Leader in HDV
Forward Looking Statement

This presentation and the oral commentary may contain forward-looking statements that involve future events. 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. All statements other than statements of historical facts contained in this presentation, including statements regarding our future financial condition, timing for and outcomes of clinical results, prospective products, preclinical and clinical pipelines, regulatory objectives, business strategy and plans and objectives for future operations, are forward looking statements. Such statements are predictions only and involve known and unknown risks, uncertainties and other important 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. These risks and uncertainties include, among others, the costs, timing and results of preclinical studies and clinical trials and other development activities for lonafarnib, interferon lambda, and avexitide, and any of our future product candidates; our ability to achieve timelines and obtain approval without the need to conduct large Phase 3 clinical trials for our product candidates or additional exploratory or pivotal trials beyond what we anticipate; our ability to obtain funding for our operations, including funding necessary to complete all clinical trials that may potentially be required to file any NDA or MAA for our product candidates, and complete all clinical trials that may potentially be required to file for regulatory approval, for any of our product candidates; the uncertainties inherent in the initiation and enrollment of clinical trials; expectations of expanding on-going clinical trials; availability and timing of data from clinical trials; the unpredictability of the duration and results of regulatory review; the commercialization of our product candidates, if approved, including whether commercializing lonafarnib for use in the progeria and progeroid laminopathies indications would result in receipt of a priority review voucher or otherwise be cash flow positive as a program for us; our plans to research, develop and commercialize our product candidates; our ability to attract collaborators with development, regulatory and commercialization expertise; the size and growth potential of the markets for our product candidates, and our ability to serve those markets; our ability to obtain favorable reimbursement and pricing and the rate and degree of market acceptance of our product candidates; regulatory developments in the United States and foreign countries; the performance of our third-party suppliers and manufacturers; market acceptance for approved products and innovative therapeutic treatments; competition; the accuracy of our estimates regarding expenses, future revenues, capital requirements and needs for additional financing; our expectations regarding the period during which we qualify as an emerging growth company under the Jumpstart Our Business Startups Act of 2012, or the JOBS Act; the possible impairment of, inability to obtain and costs of obtaining intellectual property rights; impacts of COVID-19 pandemic on our operations; and possible safety or efficacy concerns, general business, financial and accounting risks and litigation. Because forward-looking statements are inherently subject to risks and uncertainties, some of which cannot be predicted or quantified and some of which are beyond our control, you should not rely on these forward-looking statements as predictions of future events. More information concerning us and such risks and uncertainties is available on our website and in our press releases and in our public filings with the U.S. Securities and Exchange Commission. We are providing this information as of its date and do not undertake any obligation to update or revise it, whether as a result of new information, future events or circumstances or otherwise. Additional information may be available in press releases or other public announcements and public filings made after the date of this presentation.

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# Highlights

| DIVERSE PIPELINE | • Focus on rare and ultra-rare diseases with no approved therapies  
• Late stage assets with paths to commercialization  
• 4 Breakthrough Therapy Designations |
| LEADER IN HDV | • Only oral therapy in development (Lonafarnib)  
• Two complementary therapeutic options (Lonafarnib and Peginterferon lambda)  
• $1B+ annual commercial opportunity in U.S. and E.U. |
| PROGERIA NDA & MAA FILED | • Lonafarnib approval expected in 2020  
• Priority Review Voucher upon approval  
• Preparing for commercial launch |
| AVEXITIDE PROGRAM | • Phase 3 ready pipeline asset for Post-Bariatric Hypoglycemia |
Eiger HDV Franchise

Lonafarnib + Ritonavir

1st in class small molecule, oral prenylation inhibitor

Phase 3

Peginterferon Lambda

1st in class type III interferon

Phase 3 Ready
HDV Requires HBsAg to Complete Virus Assembly

**HDV** consists of a single stranded, circular RNA virus, with an envelope made up of HBsAg.

HBsAg Acquired Through PROTEIN PRENYLATION
This is mechanism targeted by lonafarnib.
HDV: Most Severe Form of Viral Hepatitis

Always a Co-infection with HBV

Rapid Disease Progression to Cirrhosis

- 20% Within 5 years
  - HBV Mono-infection

- 70% Within 5-10 years
  - HDV/HBV Co-infection

HDV: Most Severe Form of Viral Hepatitis

4-6% HDV

20% HBV Mono-infection

70% HDV/HBV Co-infection

Within 5 years

Within 5-10 years
15-20M HDV Patients Worldwide

~4-6% OF HBV-INFECTED POPULATION

Migration Contributing to Globalization of Disease
HDV: High Unmet Need and Disease Burden

LOW SURVIVAL RATE

~60% Mortality\(^1\)
Within 10 Years

Similar to some cancers

HIGH COST TRANSPLANTS

~$575K Cost\(^2\)
>14,000 person Waiting List

25% of people on waiting list die each year before receiving a liver transplant\(^1\)

No approved treatment

\(^1\) Serrano et al, EASL 2011   \(^2\) UPMC Health Beat, 2018, US liver transplant cost
>$1B HDV Market Opportunity

**CONSERVATIVE MARKET PENETRATION, ORPHAN PRICING**

<table>
<thead>
<tr>
<th></th>
<th>ADDRESSABLE MARKET</th>
<th>CONSERVATIVE PENETRATION</th>
<th>ORPHAN PRICING</th>
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<tbody>
<tr>
<td>~300,000 Patients⁴</td>
<td>~100K in US</td>
<td>~3% of Patients²</td>
<td>~150,000</td>
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<tr>
<td>~300,000 Patients⁴</td>
<td>~200K in EU</td>
<td>~3% of Patients²</td>
<td>~98,000</td>
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<tr>
<td>~100K in US</td>
<td>~200K in EU</td>
<td>~3K in US</td>
<td>~6K in US</td>
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</tbody>
</table>

>$1B Potential Peak Year Market Opportunity⁴,⁵

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⁴ Triangle Insights 2016  
⁵ Conservative penetration for illustrative purposes  
⁶ Pricing for illustrative purposes
Complementary Treatments for HDV

Lonafarnib + Ritonavir
1st in class small molecule, oral prenylation inhibitor
Phase 3

Peginterferon Lambda
1st in class type III interferon
Phase 3 Ready
Lonafarnib for HDV

**FIRST AND ONLY ORAL AGENT IN DEVELOPMENT FOR HDV**

- Well-characterized in patients
  - > 2,000 patients dosed in oncology program by Merck (Schering)
  - > 90 children dosed in Progeria program by Boston Children’s Hospital
  - > 170 patients dosed in HDV program
  - Longest duration of dosing > 10 years

- Most common experienced AEs are GI related (class effect)

- Patent estate covers broad range of lonafarnib + ritonavir doses and durations
  - US, Europe, Japan, China and South Korea
Reducing HDV-RNA Improves Survival

**HDV-RNA REDUCTION IMPROVES CLINICAL OUTCOMES**

Interferon-α for 48 Weeks with 15 year Follow Up

![Change in HDV-RNA](image)

- Log Change in Serum HDV-RNA
- n=36
- P = 0.009

![Survival](image)

- Proportion of Patients Surviving
- Years after Termination of Therapy

Farci et al, Gastroenterology 2004: Long-Term Benefit of Interferon-α Therapy of Chronic HDV: Regression of Advanced Hepatic Fibrosis
Lonafarnib Phase 2 Data

TWO LONAFARNIB-BASED REGIMENS IDENTIFIED FOR REGISTRATION

Change in Log HDV-RNA

Week

LNF 50 mg BID + RTV (N=12)
LNF 50 mg BID + RTV + PEG IFN-alfa-2a (N=4)

100-fold INCREASE IN ACTIVITY

≥ 2 log Decline HDV-RNA + ALT Normalization

COMPOSITE ENDPOINT

29%

63%

Yurdaydin et al., J Hepatology 2018, Phase 2 LOWR 2 Study, Abstract #PS-161
All patients will be maintained on background HBV nucleoside therapy. Superiority over PEG IFN-alfa-2a not required.

N=400 allows for single pivotal study for registration.
Phase 3 Global Study

Stratification by baseline viral load

High baseline viral load
>4 log HDV RNA

Low baseline viral load
<4 log HDV RNA

All patients will be maintained on background HBV nucleoside therapy. Superiority over PEG IFN-alfa-2a not required.
Phase 3 Global Study

**ORAL PATHWAYS TO APPROVAL**

**Stratification by baseline viral load**

- **High baseline viral load**
  - >4 log HDV RNA

- **Low baseline viral load**
  - <4 log HDV RNA

---

**Phase 3 Global Study**

**Primary Endpoint at Week 48**

≥ 2 log decline in HDV RNA + Normalization of ALT

**Secondary Endpoint at Week 48**

Histologic improvement
Improvement of fibrosis

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**Contribution of effect only**

**ORAL**

- N = 175
- Lonafarnib 50 mg BID
- Ritonavir 100 mg BID
- Follow Up

**COMBO**

- N = 125
- Lonafarnib 50 mg BID
- Ritonavir 100 mg BID
- PEG IFN-alfa-2a
- Follow Up

**MONO**

- N = 50
- PEG IFN-alfa-2a
- Follow Up

**Placebo**

- N = 50
- Follow Up

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* biopsy
All patients will be maintained on background HBV nucleoside therapy. Superiority over PEG IFN-alfa-2a not required.
Phase 3 Global Study

**COMBO PATHWAYS TO APPROVAL**

**Stratification by baseline viral load**
- High baseline viral load >4 log HDV RNA
- Low baseline viral load <4 log HDV RNA

**On-treatment 48 weeks**
- **ORAL**
  - N = 175
  - Lonafarnib 50 mg BID
  - Ritonavir 100 mg BID
- **COMBO**
  - N = 125
  - Lonafarnib 50 mg BID
  - Ritonavir 100 mg BID
  - PEG IFN-alfa-2a
- **MONO**
  - N = 50
  - PEG IFN-alfa-2a
- **Placebo**
  - N = 50

**Post-treatment 24 weeks**
- Follow Up

**Primary Endpoint at Week 48**
≥ 2 log decline in HDV RNA + Normalization of ALT

**Secondary Endpoint at Week 48**
- Histologic improvement
- Improvement of fibrosis

- Contribution of effect only

---

* biopsy

All patients will be maintained on background HBV nucleoside therapy. Superiority over PEG IFN-alfa-2a not required.
Study Mirrors HDV Global Footprint

COMPLETION OF ENROLLMENT EXPECTED IN 2021

HDV Phase 3 Sites

20 COUNTRIES

90 ACTIVE SITES

Key sites to be activated: NIH, Mongolia, Germany
Complementary Treatments for HDV

Lonafarnib + Ritonavir
1st in class small molecule, oral prenylation inhibitor
Phase 3

Peginterferon Lambda
1st in class type III interferon
Phase 3 Ready
Peginterferon Lambda (Lambda)

A WELL TOLERATED TYPE III INTERFERON

- Binds to a unique receptor vs type I IFN-α
  - Highly expressed on hepatocytes
  - Limited expression on hematopoietic and CNS cells
- Uses similar downstream signaling pathway to IFN-α
- 3,000+ patients in 19 clinical trials (HCV / HBV / HDV)

Lambda Receptors
Highly Expressed in the Liver

Lambda Receptors
Not Widely Distributed Throughout Body

IFN-α Receptors
Widely Distributed Throughout Body
LIMT: Phase 2 Lambda Monotherapy Study

36% DURABLE VIROLOGIC RESPONSE (DVR) WITH LAMBDA

DVR = below the limit of quantification (BLQ) at 24 weeks post-treatment
Robogene® 2.0 HDV RNA PCR assay, LOQ = 14 IU/mL; LOD = 6 IU/mL
Etzion et al, EASL 2019; dose reductions allowed
Lambda is Phase 3 Ready for HDV

CONCURRENCE WITH FDA & EMA ON SINGLE PIVOTAL STUDY

- **Arm 1**
  - N=100
  - Nuc Run-in
  - Randomization
  - 12 weeks
  - 48 weeks
  - Lambda 180 mcg QW
  - Follow-up

- **Arm 2**
  - N=50
  - No TRx
  - 48 wk
  - Lambda 180 mcg QW
  - Follow-up

- Run-in
  - 12 weeks

- HBV Nuc
Lambda is Phase 3 Ready for HDV

CONCURRENCE WITH FDA & EMA ON SINGLE PIVOTAL STUDY

Primary Endpoint*
DVR at 24 Weeks Post-TRx versus Placebo at 12 Weeks Post-No TRx

DVR = below the limit of quantification (BLQ) at 24 weeks post-treatment
LIFT: Phase 2 Lambda – Lonafarnib Combo Study

A WELL TOLERATED INTERFERON FOR COMBINATION

**Primary Endpoint:**
> 2 Log HDV RNA reduction at EOT

**Secondary Endpoint:**
Histological improvement (biopsy confirmed)

N=26

**On-treatment**
- Lambda 180 mcg QW
- Lonafarnib 50 mg BID
- Ritonavir 100 mg BID

24 Weeks

**Post-treatment**
Follow Up

24 Weeks

- Interim End of Treatment Data AASLD 2019
- End of Study Data Planned at AASLD 2020
LIFT Study: >50% HDV RNA BLQ at Week 24

INTERIM END OF TREATMENT DATA (N=19/26)

<table>
<thead>
<tr>
<th>Week 24 HDV RNA</th>
<th>% of Patients</th>
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<tbody>
<tr>
<td>&gt; 2 Log Decline</td>
<td>95%</td>
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<tr>
<td>BLQ</td>
<td>53%</td>
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<tr>
<td>Undetectable</td>
<td>37%</td>
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Median Change in HDV RNA (Log)

Weeks

BLQ = below the limit of quantitation
Convenience and Optionality for HDV Patients

- Potential HDV cure and maintenance therapies
- Foundational therapies for future combinations
Lambda for COVID-19

Peginterferon Lambda

1st in class
type III interferon
Lambda for Mild COVID-19

THERAPEUTIC WINDOWS OF INTERVENTION

Severity

Disease Course (Time)

Early Infection

Treatment of Mild Illness

IFNλ

SARS-CoV-2

IFNαβ (low?)

IFNλ (low?)

ANTIVIRAL RESPONSE

Progressive Infection and Hyperinflammation

Treatment of Severe Disease

IFNλ

SARS-CoV-2

IFNλ

Cytokine Storm

TNF | IL-1 | IL-6 |

MCP-1

INFLAMMATORY RESPONSE

Respiratory Failure

Mild Symptoms

Fever (≤ 38.5 °C)
Cough, Running Nose, Headache

Moderate to Severe Symptoms

Pneumonia
Shortness of Breath
Oxygen Saturation Drop
Abnormal Chest Imaging

Severe Symptoms

ARDS
Shock
Cardiac Dysfunction

Based on Andreakos et al 2020, EMBO Molecular Medicine
Lambda for COVID-19: Investigator Sponsored Studies

MULTIPLE OPPORTUNITIES TO PROVE CONCEPT

Eiger Involved in Protocol Development, Regulatory Interaction, and Lambda Supply

Multiple Institutions / Protocols Processing in Parallel:

• Stanford University (Upinder Singh, MD – Palo Alto)
• Toronto General Hospital (Jordan Feld, MD – Toronto)
• Mass General Hospital (Raymond Chung, MD – Boston)
• Mount Sinai (Scott Friedman, MD – NYC)
• Soroka University (Ohad Etzion, MD – Israel)
• Johns Hopkins University (Mark Sulkowski, MD – Baltimore)
for Progeria and Progeroid Laminopathies

NDA and MAA Filed
FDA Approval Expected in 2020
Progeria: Ultra-Rare, Fatal, Premature Aging Pediatric Disease

HUTCHINSON-GILFORD PROGERIA SYNDROME

• Point mutation in the Lamin A gene
  - Results in a farnesylated aberrant protein, Progerin
  - Disruption of scaffold structure of the nuclear membrane
• Accelerated atherosclerosis with cardiovascular decline
• Average lifespan = 14.5 years
• Prevalence of 1 in 20 million (~400 worldwide)
• No FDA approved Rx
• 90+ children and young adults treated with Zokinvy
Zokinvy Improved Survival in Progeria

77% REDUCTION IN RISK OF MORTALITY COMPARED TO NO TREATMENT

Hazard Ratio = 0.23, p = 0.04

Gordon, L et al, JAMA, 2018, 319(16): 1687
Average follow-up period of 2.2 years
Managed Access Program (MAP)

ENSURING ACCESS TO ZOKINVY

MAP spans > 40 countries

Preparing For Commercial Launch

Working with the Progeria Community
Lonafarnib for Progeria and HDV

DISTINCT DISEASES, DISTINCT TREATMENT REGIMENS, DISTINCT COMMERCIAL STRATEGIES

PROGERIA

Lonafarnib (Weight-based) Monotherapy

HDV

Lonafarnib / Ritonavir (Combo Dose Pak) ± Peginterferon
Avexitide for PBH and CHI

**POST-BARIATRIC HYPOGLYCEMIA (PBH)**

- Complication of bariatric surgery
- Dangerously low blood sugar after meals
- ~10% of Roux-en-Y Gastric Bypass (RYGB)
- ~2.5% of Vertical Sleeve Gastrectomy (VSG)
- Prevalence ~120K in US / ~30K in EU
- No approved therapy

Results in **SEVERE HYPOGLYCEMIA:** altered mental status, loss of consciousness, seizures, coma

**CONGENITAL HYPERINSULINISM (CHI)**

- Ultra-rare pediatric metabolic disorder
- Most frequent cause of persistent hypoglycemia in neonates and children
- Occurs in 1:25,000 to 1:50,000 live births
- Near-total pancreatectomy is indicated
- Better therapies needed

Results in **PERMANENT BRAIN DAMAGE** with neurodevelopmental deficits in up to 50% of patients

* American Society for Metabolic and Bariatric Surgery 2015
<table>
<thead>
<tr>
<th>TARGETED INDICATION</th>
<th>DRUG</th>
<th>ORPHAN US / EU</th>
<th>BREAKTHROUGH THERAPY</th>
<th>RARE PEDIATRIC DISEASE*</th>
<th>STATUS</th>
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<tr>
<td>Hepatitis Delta Virus</td>
<td>Lonafarnib + Ritonavir</td>
<td>✔️</td>
<td>✔️</td>
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<td>Peginterferon Lambda</td>
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<td>✔️</td>
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<td>Phase 3 Ready</td>
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<tr>
<td>Progeria and Progeroid Laminopathies</td>
<td>Zokinvy™ (Lonafarnib)</td>
<td>✔️</td>
<td>✔️</td>
<td>✔️</td>
<td>NDA / MAA Filed; FDA Approval expected in 2020</td>
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<td>Post-Bariatric Hypoglycemia</td>
<td>Avexitide</td>
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<td>✔️</td>
<td>N/A</td>
<td>Phase 3 Ready</td>
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<tr>
<td>Congenital Hyperinsulinism</td>
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<td></td>
<td>✔️</td>
<td>Phase 2</td>
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# Experienced Management Team

<table>
<thead>
<tr>
<th>Name</th>
<th>Title</th>
<th>Companies</th>
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<tbody>
<tr>
<td><strong>DAVID CORY, RPH, MBA</strong></td>
<td>Business Founder&lt;br&gt;President&lt;br&gt;Chief Executive Officer</td>
<td><img src="#" alt="gsk" /> <img src="#" alt="InterMune" /> <img src="#" alt="Prestwick Pharmaceuticals" /> <img src="#" alt="Cotherix" /></td>
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<td><strong>SRI RYALI, MBA</strong></td>
<td>Chief Financial Officer</td>
<td><img src="#" alt="aimmune Therapeutics" /> <img src="#" alt="Jazz Pharmaceuticals" /> <img src="#" alt="Onyx Pharmaceuticals" /> <img src="#" alt="AMGEN" /></td>
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<td><strong>STEPHANA PATTON, PHD, JD</strong></td>
<td>General Counsel&lt;br&gt;Corporate Secretary&lt;br&gt;Chief Compliance Officer</td>
<td><img src="#" alt="Salix Pharmaceuticals" /> <img src="#" alt="Biodelivery Sciences" /> <img src="#" alt="BIO TIME" /></td>
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<td><strong>ELDON MAYER, MBA</strong></td>
<td>Executive Vice President&lt;br&gt;Chief Commercial Officer</td>
<td><img src="#" alt="Rigel Pharmaceuticals" /> <img src="#" alt="Questcor Pharmaceuticals, Inc." /> <img src="#" alt="Schering-Plough" /></td>
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<td><strong>JIM SHAFFER, MBA</strong></td>
<td>Chief Business Officer</td>
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<td><strong>INGRID CHOONG, PHD</strong></td>
<td>Senior Vice President&lt;br&gt;Clinical Development</td>
<td><img src="#" alt="sunesis" /> <img src="#" alt="Berkeley University" /> <img src="#" alt="Stanford Medicine" /></td>
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Leader in HDV
Late stage pipeline with 1st in class therapies
Strong clinical data
Large commercial market (HDV)
Progeria approval expected with PRV
$78M cash & investments as of 3/31/20