Forward-Looking Statements

This presentation and the oral commentary contain “forward-looking” statements that involve substantial risks and uncertainties. All statements other than statements of historical facts, including statements regarding our future financial condition, timing for and outcomes of clinical results, business strategy and plans and objectives for future operations, are forward looking statements. These forward-looking statements include terminology such as “believe,” “will,” “may,” “estimate,” “continue,” “anticipate,” “contemplate,” “intend,” “target,” “project,” “should,” “plan,” “expect,” “predict,” “could,” “potentially” or the negative of these terms Forward looking statements are our current statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our ongoing and planned clinical development, the timing of and our ability to initiate or enroll clinical trials, and our ability to make regulatory filings and obtain and maintain regulatory approvals for lonafarnib, ubenimex, PEG IFN Lambda, exendin 9-39 and our other product candidates, our intellectual property position, the potential safety, efficacy, reimbursement, convenience clinical and pharmaco-economic benefits of our product candidates, commercial opportunities, including potential market sizes and segments, our ability to commercialize, expectations regarding clinical trial data and FDA outcomes, our results of operations, cash needs, spending of the proceeds from this offering, financial condition, liquidity, prospects, growth and strategies, the industry in which we operate and the trends that may affect the industry or us.

Forward-looking statements involve known and unknown risks, uncertainties, assumptions and other factors that may cause our actual results, performance or achievements to be materially different from any future results, performance or achievements expressed or implied by the forward-looking statements. Forward-looking statements represent our beliefs and assumptions only as of the date of this presentation. Except as required by law, we assume no obligation to update these forward-looking statements publicly, or to update the reasons why actual results could differ materially from those anticipated in the forward-looking statements, even if new information becomes available in the future.

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Orphan Disease Focus

- 5 - Phase 2 programs in the clinic
- 4 - Well characterized compounds
- 4 - Therapeutically diverse orphan diseases
- Phase 2 POC data generated in lead programs
- Phase 2 data from all programs planned in 6-12 months
- Multiple shots on goal for clinical & regulatory success
Corporate Summary

**Background**
- Founded in 2008
- Went public in March 2016 via reverse merger
- 8.4M common shares outstanding

**Solid Financials**
- $49M cash and investments as of March 31, 2017
- Resources expected to fund operations through mid-2018

**Experienced Team**
## Development Pipeline

<table>
<thead>
<tr>
<th>Product</th>
<th>Indication</th>
<th>Phase II</th>
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</thead>
<tbody>
<tr>
<td>Lonafarnib</td>
<td>Hepatitis Delta</td>
<td></td>
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<tr>
<td>PEG IFN Lambda</td>
<td>Hepatitis Delta</td>
<td></td>
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<tr>
<td>Exendin 9-39</td>
<td>Post-Bariatric Hypoglycemia</td>
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<tr>
<td>Ubenimex</td>
<td>Pulmonary Arterial Hypertension</td>
<td></td>
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<tr>
<td>Ubenimex</td>
<td>Lymphedema</td>
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</table>
Hepatitis Delta Virus
Leads to the Most Severe Form of Viral Hepatitis

- **HDV leads to the most severe form of viral hepatitis**
  - More rapid progression to liver cirrhosis
  - 5-7x more likely to develop cirrhosis and HCC vs HBV

- **HDV is always associated with HBV infection**
  - HDV steals HBsAg from HBV for envelopment

- **Final step in replication involves prenylation**
  - HDV hijacks prenylation, a host process

- **No FDA approved Rx for HDV**
  - PEG IFN-α demonstrates modest benefit

- **HDV worldwide prevalence is 15 - 20 million**
  - Approximately 4-6% of HBV worldwide population is infected with HDV
**Increase in HDV Testing in the U.S.**

*Poster Presentation at Digestive Disease Week 2017*

**Increasing % of Chronic HBV Patients Tested for HDV**

---

**Poster, DDW 2017, “Prevalence of Hepatitis Delta Virus (HDV) Infection in the United States: Results from an ICD-10 Review”**

*Koh, C. et al, AASLD 2014, “Prenylation inhibition with lonafarnib decreases hepatitis D levels in humans”*
Increased HDV Patient Diagnosis
Poster Presentation at Digestive Disease Week 2017

Estimated ~ 110,000 Individuals Co-infected with HBV/HDV in the U.S.
Lonafarnib for HDV
Well-Characterized Clinical Stage Lead Compound

- Small molecule, oral, prenylation inhibitor
- Well-characterized through Phase 3
  - >2,000 patients dosed in oncology program by Merck (Schering)
  - Dose limiting toxicity is GI (class effect)
- Over 120 HDV patients dosed across international sites
- HDV Orphan Designation in US & EU, Fast Track in US
- Prenylation is a host target; potential barrier to resistance

Phase 2 proof of concept study conducted at NIH; NIH Phase 2 study results published: Koh et al, Lancet Infect Dis, 2015.
Sarasar (lonafarnib) is Active Against HDV

Week 4 Reduction in HDV-RNA with Lonafarnib

National Institutes of Health
NIH POC
Lancet ID 2015

Placebo
Lonafarnib
Lonafarnib

100 mg BID
200 mg BID

Mean ∆ - 0.74 Log
Mean ∆ - 0.2 Log
Mean ∆ - 1.6 Log

N = 4
N = 6
N = 6

Sarasar (lonafarnib) is Active Against HDV: Week 4 Reduction in HDV-RNA with Lonafarnib.
Sarasar (lonafarnib) is Active Against HDV

Week 4 Reduction in HDV-RNA with Lonafarnib

**National Institutes of Health**
NIH POC
Lancet ID 2015

Placebo
- Lonafarnib 100 mg BID
- Lonafarnib 200 mg BID

**Ankara University**
LOWR HDV -1
EASL 2015

Lonafarnib 100 mg BID
Lonafarnib 200 mg TID
Lonafarnib 300 mg BID
Lonafarnib + Ritonavir 100 mg QD
Lonafarnib + PEG IFN-α 180 mcg QW

Mean ∆ - 0.74 Log
Mean ∆ - 0.2 Log
Mean ∆ - 1.6 Log
Mean ∆ - 1.2 Log
Mean ∆ - 1.6 Log
Mean ∆ - 2.4 Log
Mean ∆ - 2.0 Log
Mean ∆ - 1.8 Log

N = 4
N = 6
N = 6
N = 3
N = 3
N = 3
N = 3
N = 3
Rapid Decline with Lonafarnib Combinations

Mean Change in Log HDV RNA

-LOWR HDV – 1

Week

-4 -3.5 -3 -2.5 -2 -1.5 -1 -0.5 0

LNF 100 mg BID + PEG IFN α 180 mcg QW (N=3)

LNF 100 mg BID + RTV 100 mg QD (N=3)
Rapid Decline with Lonafarnib Combinations
Larger Declines in HDV-RNA at Week 8 versus PEG IFN-α at Week 48

Mean Change in Log HDV-RNA

-0.5
-1
-1.5
-2
-2.5
-3
-3.5
-4
0
4
8
12
16
20
24
28
32
36
40
44
48

Week

PEG IFN α 180 mcg QW ± tenofovir QD (N=91)

LNF 100 mg BID + PEG IFN α 180 mcg QW (N=3)

LOWR HDV – 1

LNF 100 mg BID + RTV 100 mg QD (N=3)

HIDIT – 2

Hepatitis Delta International Network

Rapid Decline with Lonafarnib Combinations
Larger Declines in HDV-RNA at Week 8 versus PEG IFN-α at Week 48

Mean Change in Log HDV-RNA

-0.5
-1
-1.5
-2
-2.5
-3
-3.5
-4
0
4
8
12
16
20
24
28
32
36
40
44
48

Week

PEG IFN α 180 mcg QW ± tenofovir QD (N=91)

LNF 100 mg BID + PEG IFN α 180 mcg QW (N=3)

LOWR HDV – 1

LNF 100 mg BID + RTV 100 mg QD (N=3)

HIDIT – 2

Hepatitis Delta International Network
LNF 25 mg BID + RTV + PEG IFN α

3 of 5 patients (60%) HDV-RNA PCR-negative @ Week 24
LNF 25 mg BID + RTV
Modest Mean Decline at Week 24

Mean Change in Log HDV RNA

LOWR HDV – 2

LNF 25 mg BID + RTV (n=6)
LNF 25 mg BID + RTV vs PEG IFN α
Modest Mean Decline at Week 24

Mean Change in Log HDV RNA

EOT (LOWR HDV - 2)
EOT (HIDIT - 2)

Week

LNF 25 mg BID + RTV (n=6)
PEG IFN-α 180 +/- TDF (n=91)
LNF 25 mg BID + RTV + PEG IFN α
Most Rapid and Profound Decline in HDV-RNA

Mean Change in Log HDV RNA

-6
-5
-4
-3
-2
-1
0

0 4 8 12 16 20 24 28 32 36 40 44 48

Week

EOT (LOWR HDV - 2)
EOT (HIDIT - 2)

LNF 25 mg BID + RTV (n=6)
Peg IFN-α 180 +/- TDF (n=91)
LNF 25 mg BID + RTV + PEG IFN-α (n=5)
Phase 2: LOWR HDV Program*
Key Findings from EASL 2017

• All-oral LNF 25 or 50 mg BID + RTV suppresses HDV-RNA < LOQ
  - 5 of 14 (36%) patients < LOQ at Week 24
    • 1 patient PCR-negative at Week 24

• Addition of PEG to LNF 25 mg BID + RTV results in highest response rates
  - 4 of 5 (80%) patients < LOQ at Week 24
  - 3 of 5 (60%) patients PCR-negative at Week 24
    • 2 patients PCR-negative at 24 weeks post-treatment **

• 60-78% of patients normalized ALT at Week 24

• AEs predominantly mild / moderate for LNF 25 / 50 mg regimens

* LOWR HDV = LOnafarnib With Ritonavir in HDV
** Low level viremia off-therapy
PEG IFN Lambda for HDV
Well-Characterized Clinical Stage Compound

• A novel, first in class Type III interferon
  - Generated by human immune system

• Binds to a unique receptor
  - Highly expressed on hepatocytes
  - Limited on hematopoietic cells and CNS cells

• Similar downstream signaling pathway as Type I interferons

• >3,000 patients previously dosed in 17 HBV / HCV clinical trials

• Antiviral activity with less of the typical IFN alfa related side effects

• Goal: IFN-lambda to replace IFN-alfa in future Eiger studies
PEG IFN Lambda Safety vs PEG IFN Alfa

Limited Extra-Hepatic Receptor Distribution

Lambda associated with fewer systemic adverse events

<table>
<thead>
<tr>
<th>Adverse Events</th>
<th>Lambda 180 µg % (N = 80)</th>
<th>Alfa 180 µg % (N = 83)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Headache</td>
<td>13.8</td>
<td>28.9</td>
</tr>
<tr>
<td>Myalgia</td>
<td>3.8</td>
<td>21.7</td>
</tr>
<tr>
<td>Pyrexia</td>
<td>10.0</td>
<td>45.8</td>
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<tr>
<td>Pruritus</td>
<td>8.8</td>
<td>15.7</td>
</tr>
<tr>
<td>Neutropenia</td>
<td>2.5</td>
<td>20.7</td>
</tr>
<tr>
<td>Musculoskeletal</td>
<td>6.3</td>
<td>27.7</td>
</tr>
<tr>
<td>Neurological</td>
<td>22.5</td>
<td>36.1</td>
</tr>
<tr>
<td>Flu-like</td>
<td>16.3</td>
<td>54.2</td>
</tr>
</tbody>
</table>

Chan, HLY et al, J Hepatology 2016.
LIMT HDV “Mono” Phase 2 Study
Lambda Interferon MonoTherapy Study in HDV

Bridging to Registration: Supportive Study

On-treatment

48 weeks

Post-treatment

24 weeks

Arm 1

N = up to 15

LMD 120 mcg QW

Follow-up

Arm 2

N = up to 15

LMD 180 mcg QW

Follow-up

Safety / Tolerability
HDV-RNA Negativity
ALT Change
HDV Program Plan
Pathway to Agency Meeting

4Q 2016

LIMT HDV
Enrolling

1Q 2017

LOWR HDV
End of Rx Data Presented

2Q 2017

LOWR HDV
End of Follow Up Data Presented

3Q 2017

DAVDP Agency Meeting

4Q 2017
Potential Approaches for Development*
Goal: Provide Physicians & Patients Multiple Options to Treat HDV

- **Lonafarnib + Ritonavir**  
  All-Oral Rx

- **PEG IFN Lambda**  
  Monotherapy Sub Q Rx

- **Lonafarnib + Lambda ± Ritonavir**  
  Combination Rx

* Investigational treatments to be discussed with regulatory agencies
Bariatric Surgery increasing worldwide
• 200,000 bariatric surgeries in the US in 2015 and growing*

Post prandial hyperinsulinemia and hypoglycemia
• Neuroglycopenia – seizures, loss of consciousness, and even death
• Disability – impaired ability to work, drive, perform daily activities

Impacts 5-10% of Roux-en-Y patients: Orphan Disease
• ~ 30,000 current patients in US (prevalence)

No approved therapy; high unmet medical need

* American Society for Metabolic and Bariatric Surgery 2015
Exendin 9-39 in Post-Bariatric Hypoglycemia

Exendin 9-39 is a GLP-1 Antagonist

- 31 Amino acid fragment of Byetta® (exenatide); a GLP-1 agonist
- Normalizes insulin secretion

Proof of Concept

- 36 patients dosed at Stanford
- Multiple clinical studies completed
- IV infusion and Sub Q administration studied

Previous experience as investigational agent

- >300 patients reported dosed worldwide*

Novel liquid formulation

Orphan Designation granted in US and EU

*ClinicalTrials.Gov
## 3 Clinical Studies of Exendin 9-39 at Stanford

### 36 Patients Dosed

<table>
<thead>
<tr>
<th>Study</th>
<th># Patients</th>
<th>Duration of dosing</th>
<th>Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>IV Infusion</td>
<td>8</td>
<td>Single dose</td>
<td>Completed, Published <em>Diabetologia</em></td>
</tr>
<tr>
<td>Sub Q Injection SAD Study</td>
<td>8</td>
<td>Single dose</td>
<td>Completed, Oral presentation 2016 ADA, Manuscript submitted</td>
</tr>
<tr>
<td>Sub Q Injection MAD Study</td>
<td>20</td>
<td>Up to 3 days BID dosing</td>
<td>Completion in June 2017, Presentation at 2017 ADA</td>
</tr>
</tbody>
</table>

Over 300 patients are reported to have received exendin 9-39 as an investigational agent worldwide.
Sub Q Exendin 9-39 SAD Results
All Doses Therapeutic

Mean Glucose Levels

- Sub Q Ex 9-39 (N=8)
- Baseline (N=8)

Rate of glucose decline reduced

All subjects required rescue

No patient became hypoglycemic

* P < 0.05, ** P < 0.01
**Sub Q Exendin 9-39 SAD Results**

*All Doses Therapeutic*

**Mean Glucose Levels**

- **Sub Q Ex 9-39 (N=8)**
- **Baseline (N=8)**

**Graphical Description:**
- Rapid decline
- Rate of glucose decline reduced
- No patient became hypoglycemic
- All subjects required rescue
- Increase in glucose nadir
- Improvement in patient reported outcomes

*P < 0.05, **P < 0.01*
Exendin 9-39
Clinical and Regulatory Path to Registration

2017

Phase 2
SQ MAD Study
N=20
Phase 2
SQ MAD Data

Phase 1
PK Study
Liquid Formulation

2018

Phase 2
28-day Study
N=24

EOP2
PAH is a $4 Billion+ Orphan Disease market
- Approved agents for PAH are all Vasodilators (palliative)

Inflammation now recognized as major component in PAH
- LTB₄ identified as an inflammatory mediator in PAH

LTB₄ is elevated in PAH animals and human PAH disease
- Targeted inhibition of LTB₄ reverses PAH in animal models

Ubenimex is a targeted inhibitor of LTA₄H
- Approved in Japan for a different indication; well characterized

Potential for PAH Disease Modification & Reversal
Human PAH Lung Tissue and Serum

LTA₄H and LTB₄ Levels are Elevated in PAH

Human Serum LTB₄ (pg/mL)
N=10 PAH Patients*

*Tian et al Hypertension 2015
Ubenimex
Marketed in Japan Since 1987

• Oral, small molecule, LTA$_4$H inhibitor

• Well-characterized, safe and well tolerated

• Approved adjuvant to chemotherapy for non-lymphocytic leukemia in JP

• Never introduced in the US or EU

• Orphan Designation in PAH in US / EU Granted

• US Patent Allowance for Claims in PAH Granted
LIBERTY: Phase 2 Study

A Randomized, Double-Blind, Placebo-Controlled Study of UBEnimex in Patients with Pulmonary ARTerial Hypertension*

Months 1-6

N≈40
Standard of Care¹ + Ubenimex 150 mg TID

N≈20
Standard of Care¹ + PBO

Primary Endpoint:
Pulmonary Vascular Resistance

Secondary Endpoint:
Six Minute Walk Distance (6MWD)

Enrollment Complete

¹ On at least one of PDE5 inhibitor / sGC inhibitor and/or endothelin receptor antagonist and/or prostacyclin
² Enrolling Functional Class 2 and 3
Ubenimex in PAH Timeline

Phase 2 / Phase 3 Plan

- **2016**
  - **Enrollment**
    - LIBERTY
      - Phase 2 Study
      - N = 61
  - **Dose**

- **2017**
  - Enrollment Complete
  - KOL / Analyst Event
    - May 10, 2017
    - NYC

- **2018**
  - Topline Data
    - JPM 2018
  - EOP2
  - Initiate
    - Phase 3 Study
Lymphedema
A Disabling Disorder with Significant Impact on Quality of Life

No Approved Rx Therapy

• Lymphedema is a state of vascular insufficiency
  - Decreased clearance of interstitial fluid through lymphatics
  - Debilitating architectural alterations in skin & supporting tissues
• Primary Lymphedema – hereditary (Orphan)
• Secondary Lymphedema – due to a causative event
• Elevated LTB$_4$ in animal models and human lymphedema
  - Targeted blockade of LTB$_4$ improves preclinical lymphedema
• Potential for Disease Modification & Reversal

LTB₄ is Elevated in Human Lymphedema

Cross section of arm of lymphedema patient

- skin thickening
- muscle atrophy
- Growth of insulatory layer of fat

Human Serum

$P < 0.0001$

A Randomized, Placebo-Controlled Trial to Evaluate Efficacy, Safety, and Tolerability of Ubenimex in Patients with Lymphedema

Entry Criteria:
Secondary lymphedema of the lower limbs

Primary Endpoint:
Skin Thickness

Secondary Endpoint:
Histology, Limb Volume, Symptom Measures

Efficacy Evaluation

Ubenimex 150 mg TID

Placebo

N=20

N=20

Months 1-6

Enrolling
**Ubenimex in Lymphedema Timeline**

**Phase 2 Plan**

- **2016**
  - IND Approved
  - Science Translational Medicine Published May 2017

- **2017**
  - Enroll
  - Dose
  - Topline Data

- **2018**
  - If Positive Results
  - Agency Meeting

**Phase 2 Study**

- N=40 (planned)
Clinical News Flow Plan

**HDV Lonafarnib**
- LOWR HDV Program Data EASL 2017
- Agency Meeting

**HDV PEG IFN Lambda**
- US IND Filed
- LIMT Study Interim Data AASLD 2017

**PBH Exendin 9-39**
- MAD Study Complete ADA 2017
- PK Study Liquid Formulation Complete
- Phase 2 28-Day Study Initiation
- 28-Day Study Data

**PAH Ubenimex**
- LIBERTY Enrollment Complete KOL Analyst Event
- LIBERTY Data JPM 2018

**Lymphedema Ubenimex**
- ULTRA Enrollment Complete
- ULTRA Data
Experienced Management

David Cory, RPh, MBA
President and CEO

Jim Welch, MBA
Chief Financial Officer

Joanne Quan, MD
Chief Medical Officer

Eduardo Martins, MD, PhD
Senior Vice President, Liver & Infectious Diseases

Lisa Porter, MD
Senior Vice President, Metabolic Diseases

Jim Shaffer, MBA
Chief Business Officer

Shelly Xiong, PhD, RAC
Senior Vice President, Regulatory Affairs
<table>
<thead>
<tr>
<th>Indication</th>
<th>Faculty / Inventors / Advisors</th>
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<tbody>
<tr>
<td>Hepatitis Delta</td>
<td>Jeffrey Glenn, MD, PhD</td>
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<td>Post-Bariatric Hypoglycemia</td>
<td>Tracey McLaughlin, MD, MPH</td>
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<td>Pulmonary Arterial Hypertension</td>
<td>Mark Nicolls, MD</td>
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<tr>
<td>Lymphedema</td>
<td>Stanley Rockson, MD</td>
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An Orphan Disease Company by Design