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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
• 182 Patents & Patent Applications
• LUNAR® Delivery Technology
• RNA Drug Substance & Drug Product Process Manufacturing

Promising Preclinical Safety Data for LUNAR® Delivery and mRNA Drug Products
2019 Summary

Ultragenyx Collaboration Expanded, $30M

Cystic Fibrosis Foundation Increased Commitment to $15M

Fundraising Completed, $23M from Institutional Investors

Advanced Pipeline

• **LUNAR-OTC**: ARCT-810 Nominated, Received Orphan Drug Designation from FDA, GMP Manufacturing of Drug Product Completed and Released, On-track for Q1-2020 IND

• **LUNAR-CF**: Preclinical Lung Data Collected, CF Foundation Financial Support Received

Expanded Platform to include STARR Technology™
STARR Technology™ Superior to mRNA in vivo (mouse)

Self-Transcribing and Replicating RNA (STARR) delivered with LUNAR® provides longer-lasting, higher protein expression.
Key Value Drivers → Platform & Pipeline

Platform: LUNAR® Delivery, mRNA Drug Substance, and STARR (Self-Transcribing And Replicating RNA) Technology™

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

Pipeline: Arcturus mRNA Medicines

LUNAR-OTC (ARCT-810) to treat Ornithine Transcarbamylase (OTC) Deficiency
  OTC Deficiency Market Potential $500M Annual Sales
  Orphan Drug Designation is received from U.S. FDA

LUNAR-CF to treat Cystic Fibrosis (CF)
  Class I CF Market Potential $900M Annual Sales
LUNAR® Delivery Technology

- 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

ARCURUS THERAPEUTICS
BUILDING INNOVATIVE RNA MEDICINES
# Arcturus Platform: Enabling Genetic Medicines

<table>
<thead>
<tr>
<th>Program</th>
<th>Partner</th>
<th>Indication</th>
<th>Arcturus Chemistry</th>
<th>Arcturus Delivery</th>
<th>Program Status</th>
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<tbody>
<tr>
<td>LUNAR-GSD3</td>
<td>ultragenyx Pharmaceuticals</td>
<td>Glycogen Storage Disease Type III</td>
<td>mRNA</td>
<td>LUNAR® Hepatocytes</td>
<td>Target IND 2020+</td>
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<td>mRNA</td>
<td>LUNAR® Hepatocytes</td>
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<td>LUNAR-HBV</td>
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<td>LUNAR® Hepatocytes</td>
<td>Preclinical</td>
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<td>LUNAR-NASH</td>
<td>Takeda</td>
<td>NASH</td>
<td>RNA</td>
<td>LUNAR® Stellate Cells</td>
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<td>LUNAR-RPL</td>
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<td>Infectious Disease Prophylactic Vaccines</td>
<td>SGI’s Replicon RNA</td>
<td>LUNAR®</td>
<td>Preclinical</td>
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<td>LUNAR-AH</td>
<td>Large Animal Health Pharma</td>
<td>Infectious Disease Prophylactic Vaccines</td>
<td>SGI’s Replicon RNA</td>
<td>LUNAR®</td>
<td>Preclinical</td>
</tr>
</tbody>
</table>

- 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 2020+
## Arcturus Pipeline of mRNA Medicines

<table>
<thead>
<tr>
<th>Name</th>
<th>Indication</th>
<th>Expected Regulatory Filing Date</th>
<th>Route of Administration</th>
<th>Target Organ</th>
<th>Target Cells</th>
<th>Prevalence Worldwide</th>
</tr>
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<tbody>
<tr>
<td>LUNAR-OTC (ARCT-810)</td>
<td>Ornithine Transcarbamylase (OTC) Deficiency</td>
<td>Q1 2020</td>
<td>Intravenous (i.v.)</td>
<td>Liver</td>
<td>Hepatocytes</td>
<td>&gt; 10,000</td>
</tr>
<tr>
<td>LUNAR-CF</td>
<td>Cystic Fibrosis</td>
<td>2021</td>
<td>Nebulized Aerosol</td>
<td>Lung</td>
<td>Bronchial Epithelial Cells</td>
<td>&gt; 70,000</td>
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<tr>
<td>LUNAR-COV19</td>
<td>Coronavirus COVID-19 Vaccine</td>
<td>2020+</td>
<td>Intramuscular (i.m.)</td>
<td>Muscle</td>
<td>Myocyte</td>
<td>N/A</td>
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<tr>
<td>LUNAR-CV</td>
<td>Rare Cardiovascular Disease</td>
<td>Preclinical</td>
<td>Intravenous (i.v.)</td>
<td>Liver</td>
<td>Hepatocytes</td>
<td>Undisclosed</td>
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<tr>
<td>LUNAR-MD</td>
<td>Rare Metabolic Disease</td>
<td>Preclinical</td>
<td>Intravenous (i.v.)</td>
<td>Liver</td>
<td>Hepatocytes</td>
<td>Undisclosed</td>
</tr>
</tbody>
</table>

- Pipeline programs focus on messenger RNA (mRNA) drug products for rare diseases
- LUNAR-OTC (ARCT-810, intravenous mRNA medicine): IND Filing Target Q1 2020
- LUNAR-CF is funded by the Cystic Fibrosis (CF) Foundation: IND Filing Target 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 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
Survival of OTC-deficient Mice on High Protein Diet - Weekly LUNAR-OTC Treatment

- **LUNAR-OTC 1 mg/kg, n = 10**
- **LUNAR-OTC 0.3 mg/kg, n = 10**
- **High Protein Diet (Control), n = 10**
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

*L i , L .  e t  a l .  P G C - α P ro m o t e s  U re a g e n e s i s  i n  M o u s e  P e r i p o rt a l  H e p a t o c y t e s  t h ro u g h  S I R T 3  a n d  S I R T 5  i n  R e s p o n s e  t o  G l u c a g o n .  S c i e n t i f i c  R e p o r t s .  6 : 2 4 1 5 6 | D O I : 1 0 . 1 0 3 8 / s r e p 2 4 1 5 6 ,  A p ri l 2 0 1 6

*L a m e r s , W . H . , H a k v o o r t , T . B . M . , a n d  K ö h l e r , E . S .  ' M o l e c u l a r  P a t h o l o g y  o f  L i v e r  D i s e a s e s ' i n  M o n g a S . P . S . ( e d . ) , M O L E C U L A R  P A T H O L O G Y  L I B R A R Y  S E R I E S , S p r i n g e r  P u b l i s h i n g , N e w  Y o r k , p p . 1 2 5 - 1 3 2 | D O I : 1 0 . 1 0 0 7 / 9 7 8 - 1 - 4 4 1 9 - 7 1 0 7 - 4

LUNAR-OTC treatment increases OTC expression in mouse periportal hepatocytes (main site of ureagenesis)
ATX Lipids are Effective and Degrade Rapidly

Protein Expression (in vivo)

<table>
<thead>
<tr>
<th></th>
<th>PBS</th>
<th>ATX 1.0</th>
<th>ATX 2.0</th>
</tr>
</thead>
<tbody>
<tr>
<td>Protein Expression (ng/mL)</td>
<td>0</td>
<td>100</td>
<td>200</td>
</tr>
</tbody>
</table>

Esterase Catalyzed Degradation (in vitro)

<table>
<thead>
<tr>
<th></th>
<th>% ATX 1.0</th>
<th>% ATX 2.0</th>
<th>% MC3</th>
</tr>
</thead>
<tbody>
<tr>
<td>% Remaining</td>
<td>120%</td>
<td>100%</td>
<td>80%</td>
</tr>
<tr>
<td>Time (hr)</td>
<td>0</td>
<td>5</td>
<td>10</td>
</tr>
</tbody>
</table>

Next Generation ATX Lipids Retain Degradability & Improve Delivery Efficiency
ATX 2.0 Lipid Rapidly Clears in vivo

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

- Nebulization
- LUNAR® Delivery of mRNA into Bronchial Epithelial Cells (BECs)
- LUNAR® + Luciferase mRNA

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

TdTomato / FoxJ1 / Dapi
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)
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

![Graph showing weekly dosing in NHPs](image_url)
## Drug Substance (mRNA) Manufacturing

### DNA Template Production

### IVT and Capping Reaction

### Purification Process

### Buffer Exchange & Concentration

### Features vs. Benefits Table

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

### Arcturus Internal non-GMP mRNA Production Capabilities: Up to 30 g in Less Than One Week
Drug Substance (mRNA) Manufacturing

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

Non-GMP Lots Produced at Arcturus
GMP Lots Produced at CMO as part of recent GMP campaign
Drug Product (LUNAR® + mRNA) Manufacturing

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

Joseph E. Payne, MSc
President & CEO

Pad Chivukula, Ph.D.
CSO & COO

Andrew Sassine, MBA
CFO

Steve Hughes, M.D.
Chief Development Officer

MERCK
Nitto
Fidelity Investments
IONIS Pharmaceuticals