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 and the timing of the NDA filing; our ability to meet the quality and documentation requirements for potential approval 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; results of the Phase 3 D-LIVR study; 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 Phase 2 results in any of our clinical trial candidates to date will be indicative of larger, controlled Phase 3 clinical trial results; whether PREVENT Phase 2 study results will support further development of axevitide; our ability to make timely regulatory filings and obtain and maintain regulatory approvals for lonafarnib as a single agent or in combination,ufenimex, 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.

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, including the risks described in the “Risk Factors” section in our Quarterly Report on Form 10-Q for the quarter ended June 30, 2018, and subsequent filings with the Securities and Exchange Commission (SEC). 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.

This presentation concerns products that have not yet been approved for marketing by the FDA. No representation is made as to their safety or effectiveness for the purposes for which they are being investigated.

A shelf registration statement on Form S-3 relating to the public offering of the shares of common stock described above was declared effective by the SEC on December 20, 2017. Before you invest, you should read the prospectus in the registration statement and related preliminary prospectus supplement that we will file with the SEC for more complete information about Eiger and this offering. An electronic copy of the preliminary prospectus supplement and accompanying prospectus relating to the offering will be available on the website of the SEC at www.sec.gov. Copies of the preliminary prospectus supplement, when available, and the accompanying prospectus relating to the offering may also be obtained by contacting BTIG, LLC, 825 Third Avenue, 32nd Floor, New York, NY 10022, by telephone at (212) 593-7555, or by e-mail at equitycapitalmarkets@btig.com.

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EIGER is a late stage biopharmaceutical company focused on the development and commercialization of targeted therapies for multiple rare diseases.

ALL programs have reported critical Phase 2b positive results using well-characterized drugs in targeted rare diseases.

LONAFARNIB is our lead compound advancing into:

- Phase 3 in a single, pivotal trial to treat hepatitis delta virus (HDV) infection by end of 2018
- NDA for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) in 2019
Portfolio of Clinical Programs
Targeting Diverse Rare Indications

Multiple Programs Positioned for Success

HEPATITIS
DELTA VIRUS

PROGERIA

POST-BARIATRIC
HYPOGLYCEMIA
NOVEL TARGETS VALIDATED

Faculty Inventors / Advisors

Jeffrey Glenn, MD, PhD

Leslie Gordon, MD, PhD*

Tracey McLaughlin, MD, MPh

MATCHING DRUGS IDENTIFIED

Partners / Licensors

* volunteer
# 4Q 2018 MILESTONES

**Analyst Day December 11th**

<table>
<thead>
<tr>
<th>Project</th>
<th>Milestones</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>HDV Lonafarnib</strong></td>
<td>Phase 3 Initiating (D-LIVR)</td>
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<td><strong>HDV PEG IFN Lambda</strong></td>
<td>LIFT Study Lambda Combo Dosing (AASLD)</td>
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<td><strong>Progeria Lonafarnib</strong></td>
<td>NDA Preparation (IND), Rare Pediatric Disease Designation (Prevent)</td>
</tr>
<tr>
<td><strong>PBH Avexitide</strong></td>
<td>Phase 2 Data (Prevent)</td>
</tr>
</tbody>
</table>

**Q4 2018 Achieved:**
- HDV Lonafarnib
- PEG IFN Lambda
- PBH Avexitide

**Q4 2018 Planned:**
- HDV Lonafarnib
- Progeria Lonafarnib
- NDA Preparation
- Rare Pediatric Disease Designation
- Expanded Access Program
HEPATITIS DELTA VIRUS (HDV)

OVERVIEW

- HDV is the most severe form of human viral hepatitis
- HDV is always a co-infection with HBV
  - HDV requires HBsAg to complete virion assembly
  - 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 consists of a single stranded, circular RNA virus, with an envelope made up of HBsAg.
**HDV: MOST RAPID PROGRESSION OF VIRAL HEPATITIS**

50% of HDV-Infected Patients are Cirrhotic at Diagnosis

<table>
<thead>
<tr>
<th>Progression to Cirrhosis</th>
</tr>
</thead>
<tbody>
<tr>
<td>HCV</td>
</tr>
<tr>
<td>HBV</td>
</tr>
<tr>
<td>HDV</td>
</tr>
</tbody>
</table>

Westbrook et al, *J Hepatology* 2014
Fattovich et al, *Seminars in Liver Diseases* 2003
AT DIAGNOSIS, >50% OF HDV PATIENTS ARE CIRRHOTIC

Risk of Hepatocellular Carcinoma, Decompensation, Mortality Increase

Evolution from Chronic Active Hepatitis to Cirrhosis

Progression to Cirrhosis (%)

Follow-up Years

HBV + HDV

HBV

P = 0.001

Cumulative Survival

Time to Event (Years)

Survival

HBV

HBV + HDV

P = 0.0002

PEG IFN-ALFA REDUCED HDV RNA IN PATIENTS

Not Approved for HDV

Wobse et al, *Hepatology* 2014
Wedemeyer et al, *Hepatology* 2014
REDUCING HDV-RNA WITH IFNα IMPROVED SURVIVAL

Improved Clinical Benefit without Clearance of HDV-RNA

Interferon-α for 48 weeks with 15 year Follow Up

Farci et al, Gastroenterology 2004: Long-Term Benefit of Interferon-α Therapy of Chronic HDV: Regression of Advanced Hepatic Fibrosis
## HBV Rx APPROVALS AND REGISTRATION ENDPOINTS

### Viral Load Reduction, Biochemical Response, Histologic Improvement

<table>
<thead>
<tr>
<th>Brand (generic)</th>
<th>Approved</th>
<th>Primary Endpoint(s)</th>
<th>Secondary Endpoint(s)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Intron A® (interferon alfa-2b)</td>
<td>1991</td>
<td>• HBeAg + HBV DNA</td>
<td>• HBsAg + ALT + Histology</td>
</tr>
<tr>
<td>Epivir HBV® (lamivudine)</td>
<td>1998</td>
<td>• Histology*</td>
<td>• ALT</td>
</tr>
<tr>
<td>• HBeAg + HBV DNA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• Histology*</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Hepsera® (adefovir dipivoxil)</td>
<td>2002</td>
<td>• Histology*</td>
<td>• HBV DNA + ALT + HBeAg</td>
</tr>
<tr>
<td>• HBV DNA + ALT + HBeAg</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Baraclude® (entecavir)</td>
<td>2005</td>
<td>• Histology*</td>
<td>• HBV DNA + ALT</td>
</tr>
<tr>
<td>• HBV DNA + ALT</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Pegasys® (peginterferon alfa-2a)</td>
<td>2005</td>
<td>• HBeAg</td>
<td>• Histology</td>
</tr>
<tr>
<td>• HBV DNA</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>• ALT</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Tyzeka® (telbivudine)</td>
<td>2006</td>
<td>• HBV DNA + HBeAg or ALT</td>
<td>• Histology + ALT</td>
</tr>
<tr>
<td>Viread® (tenofovir disoproxil fumurate)</td>
<td>2008</td>
<td>• HBV DNA + Histology</td>
<td>• ALT</td>
</tr>
<tr>
<td>Vemlidy® (tenofovir alafenamide)</td>
<td>2016</td>
<td>• HBV DNA</td>
<td>• ALT + HBsAg + HBeAg</td>
</tr>
</tbody>
</table>

* ≥ 2 point decrease in the Knodell necro-inflammatory score with no worsening of the Knodell fibrosis score
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
- Patent issued allowing broad range of lonafarnib + ritonavir doses and durations
- Prenylation is a host target; potential barrier to resistance
ALL-ORAL REGIMEN: IFN-FREE OPTION

Lonafarnib 50 mg BID + Ritonavir 100 mg BID

Change in Log HDV-RNA

Week

LNF 50 mg BID + RTV (N=12) LOWR – 2 STUDY

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

Lonafarnib 50 mg BID + Ritonavir 100 mg BID

Change in Log HDV-RNA

PEG IFN-alfa-2a 180 mcg ± TDF (N=91)
HIDIT – 2 STUDY

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

![Graph showing change in Log HDV-RNA over weeks for different regimens.](Image)

- **LNF 50 mg BID + RTV (N=12)**
- **PEG IFN-alfa-2a 180 mcg ± TDF (N=91)**

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
  - 39% (7 of 18) patients $\geq$ 2 log decline or BLQ at Week 24
  - 60% patients normalized ALT at Week 24

• **Combination:** Lonafarnib boosted with Ritonavir + PEG IFN-alfa-2a
  - 89% (8 of 9) patients $\geq$ 2 log decline or BLQ at Week 24
  - 78% patients normalized ALT at Week 24

• 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
**D-LivR: Phase 3 Study Initiating Q4 2018**

**Delta-Liver Improvement and Virologic Response in HDV**

- **N = 175**
  - LNF 50 mg BID + RTV
  - *All-Oral*

- **N = 125**
  - LNF 50 mg BID + RTV + PEG IFN-alfa-2a
  - *Combination*

- **N = 50**
  - PEG IFN-alfa-2a
  - *Mono*

- **N = 50**
  - Placebo

**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

*All patients will be run-in and maintained on background HBV nucleoside therapy*
PHASE 3  D-LIVR  GLOBAL FOOTPRINT

United States  Canada
United Kingdom  France  Spain
Belgium  Germany  Switzerland
Italy  Greece
Bulgaria  Romania  Moldova
Israel  Turkey  Pakistan
Taiwan  Vietnam  New Zealand
U.S. HDV PATIENT IDENTIFICATION PROGRAM

- 600,000 diagnosed HBV patients provide readily identifiable HDV market
- HDV patients clustered in major metro hotspots

HDV RNA quantification and HBV/HDV reflex test for commercial testing

Patient and physician outreach supporting Eiger’s HDV testing initiative
U.S. MAJOR METRO HOTSPOTS IDENTIFIED

HDV Geographic Footprint is Growing

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 ~ 110,000

Increased Screening Leads to Increased HBV and HDV Diagnosis

* 11.8% of HBV Patients May Be Co-infected with HDV

Newly Diagnosed Chronic HBV Patients

% of Chronic HBV Patients with HDV

Newly Diagnosed HDV Patients in the U.S. Each Year
PEGYLATED INTERFERON 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 HDV “MONO”: PHASE 2 STUDY**

**Lambda Interferon MonoTherapy Study in HDV**

- **Arm 1**
  - n = 17
  - LMD 120 mcg QW
  - Follow-up

- **Arm 2**
  - n = 16
  - LMD 180 mcg QW
  - Follow-up

---

**48 weeks**

**24 weeks**

---

**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</th>
</tr>
</thead>
<tbody>
<tr>
<td>120 mcg*</td>
<td>14/17</td>
<td>-1.5 log</td>
<td>6 of 14 (42.9%)</td>
</tr>
<tr>
<td>180 mcg*</td>
<td>10/16</td>
<td>-2.4 log</td>
<td>6 of 10 (60.0%)</td>
</tr>
</tbody>
</table>

* Randomization dose

Change in Log HDV-RNA

Week

Mean 180 mcg

Mean 120 mcg
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 STUDY**

**Lambda InterFeron combination Therapy**

- Open-label, Phase 2 study evaluating Lambda + LNF + RTV
- To be conducted at the NIH
- Enrollment and Dosing Ongoing

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

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

*N ~ 26

Lambda + LNF / RTV Combination

Follow Up

* 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 PROGRAM: DEVELOPING MULTIPLE TREATMENT OPTIONS

<table>
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<td>D-LIVR</td>
<td>LIMT Study Lambda Mono EOT Data</td>
</tr>
<tr>
<td>Q4 2018</td>
<td>AASLD</td>
</tr>
</tbody>
</table>
EIGER HDV PROGRAM UPDATES AT AASLD 2018

- Liver Institute and Foundation for Education and Research: The Search for HBV/HDV Cure, L.I.F.E.R. Meeting, November 9
- Eiger Phase 3 D-LIVR Study Investigator Reception, November 9
- Hepatitis Delta International Network (HDIN) Meeting, November 10
Chronic Hepatitis Delta Virus Infection leads to the most severe form of human viral hepatitis and a growing unmet medical need in the United States and Western Europe.

Eiger BioPharmaceuticals is committed to developing treatments for HDV patients worldwide.

D-LIVR is the first-ever, Phase 3 study in Hepatitis Delta Virus (HDV) Infection.

D-LIVR will evaluate an “All-Oral” regimen of investigational drug LONAFARNIB boosted with RITONAVIR and a “Combination” regimen with PEGYLATED INTERFERON-ALFA in HDV-infected patients.

D-LIVR is a global study, currently activating clinical sites across 19 countries around the world.
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**

- **Lonafarnib blocks production of Progerin**

- 50 amino acids deleted
- **LONAFARNIB**
- No cleavage site
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

Gordon, L et al. JAMA, 2018, 319(16): 1687
LONAFARNIB IMPROVES SURVIVAL IN PROGERIA

77% Reduction in Risk of Mortality Compared to No Treatment

![Graph showing survival probability over time for treated and untreated groups with hazard ratio and p-value]

Hazard Ratio = 0.23, p = 0.04

Gordon, L et al, JAMA, 2018, 319(16): 1687
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 PROGRAM: PREPARING NDA

**NDA Filing Planned in 2019**

<table>
<thead>
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**PROGERIA PROGRAM: PREPARING NDA**

**NDA Filing Planned in 2019**

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<td>IND Rare Pediatric Disease Designation</td>
</tr>
<tr>
<td>Planned</td>
<td>Planned</td>
</tr>
</tbody>
</table>

**IND**

**Expanded Access Program**
PROGERIA AND HDV

Distinct Diseases, Distinct Treatment Regimens, Distinct Commercial Strategies

PROGERIA

Lonafarnib Monotherapy (weight-based)

HDV

Lonafarnib Boosted with Ritonavir ± Pegylated Interferon-Alfa
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

Craig et al. Diabetes, Obesity and Metabolism 2017

Autoonomic
• 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
# PHASE 2 CLINICAL 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>Topline data press released October 16, 2018</td>
</tr>
</tbody>
</table>
**AVEXITIDE REDUCES PBH**

Single Ascending Dose Study Results

- All subjects required rescue
- Rapid decline
- Rate of glucose decline reduced
- 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.
PREVENT 28-DAY PHASE 2 OUTPATIENT STUDY

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

Primary Endpoint: Magnitude of postprandial hypoglycemia defined as the plasma glucose nadir occurring within 3 hours of mixed meal tolerance test (MMTT)

Secondary Endpoints: Postprandial neuroglycopenic signs & symptoms; peak postprandial insulin response; require glucose rescue during MMTT

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

Primary Endpoint Achieved

- Placebo
  - 30 mg BID
  - 60 mg QD

- Glucose Nadir:
  - Placebo: 47.1 mg/dL
  - 30 mg BID: 57.1 mg/dL
  - 60 mg QD: 59.2 mg/dL

- Significance:
  - Placebo vs 30 mg BID: P=0.0011
  - Placebo vs 60 mg QD: P=0.0002
  - 30 mg BID vs 60 mg QD: P=0.0011

28-day Study
REDUCED POSTPRANDIAL INSULIN PEAK

Secondary Endpoint Achieved

![Graph showing reduced postprandial insulin peak]
FEWER EPISODES OF HYPOGLYCEMIA WITH AVEXITIDE

Exploratory Secondary Endpoints Achieved with CGM

- Metabolic and clinical improvements corroborated by continuous glucose monitoring (CGM)
- Fewer episodes of hypoglycemia (< 70 mg/dL)
- Fewer episodes of severe hypoglycemia (< 55 mg/dL)
- Fewer neuroglycopenic symptoms confirmed by CGM
**PREVENT PHASE 2 STUDY**

Clinically Meaningful Improvements Throughout 28-days of Avexitide Treatment

- Improved postprandial glucose nadir
- Reduced postprandial insulin peak
- Fewer episodes of hypoglycemia; less rescue required
- Statistical significance achieved with QD and BID dosing
- Well tolerated
- No approved therapy

Next Steps: Regulatory Guidance in 2019
# PBH PROGRAM: REGULATORY GUIDANCE IN 2019

<table>
<thead>
<tr>
<th>Q4 2018</th>
<th>( \text{HDV Lonafarnib} )</th>
<th>( \text{HDV PEG IFN Lambda} )</th>
<th>( \text{Progeria Lonafarnib} )</th>
<th>( \text{PBH Avexitide} )</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Phase 3 Initiating</td>
<td>LIFT Study Lambda Combo Enrolling</td>
<td>LIMT Study Lambda Mono EOT Data</td>
<td>Rare Pediatric Disease Designation</td>
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</table>

51Achieved
## 4Q 2018 MILESTONES

**Analyst Day December 11th**

<table>
<thead>
<tr>
<th><strong>HDV</strong> Lonafarnib</th>
<th><strong>Q4 2018</strong></th>
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<td>Phase 3 Initiating</td>
<td>D-Livr</td>
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<th><strong>HDV</strong> PEG IFN Lambda</th>
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<tr>
<th><strong>Progeria</strong> Lonafarnib</th>
<th>NDA Preparation</th>
<th>Rare Pediatric Disease Designation</th>
<th>IND</th>
<th>Expanded Access Program</th>
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<tr>
<th><strong>PBH</strong> Avexitide</th>
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<tbody>
<tr>
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<td>PREVENT</td>
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</tbody>
</table>

**52**

- **Achieved**
- **Planned**
**FINANCIAL SUMMARY**

- Raised net proceeds of $47.7 million in October 2018 financing
- $64.9 million cash as of September 30, 2018
- Total cash resources of $112.6 million as of October 25, 2018
- 19.1 million shares outstanding as of October 25, 2018
- Analyst Day on December 11, 2018 -- St. Regis New York
# EXPERIENCED MANAGEMENT

<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
</tr>
</thead>
<tbody>
<tr>
<td>DAVID CORY, RPH, MBA</td>
<td>President and Chief Executive Officer</td>
</tr>
<tr>
<td>DAVID APELIAN, MD, PHD, MBA</td>
<td>Chief Operating Officer and Executive Medical Officer</td>
</tr>
<tr>
<td>JIM WELCH, MBA</td>
<td>Chief Financial Officer</td>
</tr>
<tr>
<td>LISA PORTER, MD</td>
<td>Chief Medical Officer - Metabolic Diseases</td>
</tr>
<tr>
<td>JIM SHAFFER, MBA</td>
<td>Chief Business Officer</td>
</tr>
<tr>
<td>INGRID CHOONG, PHD</td>
<td>Vice President - Investor Relations and Corporate Development</td>
</tr>
<tr>
<td>JOHN FERRARO, MBA</td>
<td>Vice President - Clinical Operations</td>
</tr>
</tbody>
</table>
## Seasoned Board

<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
</tr>
</thead>
<tbody>
<tr>
<td>Thomas Dietz, PhD</td>
<td>Chairman</td>
</tr>
<tr>
<td>David Cory, RPh, MBA</td>
<td>President and CEO</td>
</tr>
<tr>
<td>David Apelian, MD, PhD, MBA</td>
<td>COO and EMO</td>
</tr>
<tr>
<td>Evan Loh, MD</td>
<td>Independent Director</td>
</tr>
<tr>
<td>Jeffrey Glenn, MD, PhD</td>
<td>Independent Director</td>
</tr>
<tr>
<td>Eldon Mayer, MBA</td>
<td>Independent Director</td>
</tr>
</tbody>
</table>
COMMITTED TO RARE DISEASES