A RARE DISEASE COMPANY
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, whether the FDA minutes confirm the understanding that existing data will support an NDA filing for lonafarnib in Progeria; our ability to meet the quality and documentation requirements for potential approval of an NDA; and the timing for filing of an NDA; our ongoing and planned clinical development, including whether the D-LIVR study will be supported by the FDA as a single, pivotal study to support registration; the timing of and our ability to initiate or enroll clinical trials, including whether our D-LIVR study can be advanced by the end of this year; whether PREVENT Phase 2 study results will support further development of avexitide; our ability to make timely regulatory filings and obtain and maintain regulatory approvals for lonafarnib as a single agent or in combination, PEG IFN lambda, avexitide and our other product candidates; our intellectual property position; and the potential safety, efficacy, reimbursement, convenience clinical and pharmaco-economic benefits of our product candidates as well as the commercial opportunities, including potential market sizes and segments; our ability to finance the continued advancement of our development pipeline products, including our results of operations, cash available, financial condition, liquidity, prospects, growth and strategies; and the potential for success of any of our product candidates.

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DE-RISKED
FIRST-IN-CLASS
PIPELINE
TARGETING
RARE
AND
ULTRA-RARE
DISEASES

EIGER is a late stage biopharmaceutical company focused on the development and commercialization of well-characterized drugs for life-threatening, rare and ultra-rare diseases with high unmet medical needs and no approved therapies.

EIGER has reported positive proof-of concept clinical results in all programs, all with first-in-class drugs, now advancing toward NDA or Phase 3 clinical development.
Diversified Pipeline

Focused on Serious Rare and Ultra-Rare Diseases

Accelerating Development of Treatments

HEPATITIS DELTA VIRUS

HUTCHINSON-GILFORD PROGERIA SYNDROME

POST-BARIATRIC HYPOGLYCEMIA
### FIRST-IN-CLASS THERAPIES IN DEVELOPMENT

Targeting Rare and Ultra-Rare Diseases with No Approved Treatments

<table>
<thead>
<tr>
<th>Indication</th>
<th>Drug</th>
<th>Regulatory Status</th>
<th>Clinical Status</th>
</tr>
</thead>
</table>
| Hepatitis Delta Virus | Lonafarnib + Ritonavir | • Orphan US & EU  
• Fast Track & Breakthrough  
• PRIME EMA | Phase 3 |
| Hepatitis Delta Virus | Peginterferon Lambda | • Orphan US  
• Fast Track | Phase 2 |
| Progeria* and Progeroid Laminopathies | Lonafarnib | • Orphan* US & EU  
• Breakthrough  
• Rare Disease Designation | NDA Prep |
| Post-Bariatric Hypoglycemia | Avexitide | • Orphan US & EU | Phase 2 |
# RARE AND ULTRA-RARE DISEASE TARGETS

## Advancing Toward NDA

<table>
<thead>
<tr>
<th>Disease</th>
<th>Q1 2019</th>
<th>Q2 2019</th>
<th>Q3 2019</th>
<th>Q4 2019</th>
</tr>
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<tbody>
<tr>
<td>HDV</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Lonafarnib + Ritonavir</td>
<td></td>
<td></td>
<td></td>
<td>D-LIVR Phase 3 Enrolling</td>
</tr>
<tr>
<td>HDV</td>
<td></td>
<td></td>
<td>LIFT Study Phase 2 Combo Dosing</td>
<td></td>
</tr>
<tr>
<td>Peginterferon Lambda</td>
<td></td>
<td></td>
<td></td>
<td>LIFT Study EOT</td>
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<tr>
<td>Progeria &amp; PL Lonafarnib</td>
<td></td>
<td>NDA Preparation Ongoing</td>
<td></td>
<td>Expanded Access Program</td>
</tr>
<tr>
<td>PBH</td>
<td></td>
<td>Regulatory Guidance</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Avexitide</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
OVERVIEW

- HDV is the most severe form of human viral hepatitis
- HDV is always a co-infection with HBV
  - HBsAg in HDV viral envelope
  - HBsAg acquired through protein prenylation
- 4-6% of HBV infected patients co-infected with HDV
- HDV causes more rapid disease progression
  - Compared to HBV mono-infection
- No FDA approved Rx
- 15-20 M HDV infected patients worldwide
  - > 100K patients in US; > 200K patients in EU
HDV: MOST RAPID PROGRESSION OF VIRAL HEPATITIS

50% of HDV-Infected Patients are Cirrhotic at Diagnosis

### Progression to Cirrhosis

<table>
<thead>
<tr>
<th>Virus</th>
<th>Timeframe</th>
</tr>
</thead>
<tbody>
<tr>
<td>HCV</td>
<td>10-20% within 20 Years</td>
</tr>
<tr>
<td>HBV</td>
<td>20% within 5 Years</td>
</tr>
<tr>
<td>HDV</td>
<td>70% within 5-10 Years</td>
</tr>
</tbody>
</table>

Westbrook et al, *J Hepatology* 2014  
Fattovich et al, *Seminars in Liver Diseases* 2003  
**HBV INVESTIGATIONAL THERAPIES DO NOT ERADICATE HDV**

Targeting Functional Cure vs Sterilizing Cure

- Approved HBV nucleos(t)ide treatments only suppress HBV DNA
  - Do not affect HBsAg and have no impact on HDV

- Investigational HBV treatments target **functional cure**
  - Not expected to eliminate extra-hepatic reservoirs of HBsAg

**HBV Functional Cure (If Achieved) Will Not Eradicate HDV**
HDV WORLDWIDE PREVALENCE: 15-20 MILLION

6% of HBV Population Co-Infected with HDV
**PEG IFN-ALFA REDUCED HDV RNA IN PATIENTS**

Not Approved for HDV

**HIDIT - 2 Study**

HIDIT - 2: PEG IFNα 180 mcg QW + tenofovir (N=91)

Mean Change in Log HDV-RNA

Wobse et al, *Hepatology* 2014
Wedemeyer et al, *Hepatology* 2014
REDUCING HDV-RNA WITH IFN\textsubscript{\alpha} IMPROVES SURVIVAL

Improved Clinical Benefit without Clearance of HDV-RNA

Interferon-α for 48 weeks with 15 year Follow Up

Log Change in Serum HDV-RNA

Proportion of Patients Surviving

Farci et al, Gastroenterology 2004: Long-Term Benefit of Interferon-α Therapy of Chronic HDV: Regression of Advanced Hepatic Fibrosis
LONAFARNIB FOR HDV

Well-characterized Clinical Stage Lead Compound

• Small molecule, first-in-class, 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

• Orphan in US & EU, Fast Track & Breakthrough in US, PRIME in EU

• Patent issued allowing broad range of lonafarnib + ritonavir doses and durations
ALL-ORAL REGIMEN: INTERFERON-FREE OPTION

Lonafarnib 50 mg BID + Ritonavir 100 mg BID

Yurdaydin et al, J Hepatology 2018, Abstract #PS-161
ALL-ORAL REGIMEN: INTERFERON-FREE OPTION

Comparable Antiviral Activity to PEG IFN-alfa-2a

Yurdaydin et al, J Hepatology 2018, Abstract #PS-161

LOWR 2 and HIDIT-1 enrolled comparable HDV patient populations: GT 1, well-compensated cirrhotics and non-cirrhotics, chronic HDV
**COMBO REGIMEN:** GREATEST OBSERVED DECLINE IN HDV RNA

Lonafarnib 50 mg BID + Ritonavir 100 mg BID + PEG IFN-alfa-2a 180 mg QW

<table>
<thead>
<tr>
<th>Week</th>
<th>LOWR-2 STUDY</th>
<th>HIDIT-1 STUDY</th>
</tr>
</thead>
<tbody>
<tr>
<td>0</td>
<td>-4</td>
<td>-4</td>
</tr>
<tr>
<td>4</td>
<td>-3.5</td>
<td>-3.5</td>
</tr>
<tr>
<td>8</td>
<td>-3</td>
<td>-3</td>
</tr>
<tr>
<td>12</td>
<td>-2.5</td>
<td>-2.5</td>
</tr>
<tr>
<td>16</td>
<td>-2</td>
<td>-2</td>
</tr>
<tr>
<td>20</td>
<td>-1.5</td>
<td>-1.5</td>
</tr>
<tr>
<td>24</td>
<td>-1</td>
<td>-1</td>
</tr>
</tbody>
</table>

100-fold increase in activity

Yurdaydin et al, *J Hepatology* 2018, Abstract #PS-161
**LONAFARNIB PHASE 2 HDV PROGRAM**

Dose, Combinations and Endpoints Defined

- **All-oral:** Lonafarnib boosted with Ritonavir
  - 33% (6 of 18) patients ≥ 2 log decline or BLQ at Week 24
  - 47% (7 of 15) patients normalized ALT at Week 24
  - **Composite endpoint: 29% (4 of 14)**

- **Combination:** Lonafarnib boosted with Ritonavir + PEG IFN-alfa-2a
  - 78% (7 of 9) patients ≥ 2 log decline or BLQ at Week 24
  - 88% (7 of 8) patients normalized ALT at Week 24
  - **Composite endpoint: 63% (5 of 8)**

- Predominant AEs were GI-related (mild / moderate)

---

Yurdaydin et al, *J Hepatology* 2018, Abstract #PS-161

Most common reported AEs: nausea, diarrhea, fatigue, weight loss, anorexia, vomiting

Presented EASL 2018
# HIGH RESPONSE RATES IN LOW BASELINE VIRAL LOAD

All-Oral: Lonafarnib 50 mg BID + Ritonavir 100 mg BID

<table>
<thead>
<tr>
<th>Regimen</th>
<th># Patients Dosed 24 Weeks</th>
<th>BL VL ≤ 4 log (%):</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td>&lt; LLOQ (%)</td>
</tr>
<tr>
<td>LNF 50 mg BID + RTV 100 mg BID + PEG IFN-α</td>
<td>4</td>
<td>0 / 0 (0%)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>2 / 4 (50%)</td>
</tr>
<tr>
<td>LNF 25 mg BID + RTV 100 mg BID + PEG IFN-α</td>
<td>5</td>
<td>1 / 1 (100%)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>2 / 4 (50%)</td>
</tr>
<tr>
<td>LNF 50 mg BID + RTV 100 mg BID</td>
<td>12</td>
<td>5 / 5 (100%)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>0 / 7 (0%)</td>
</tr>
<tr>
<td>LNF 25 mg BID + RTV 100 mg BID</td>
<td>6</td>
<td>0 / 3 (0%)</td>
</tr>
<tr>
<td></td>
<td></td>
<td>0 / 3 (0%)</td>
</tr>
</tbody>
</table>

Per protocol analysis

Yurdaydin et al, *J Hepatology* 2018, Abstract #PS-161
# Response Rates in High Baseline Viral Load HDV

Combination: Lonafarnib 50 mg BID + Ritonavir 100 mg BID + PEG IFN-alfa-2a

<table>
<thead>
<tr>
<th>Regimen</th>
<th># Patients Dosed 24 Weeks</th>
<th># of Patients</th>
<th>BL VL ≤ 4 log (%)</th>
<th>BL VL &gt; 4 log (%)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td></td>
<td></td>
<td>&lt; LLOQ (%)</td>
<td>≥ 2 log decline (%)</td>
</tr>
<tr>
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<td>0 / 0 (0%)</td>
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<tr>
<td>LNF 50 mg BID + RTV 100 mg BID</td>
<td>12</td>
<td>5 / 5 (100%)</td>
<td>5 / 5 (100%)</td>
<td>0 / 7 (0%)</td>
</tr>
<tr>
<td>LNF 25 mg BID + RTV 100 mg BID</td>
<td>6</td>
<td>0 / 3 (0%)</td>
<td>0 / 3 (0%)</td>
<td>0 / 3 (0%)</td>
</tr>
</tbody>
</table>

Per protocol analysis

Yurdaydin et al, *J Hepatology* 2018, Abstract #PS-161
LONAFARNIB TREATMENT OPTIONS

Guided by Baseline HDV Viral Loads

High Baseline Viral Load

65% of HDV population

Lonafarnib / Ritonavir + PEG IFN-alfa-2a

Low Baseline Viral Load

35% of HDV population

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

**Secondary Endpoint at Week 48**
- Histologic improvement
  - > 2 point improvement in HAI inflammatory score
  - No progression in fibrosis
- Improvement of fibrosis

* biopsy

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All patients will be run-in and maintained on background HBV nucleoside therapy. Superiority over PEG IFN-alfa-2a not required.
First site initiated
University of Miami
December 10
WORKING TO CHANGE THE FACE OF HEPATITIS DELTA VIRUS (HDV)

- US Investigator Meeting, November 29
- First Site Initiated, December 10
- EU Investigator Meeting, January 16
  - First patient dosed, Q1 2019 (expected)
**PEGINTERFERON LAMBDA**

A Better Tolerated Interferon

- A novel first-in-class Type III interferon
- Binds to a unique receptor versus Type I interferons
  - Highly expressed on hepatocytes
  - Limited expression on hematopoietic cells and CNS cells
- Uses similar downstream signaling pathway as Type I interferons
- Greater than 3,000 patients in 17 clinical trials (HCV / HBV)
- Comparable antiviral activity with less of the typical IFN alfa related side effects*

*Chan, HLY et al, J Hepatology 2016*
**LIMT: PHASE 2 LAMBDA MONOTHERAPY STUDY**

**Lambda Interferon MonoTherapy Study**

<table>
<thead>
<tr>
<th>Arm 1</th>
<th>N = 19</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mono</td>
<td>Lambda 120 mcg QW</td>
</tr>
</tbody>
</table>

<table>
<thead>
<tr>
<th>Arm 2</th>
<th>N = 14</th>
</tr>
</thead>
<tbody>
<tr>
<td>Mono</td>
<td>Lambda 180 mcg QW</td>
</tr>
</tbody>
</table>

**Goals:**
- Demonstrate comparable activity to historical PEG IFN-alfa-2a
- Demonstrate better tolerability to historical PEG IFN-alfa-2a

**Dosing Complete**
**HDV-RNA REDUCTION WITH LAMBDA THRU WEEK 48**

Dose Response Demonstrated

<table>
<thead>
<tr>
<th>Week 48</th>
<th>N</th>
<th>Mean VL Decline</th>
<th>≥ 2 Log Decline or BLQ</th>
</tr>
</thead>
<tbody>
<tr>
<td>120 mcg*</td>
<td>13/19</td>
<td>-1.1 log</td>
<td>7 of 13 (53.8%)</td>
</tr>
<tr>
<td>180 mcg*</td>
<td>11/14</td>
<td>-2.3 log</td>
<td>10 of 11 (90.1%)</td>
</tr>
</tbody>
</table>

**Etzion O, Hamid S et al, Hepatology 2017**

Limit of quantification = 14 IU/mL  
* Randomization Dose
**LIMT HDV “MONO”: PHASE 2 STUDY**

48 Week End of Treatment Data

- Lambda demonstrated comparable anti-HDV activity to historical PEG IFN-alfa-2a

- Lambda was well tolerated in the majority of patients

- Lambda is a promising investigational agent, alone or in combination Rx in HDV

*Hamid S et al, *Hepatology* 2017*
**LIFT: PHASE 2 LAMBDA COMBO WITH LONAFARNIB STUDY**

**Lambda InterFeron combination Therapy**

- **N=26**
- **On-treatment**: 24 Weeks
- **Post-treatment**: 24 Weeks

**Combination**: Lambda 180 mcg QW, Lonafarnib 50 mg BID, Ritonavir 100 mg BID

**Follow Up**

**Primary Endpoint:**
- \( \geq 2 \) Log HDV RNA reduction at EOT

**Secondary Endpoint:**
- Histological Improvement (biopsy confirmed)
**LIFT: PHASE 2 LAMBDA COMBO WITH LONAFARNIB STUDY**

**Lambda InterFeron combination Therapy**

### Primary Endpoint:
- ≥ 2 Log HDV RNA reduction at EOT

### Secondary Endpoint:
- Histological Improvement (biopsy confirmed)

**Study Design**

- **On-treatment:**
  - 24 Weeks
  - Combo: Lambda 180 mcg QW, Lonafarnib 50 mg BID, Ritonavir 100 mg BID

- **Post-treatment:**
  - 24 Weeks
  - Follow Up

- **N=26**

- Enrolling
- End of Treatment Data Q4’19

* biopsy
COMPLEMENTARY DRUGS FOR HDV

Multiple Treatment Options

- Lonafarnib
- PEG IFN Lambda

Lonafarnib + Ritonavir
- *All Oral Rx*

Lonafarnib + Ritonavir + PEG IFN Lambda
- *Combination Rx*

PEG IFN Lambda
- *Sub Q Rx*
# HDV Prevalence in the US and Major Markets

## Significant Market Opportunity

<table>
<thead>
<tr>
<th>Country</th>
<th>HDV Prevalence</th>
<th>HDV Diagnosed</th>
</tr>
</thead>
<tbody>
<tr>
<td>US</td>
<td>140,000</td>
<td>43,000</td>
</tr>
<tr>
<td>Japan</td>
<td>111,000</td>
<td>27,000</td>
</tr>
<tr>
<td>Germany</td>
<td>54,000</td>
<td>16,000</td>
</tr>
<tr>
<td>Italy</td>
<td>37,000</td>
<td>11,000</td>
</tr>
<tr>
<td>UK</td>
<td>24,000</td>
<td>7,000</td>
</tr>
<tr>
<td>France</td>
<td>15,000</td>
<td>4,000</td>
</tr>
<tr>
<td>Spain</td>
<td>13,000</td>
<td>4,000</td>
</tr>
</tbody>
</table>

![Bar chart showing HDV prevalence and diagnosed cases](chart.png)
POTENTIAL HDV PATIENT TREATMENT UPTAKE IN THE US

Cumulative New HDV Patient Starts in the US

Cumulative New Patients

Patient Counts

0 5,000 10,000 15,000 20,000 25,000 30,000 35,000 40,000 45,000

2022 2023 2024 2025 2026 2027 2028 2029 2030 2031 2032 2033 2034 2035 2036 2037
>90% OF PEOPLE TESTED FOR HDV HAVE Rx INSURANCE
Adequate Reimbursement for HDV Treatment

- Commercial: 60%
- Managed Medicare: 20%
- Managed Medicaid: 7%
- Medicaid: 8%
- Self-pay: 1%
- Managed Medicaid: 4%
BUILDING THE HDV MARKET

• 600,000 Dx HBV patients provide readily identifiable HDV market
• Claims data produces a known geographic HDV patient footprint in major metros of the US
• Key partners facilitate education and identification of HDV patients

- Highly targeted patient and physician outreach
- HDV testing program for HBV+ patients
- HDV RNA quantification and HBV/HDV reflex test for commercial testing
- HDV Connect Program: Global outreach to 111 countries
- Strong presence among ex-US and foreign-born HBV patients
- Patient and physician outreach supporting Eiger HDV testing initiative
Top 10 U.S. Cities in 2016

1. Chicago, Illinois
2. Berwyn, Illinois
3. Brooklyn, New York
4. Corona, New York
5. Waukegan, Illinois
6. New York, New York
7. Bronx, New York
8. Jamaica, New York
9. Lombard, New York
10. Aurora, Illinois
**U.S. HDV PREVALENCE IN 2018 ~ 110,000**

Increased Screening Leads to Increased HBV and HDV Diagnosis

* 11.8% of HBV Patients Co-infected with HDV

**Newly Diagnosed HDV Patients in the U.S. Each Year***

<table>
<thead>
<tr>
<th>Year</th>
<th>Newly Diagnosed HDV Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>2008</td>
<td>4,946</td>
</tr>
<tr>
<td>2009</td>
<td>4,274</td>
</tr>
<tr>
<td>2010</td>
<td>4,841</td>
</tr>
<tr>
<td>2011</td>
<td>5,043</td>
</tr>
<tr>
<td>2012</td>
<td>5,259</td>
</tr>
<tr>
<td>2013</td>
<td>5,916</td>
</tr>
<tr>
<td>2014</td>
<td>6,386</td>
</tr>
<tr>
<td>2015</td>
<td>7,442</td>
</tr>
<tr>
<td>2016</td>
<td>9,079</td>
</tr>
</tbody>
</table>

**Martins et al, DDW 2017**
HDV DISEASE AWARENESS

Rare Disease Supplement September 2018

HDV

... the most severe form of human viral hepatitis

... over 15 million people are infected worldwide

... no approved treatment

COMMITTED TO RARE DISEASES

For more information visit www.eigerbio.com
HDV AND PROGERIA

Distinct Diseases, Distinct Treatment Regimens, Distinct Commercial Strategies

HDV

Lonafarnib Boosted with Ritonavir
± Pegylated Interferon-Alfa

PROGERIA

Lonafarnib Monotherapy
(weight-based)
HUTCHINSON-GILFORD PROGERIA SYNDROME (PROGERIA)

OVERVIEW

• Ultra-rare, fatal, premature aging pediatric disease
• 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)
  - 1 child born each year in the US
• No FDA approved Rx
• >80 Children treated with lonafarnib
Berns Family
Founders of the Progeria Research Foundation (PRF)
ACCUMULATION OF PROGERIN
Disrupts Cell Scaffold, Leads to Disfigurement of Nucleus

Normal Lamin A Generation

Progerin Generation
50 amino acids deleted

LONAFARNIB

Lonafarnib blocks production of Progerin
W/W PREVALENCE ~ 400 CHILDREN WITH PROGERIA

147 Identified Across 47 Countries Worldwide with Progeria and Progeroid Laminopathies

147 Patients Identified Worldwide
- Progeria* W/W = 114
- Progeroid Laminopathies** W/W = 33

43 Patients Identified in US/EU
- Progeria* US/EU = 32
- Progeroid Laminopathies** US/EU = 11

* Progeria (HGPS) patients have a progerin-producing mutation in the LMNA gene
** Progeroid Laminopathies have a mutation in the lamin pathway but do not produce progerin
SURVIVAL OF UNTREATED PROGERIA CHILDREN

Average Lifespan = 14.5 Years

LONAFARNIB IMPROVES SURVIVAL IN PROGERIA

77% Reduction in Risk of Mortality Compared to No Treatment

Survival Probability

Hazard Ratio = 0.23, $p = 0.04$

NEXT STEPS FOR PROGERIA

LATE 2018

Launch Expanded Access Program

2019

File NDA in the U.S. and MAA in Europe

EIGER’S COMMITMENT IS TO PROVIDE ACCESS TO LONAFARNIB TO EVERY CHILD IN THE WORLD WITH PROGERIA
PROGERIA AND HDV

Distinct Diseases, Distinct Treatment Regimens, Distinct Commercial Strategies

PROGERIA
Lonafarnib Monotherapy (weight-based)

HDV
Lonafarnib Boosted with Ritonavir ± Peginterferon-Alfa
EXPANDED ACCESS PROGRAM (EAP)

- EAP supplies unlicensed drugs to patients with life threatening diseases
- A non-negotiable, global reference price will be set
- Reimbursement status is determined country by country
- Reimbursement sought through a treating physician (investigator), paid by various payers
- **Clinigen** is a global leader in expanded access execution
EXPANDED ACCESS PROGRAM: OBJECTIVES

For Progeria and Progeroid Laminopathies (PL)

• Provides global access of lonafarnib to children with Progeria and PL in 37 countries
  - Both treatment naïve and patients ending study participation are eligible

• Seek reimbursement where available

• Prepare for commercialization
**EAP: COUNTRY REIMBURSEMENT BUCKETS**

Determined by Country Regulatory Guidelines & Availability of Reimbursement

<table>
<thead>
<tr>
<th>Bucket 1</th>
<th>Bucket 2</th>
<th>Bucket 3</th>
<th>Bucket 4</th>
</tr>
</thead>
<tbody>
<tr>
<td>United States: Will Not Seek Reimbursement; Filing NDA</td>
<td>EAP Reimbursement Does Not Require Prior Approval in US</td>
<td>EAP Reimbursement Post-US approval</td>
<td>No EAP</td>
</tr>
</tbody>
</table>

# Countries: 1  
# Children: 16  
25  
64  
11  
24  
9  
9
POST-BARIATRIC HYPOGLYCEMIA (PBH)

OVERVIEW

• Bariatric Surgery Increasing due to Morbid Obesity
  - ~200K US / ~100K EU in 2015*
  - Significant Impact: Weight Loss, Glycemic Control
  - Roux-en-Y Gastric Bypass ~35% of all procedures

• Postprandial Hypoglycemia: Serious Complication
  - Dangerously low blood sugar after meals
  - Impacts 5-10% of Roux-en-Y patients

• PBH estimated prevalence ~70K in US / EU

• No approved therapy

* American Society for Metabolic and Bariatric Surgery 2015
TARGETED BLOCKADE OF GLP-1
Normalizes Insulin Secretion

ALTERED NUTRIENT TRANSIT POST ROUX-EN-Y GASTRIC BYPASS → HYPER-SECRETION OF GLP-1 → INSULIN SECRETION → SYMPTOMATIC HYPOGLYCEMIA

Hyper-secretion of GLP-1

Craig et al. Diabetes, Obesity and Metabolism 2017

Autonomic
- Sweating
- Shaking
- Palpitations
- Hunger

Neuroglycopenic
- Blurred vision
- Confusion
- Drowsiness
- Odd behavior
- Speech difficulty
- Incoordination
- Dizziness
- Inability to concentrate
AVEXITIDE: A GLP-1 ANTAGONIST

31 Amino Acid Fragment of Byetta (exenatide), a GLP-1 Agonist

- Phase 2 Activity and Safety Demonstrated in 54 PBH Patients
  - Four clinical studies completed (POC, SAD, MAD, 28-day)

- Previous experience as investigational agent
  - >300 patients reported dosed worldwide*

- Proprietary Liquid Formulation Developed

- Orphan Designation Granted in US and EU

* www.clinicaltrials.gov
# AVEXITIDE: PHASE 2 PROOF OF CONCEPT DEMONSTRATED

54 Patients Dosed in 4 Completed Clinical Studies with Avexitide

<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>Published Diabetologia</td>
</tr>
<tr>
<td>Sub Q Injection, SAD</td>
<td>8</td>
<td>Single dose</td>
<td>Presented at 2016 ADA Published Diabetes, Obesity and Metabolism</td>
</tr>
<tr>
<td>Sub Q Injection, MAD</td>
<td>20</td>
<td>Up to 3 days BID dosing</td>
<td>Presented at 2017 ADA</td>
</tr>
<tr>
<td>Sub Q Injection; Durability of Effect</td>
<td>18</td>
<td>28 days QD / BID dosing</td>
<td>Eiger R&amp;D Day: December 11, 2018</td>
</tr>
</tbody>
</table>
**AVEXITIDE REDUCES PBH**

Single Ascending Dose Study Results

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

* P<0.05, ** P<0.01
Craig C et al, Diabetes, Obesity and Metabolism 2017.
28-DAY, PHASE 2 STUDY

Goal: Demonstrate Durability of Effect, Define Dose, Safety, Tolerability

- Placebo
- Avexitide 30 mg BID
- Avexitide 60 mg QD
- Avexitide 30 mg BID

Primary Endpoint: Magnitude of postprandial hypoglycemia defined as the plasma glucose nadir during MMTT provocation

Liquid formulation of Avexitide (subcutaneous injection)
IMPROVED POSTPRANDIAL GLUCOSE NADIR

Primary Endpoint Achieved

Placebo: 30 mg BID, 60 mg QD

Glucose (mg/dL)

- Placebo: 47.1
- 30 mg BID: 57.1
- 60 mg QD: 59.2

P=0.0011

P=0.0002

P=0.0011
REDUCED POSTPRANDIAL INSULIN PEAK

Secondary Endpoint Achieved

<table>
<thead>
<tr>
<th>Treatment</th>
<th>Insulin (µIU/mL)</th>
<th>P-value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Placebo</td>
<td>454.5</td>
<td></td>
</tr>
<tr>
<td>30 mg BID</td>
<td>349.5</td>
<td>0.0417</td>
</tr>
<tr>
<td>60 mg QD</td>
<td>357.2</td>
<td>0.0288</td>
</tr>
</tbody>
</table>
**METABOLIC AND CLINICAL IMPROVEMENTS**

Reduction in Rates\(^1\) of Hypoglycemia, Severe Hypoglycemia and Rescue by eDiary

<table>
<thead>
<tr>
<th></th>
<th>Number of Episodes in 14 Day Period</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Placebo</td>
</tr>
<tr>
<td>Rate of Hypoglycemia(^2)</td>
<td>4.03</td>
</tr>
<tr>
<td>Change from Placebo</td>
<td>NA</td>
</tr>
<tr>
<td>Rate of Severe Hypoglycemia(^3)</td>
<td>2.36</td>
</tr>
<tr>
<td>Change from Placebo</td>
<td>NA</td>
</tr>
<tr>
<td>Rate of Rescue</td>
<td>4.87</td>
</tr>
<tr>
<td>Change from Placebo</td>
<td>N/A</td>
</tr>
</tbody>
</table>

1 Rate is defined as number of episodes in a 14 day period
2 Hypoglycemia is defined as hypoglycemia symptoms confirmed by SBGM concentrations of <70 mg/dL
3 Severe hypoglycemia is defined as neuroglycopenic symptoms confirmed by SBGM concentrations <55 mg/dL
SAFETY AND TOLERABILITY

• Avexitide was well-tolerated

• No treatment-related SAEs and no participant withdrawals

• AEs were typically mild to moderate in severity and transient

• Most common AEs were injection site bruising, nausea, headache
  o All occurred with higher frequency during placebo than active treatment

• Low occurrence of development of anti-drug antibodies (ADA)
  o 1 of 18 participants showed low positive titers for ADA
  o No associated AEs and no apparent effect on efficacy
SUMMARY AND NEXT STEPS

• PBH is a rare but growing disease with unmet medical need
• GLP-1 plays a critical role in mediating hyperinsulinemic hypoglycemia in PBH
• Avexitide is a targeted therapeutic approach with POC demonstrated in 4 clinical trials
• 28-days of treatment in outpatient setting demonstrated clinically meaningful improvements
  o Reductions in the magnitude of postprandial hyperinsulinemic hypoglycemia
  o Reductions in rates of hypoglycemia and severe hypoglycemia
  o Reductions in rate of rescue
• Avexitide has been well tolerated and has shown no significant safety concerns
• Regulatory guidance in 2019
RYBG SURGERIES IN THE US AND EU

Growing due to Obesity and Effectiveness of Procedure
# PBH Market Snapshot of 2028 (US and EU)

<table>
<thead>
<tr>
<th></th>
<th>US</th>
<th>EU</th>
<th>Total</th>
</tr>
</thead>
<tbody>
<tr>
<td># of Bariatric Surgeries</td>
<td>270K</td>
<td>115K</td>
<td>385K</td>
</tr>
<tr>
<td>% RYGB</td>
<td>30%</td>
<td>35%</td>
<td></td>
</tr>
<tr>
<td>Total RYGB procedures</td>
<td>81K</td>
<td>40K</td>
<td>121K</td>
</tr>
<tr>
<td>Incidence (5-10% Moderate / Severe PBH)</td>
<td>4-8K</td>
<td>2-4K</td>
<td>6-12K</td>
</tr>
<tr>
<td>Prevalence (Previously Diagnosed PBH)</td>
<td>70K</td>
<td>20K</td>
<td>90K</td>
</tr>
</tbody>
</table>

*Based on primary research with KOL and payers by Triangle Insights*
# First-in-Class Therapies in Development

Targeting Rare and Ultra-Rare Diseases with No Approved Treatments

<table>
<thead>
<tr>
<th>Indication</th>
<th>Drug</th>
<th>Regulatory Status</th>
<th>Clinical Status</th>
</tr>
</thead>
<tbody>
<tr>
<td>Hepatitis Delta Virus</td>
<td>Lonafarnib + Ritonavir</td>
<td>• Orphan US &amp; EU • Fast Track &amp; Breakthrough • PRIME EMA</td>
<td>Phase 3</td>
</tr>
<tr>
<td>Hepatitis Delta Virus</td>
<td>Peginterferon Lambda</td>
<td>• Orphan US • Fast Track</td>
<td>Phase 2</td>
</tr>
<tr>
<td>Progeria* and Progeroid Laminopathies</td>
<td>Lonafarnib</td>
<td>• Orphan* US &amp; EU • Breakthrough • Rare Disease Designation</td>
<td>NDA Prep</td>
</tr>
<tr>
<td>Post-Bariatric Hypoglycemia</td>
<td>Avexitide</td>
<td>• Orphan US &amp; EU</td>
<td>Phase 2</td>
</tr>
</tbody>
</table>
## Rare and Ultra-rare Disease Targets

**Advancing Toward NDA**

<table>
<thead>
<tr>
<th>Disease/Target</th>
<th>Q1 2019</th>
<th>Q2 2019</th>
<th>Q3 2019</th>
<th>Q4 2019</th>
</tr>
</thead>
<tbody>
<tr>
<td>HDV Lonafarnib + Ritonavir</td>
<td></td>
<td></td>
<td></td>
<td><strong>Phase 3 Enrolling</strong></td>
</tr>
<tr>
<td>HDV Peginterferon Lambda</td>
<td></td>
<td>LIFT Study Phase 2 Combo Dosing</td>
<td></td>
<td><strong>LIFT Study EOT</strong></td>
</tr>
<tr>
<td>Progeria &amp; PL Lonafarnib</td>
<td></td>
<td></td>
<td>NDA Preparation Ongoing</td>
<td><strong>Expanded Access Program</strong></td>
</tr>
<tr>
<td>PBH Avexitide</td>
<td></td>
<td></td>
<td>Regulatory Guidance</td>
<td></td>
</tr>
</tbody>
</table>
FINANCIAL SUMMARY

- Total cash resources of $112.5 million as of October 25, 2018
- 19.1 million shares outstanding as of October 25, 2018
## EXPERIENCED MANAGEMENT

<table>
<thead>
<tr>
<th>Name</th>
<th>Title and Responsibilities</th>
<th>Logos</th>
</tr>
</thead>
<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>gsk</td>
</tr>
<tr>
<td><strong>DAVID APELIAN, MD, PHD, MBA</strong></td>
<td>Chief Operating Officer&lt;br&gt;Executive Medical Officer</td>
<td>ACHILLION&lt;br&gt;globeImmune&lt;br&gt;Bristol-Myers Squibb&lt;br&gt;Schering-Plough</td>
</tr>
<tr>
<td><strong>SRI RYALI, MBA</strong></td>
<td>Chief Financial Officer</td>
<td>aimmune&lt;br&gt;Jazz Pharmaceuticals&lt;br&gt;ONYX&lt;br&gt;AMGEN</td>
</tr>
<tr>
<td><strong>LISA PORTER, MD</strong></td>
<td>Chief Medical Officer&lt;br&gt;Metabolic Diseases</td>
<td>gsk&lt;br&gt;AMYLIN&lt;br&gt;SmithKline Beecham&lt;br&gt;ZENeca&lt;br&gt;Pharmaceuticals</td>
</tr>
<tr>
<td><strong>JIM SHAFFER, MBA</strong></td>
<td>Chief Business Officer</td>
<td>gsk&lt;br&gt;Halozyme&lt;br&gt;New River Pharmaceuticals&lt;br&gt;MERCK</td>
</tr>
<tr>
<td><strong>INGRID CHOONG, PHD</strong></td>
<td>Vice President&lt;br&gt;Investor Relations and Corporate Development</td>
<td>Sunesis&lt;br&gt;Berkeley&lt;br&gt;University of California</td>
</tr>
<tr>
<td><strong>JOHN FERRARO, MBA</strong></td>
<td>Vice President&lt;br&gt;Clinical Operations</td>
<td>Fate&lt;br&gt;globeImmune&lt;br&gt;SmithKline Beecham</td>
</tr>
<tr>
<td>Name</td>
<td>Title</td>
<td></td>
</tr>
<tr>
<td>---------------------------------------</td>
<td>------------------------------</td>
<td></td>
</tr>
<tr>
<td>THOMAS DIETZ, PHD</td>
<td>Chairman</td>
<td></td>
</tr>
<tr>
<td>EVAN LOH, MD</td>
<td>Independent Director</td>
<td></td>
</tr>
<tr>
<td>ELDON MAYER, MBA</td>
<td>Independent Director</td>
<td></td>
</tr>
<tr>
<td>CHRISTINE MURRAY, MS, RAC</td>
<td>Independent Director</td>
<td></td>
</tr>
<tr>
<td>JEFFREY GLENN, MD, PHD</td>
<td>Independent Director</td>
<td></td>
</tr>
<tr>
<td>DAVID CORY, RPH, MBA</td>
<td>President and CEO</td>
<td></td>
</tr>
<tr>
<td>DAVID APELIAN, MD, PHD, MBA</td>
<td>COO and EMO</td>
<td></td>
</tr>
</tbody>
</table>
A RARE DISEASE COMPANY