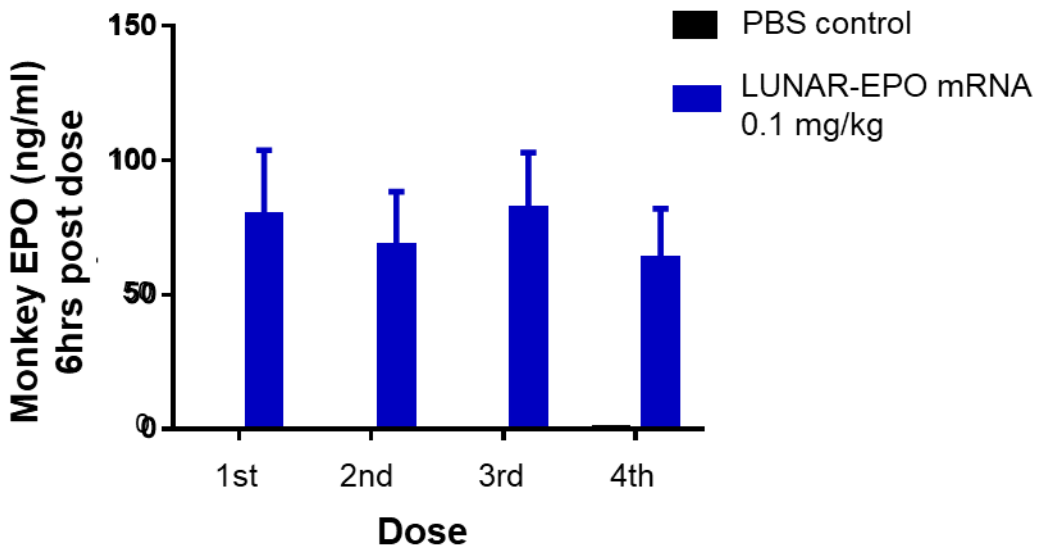
- mRNA sequence
- Chemistry
- Process optimization

→

- Improved protein expression and duration
- Improved functional activity

Sustained hEPO activity in NHPs upon repeat dosing

Weekly Dosing in Non-Human Primates



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

Arcturus 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 mRNA Production: Up to 30 g in Less Than One Week

PROPRIETARY REPRODUCIBLE & SCALABLE PRODUCTION PROCESS

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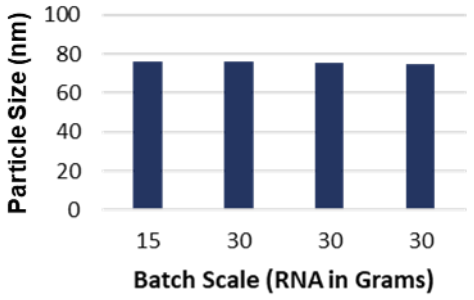
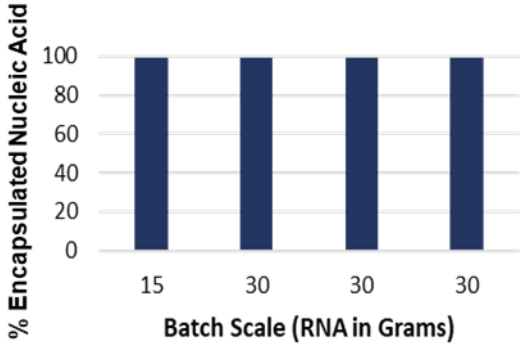
Drug Product: LUNAR® Formulation & Production

LUNAR® Reproducibility

Particle Size

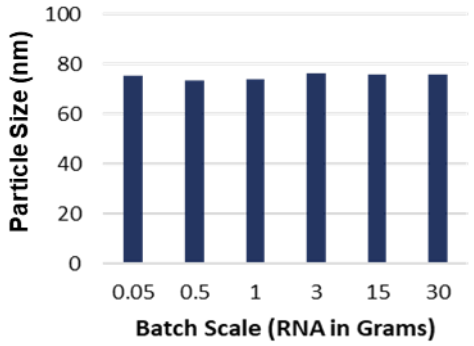


% Encapsulated RNA

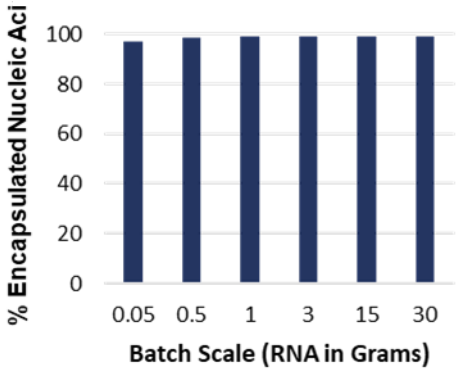


LUNAR® Scalability

Particle Size



% Encapsulated RNA



LUNAR® Has Been Successfully Scaled From Milligram to Multigram Batch Sizes

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