

# Targeted Therapies for Serious Rare and Ultra-Rare Diseases

**Corporate Presentation**  
September 2021









# Forward Looking Statements

This presentation 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. Forward-looking statements are our current statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our anticipated significant milestones in 2021 and 2022; the timing of our ongoing and planned clinical development; the sufficiency of our cash, cash equivalents and investments to fund our operations into the fourth quarter of 2023; our development programs for Zokinvy generally; and the potential approval of Zokinvy in jurisdictions outside of the U.S., including the EU in 2021; our progression and continued enrollment of our Phase 3 D-LIVR study in HDV and expectations regarding the timing and availability of topline data; our ability to maintain supply of our commercial and clinical trial materials; our plans to advance Peginterferon Lambda in HDV in the U.S. and EU; our progression of Peginterferon Lambda for COVID-19 and Avexitide for post-bariatric hypoglycemia and congenital hyperinsulinism; our ability to finance the continued advancement of our development pipeline products; and the potential for success of any of our product candidates. These statements concern product candidates that have not yet been approved for marketing by the U.S. Food and Drug Administration (FDA). No representation is made as to their safety or effectiveness for the purposes for which they are being investigated. Various important factors could cause actual results or events to differ materially from the forward-looking statements that Eiger makes, including additional applicable risks and uncertainties described in the "Risk Factors" sections in the Quarterly Report on Form 10-Q for the quarter ended June 30, 2021 and Eiger's subsequent filings with the SEC. The forward-looking statements contained in this press release are based on information currently available to Eiger and speak only as of the date on which they are made. Eiger does not undertake and specifically disclaims any obligation to update any forward-looking statements, whether as a result of any new information, future events, changed 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.

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.

# Diverse, Late-Stage Pipeline

| TARGETED INDICATION  | DRUG  | ORPHAN<br>US / EU | BREAKTHROUGH<br>THERAPY | RARE PEDIATRIC<br>DISEASE | STATUS                            |
|--|---|-------------------|-------------------------|---------------------------|-----------------------------------|
|  Hepatitis Delta Virus                | Lonafarnib  | ✓                 | ✓                       | N/A                       | Phase 3<br>>90% Enrolled*         |
|  | Peginterferon Lambda  | ✓                 | ✓                       | N/A                       | Phase 3<br>Initiating             |
|  Post-Bariatric Hypoglycemia          | Avexitide   | ✓                 | ✓                       | N/A                       | Phase 3<br>Ready                  |
|  Congenital Hyperinsulinism           |   | ✓                 | ✓                       | ✓                         | Phase 2                           |
|  COVID-19                            | Peginterferon Lambda  | N/A               | N/A                     | N/A                       | Phase 3<br>Enrolling              |
|  Progeria & Progeroid Laminopathies |  <b>Zokinvy</b> <sup>®</sup><br>(lonafarnib)<br>capsules 50 mg/75 mg | ✓                 | ✓                       | PRV<br>Sold               | FDA Approved;<br>MAA Under Review |

# Expected Key Milestones in 2021

## CATALYSTS FOR VALUE CREATION

Lonafarnib for  
HDV

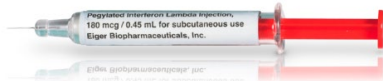
**D-LIVR**



**Phase 3  
Enrollment Completion**

Lambda for  
HDV

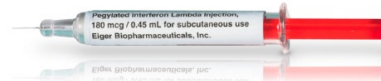
**L↓MT-2**



**Phase 3  
Study Enrolling**

Lambda for  
COVID-19

**together**  
clinical trials



**Phase 3  
Interim Futility Analysis**

Zokinvy® for  
Progeria & PL

**Zokinvy®**  
(lonafarnib)  
capsules 50 mg/75 mg

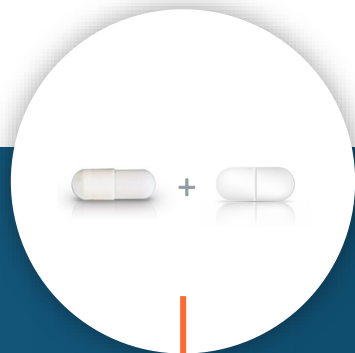


**EMA Approval**



# Eiger HDV Platform in Phase 3

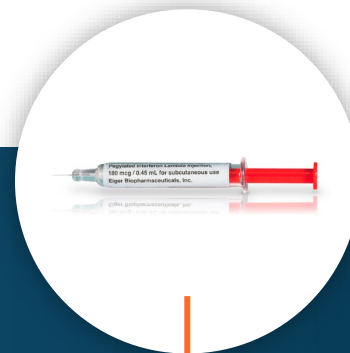
## FOUNDATIONAL THERAPIES FOR FUTURE COMBINATIONS



Lonafarnib/Ritonavir

ORAL

**D-LIVR**



Peginterferon Lambda

WEEKLY SUB Q

**LMT-2**

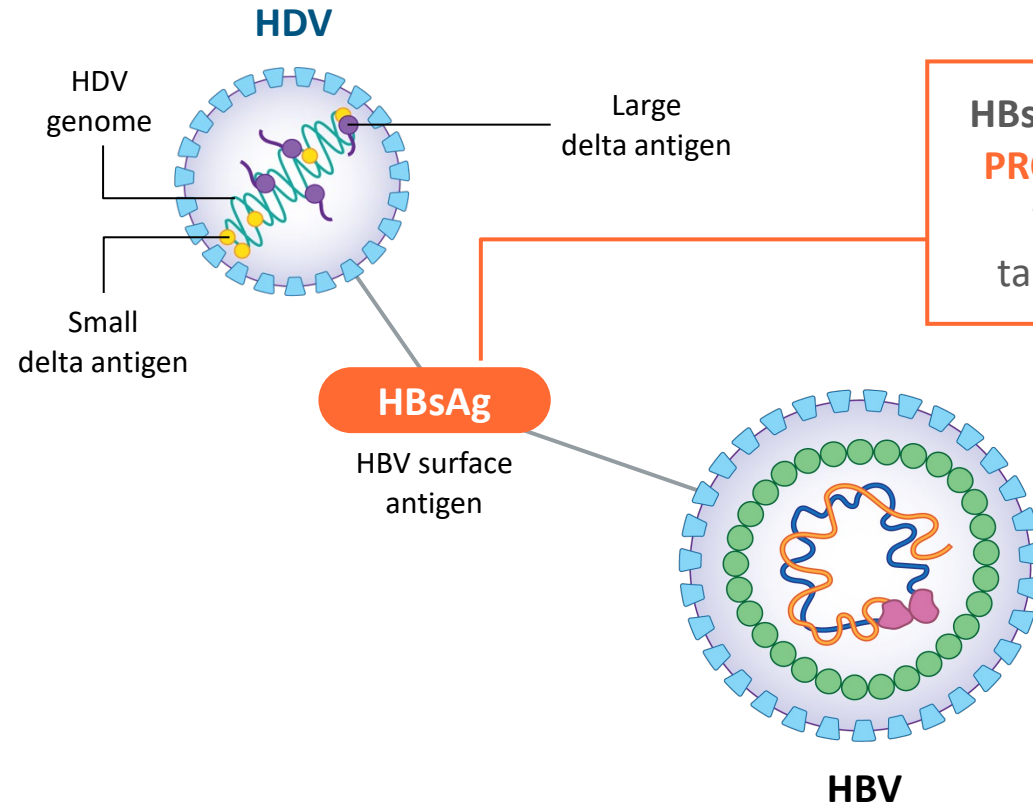
Convenient administration for improved patient compliance  
Potential HDV cure and maintenance therapies



# HDV is Always a Co-infection with HBV

## HDV REQUIRES HBsAg TO COMPLETE VIRUS ASSEMBLY

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







# HDV: Most Severe Form of Viral Hepatitis

60% OF HDV-INFECTED PATIENTS DIE WITHIN 10 YEARS AFTER INFECTION

## Co-infection with HBV

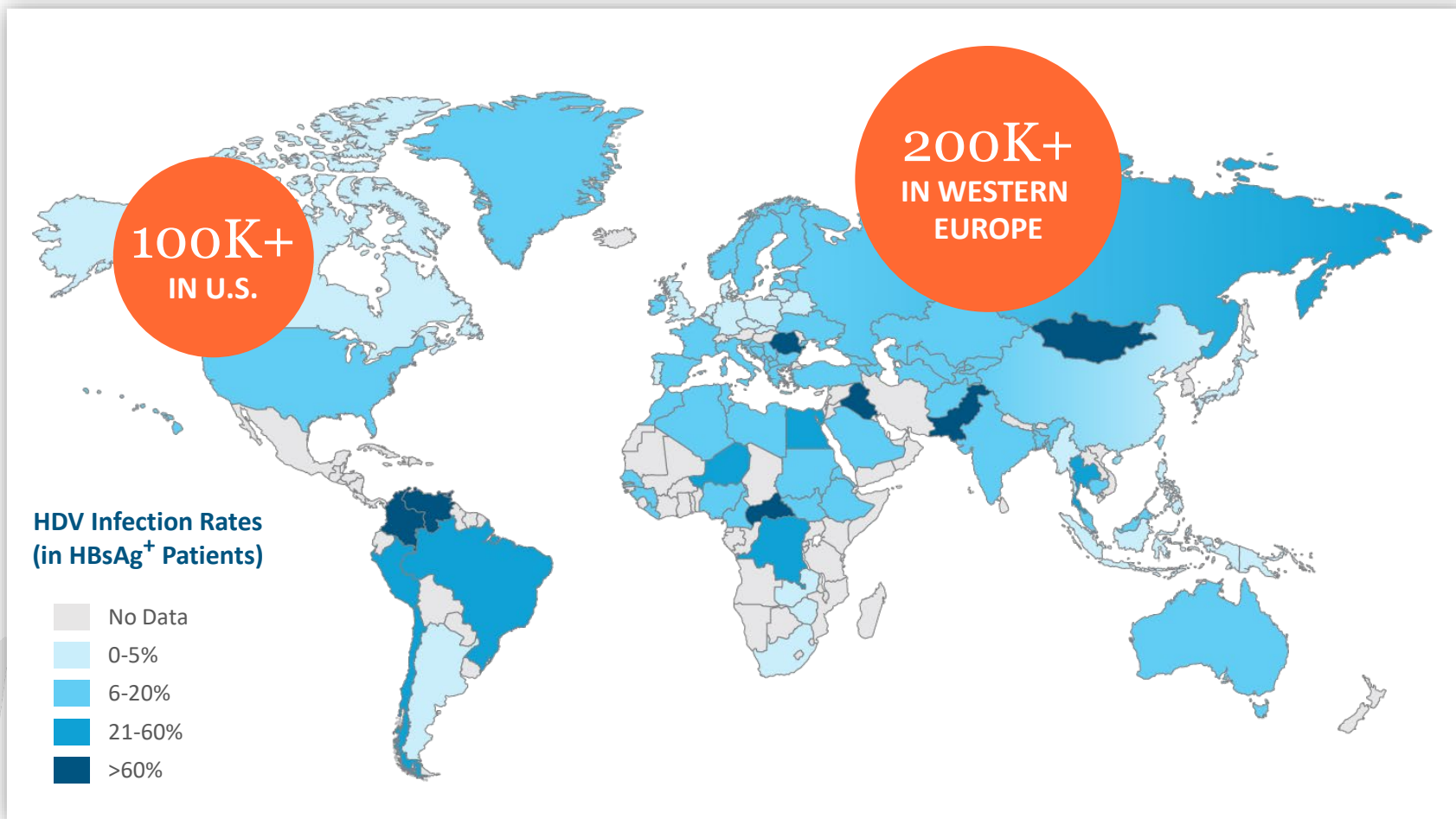


| Progression to Cirrhosis |                              |
|--------------------------|------------------------------|
| HCV                      | 10-20% within 20 Years       |
| HBV                      | 20% within 5 Years           |
| <b>HDV</b>               | <b>70% within 5-10 Years</b> |



# 12M HDV Patients Worldwide

~4-6% OF HBV-INFECTED POPULATION



Migration  
Contributing to  
**Globalization**  
of Disease





# \$1B+ HDV Market Opportunity

ONLY 3% MARKET PENETRATION REQUIRED



## ADDRESSABLE MARKET

**~300,000 Patients<sup>1</sup>**

**~100K**  
in US

**~200K**  
in EU



## PENETRATION REQUIRED FOR \$1B SALES

**~3% of Patients<sup>2</sup>**

**~3K**  
in US

**~6K**  
in EU



## ORPHAN PRICING

**Per Year<sup>3</sup>**

**~\$150,000**  
in US

**~\$98,000**  
in EU



# What Does a Win Look Like for HDV Patients?

CONSISTENT WITH FDA GUIDANCE ON DEVELOPMENT OF TREATMENTS FOR HDV



- Reduction in HDV Viral Load
- Improvement in Liver Inflammation (ALT)



- Slows Disease Progression
- Improves Liver Histology
- Improves Survival



# Lonafarnib for HDV

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

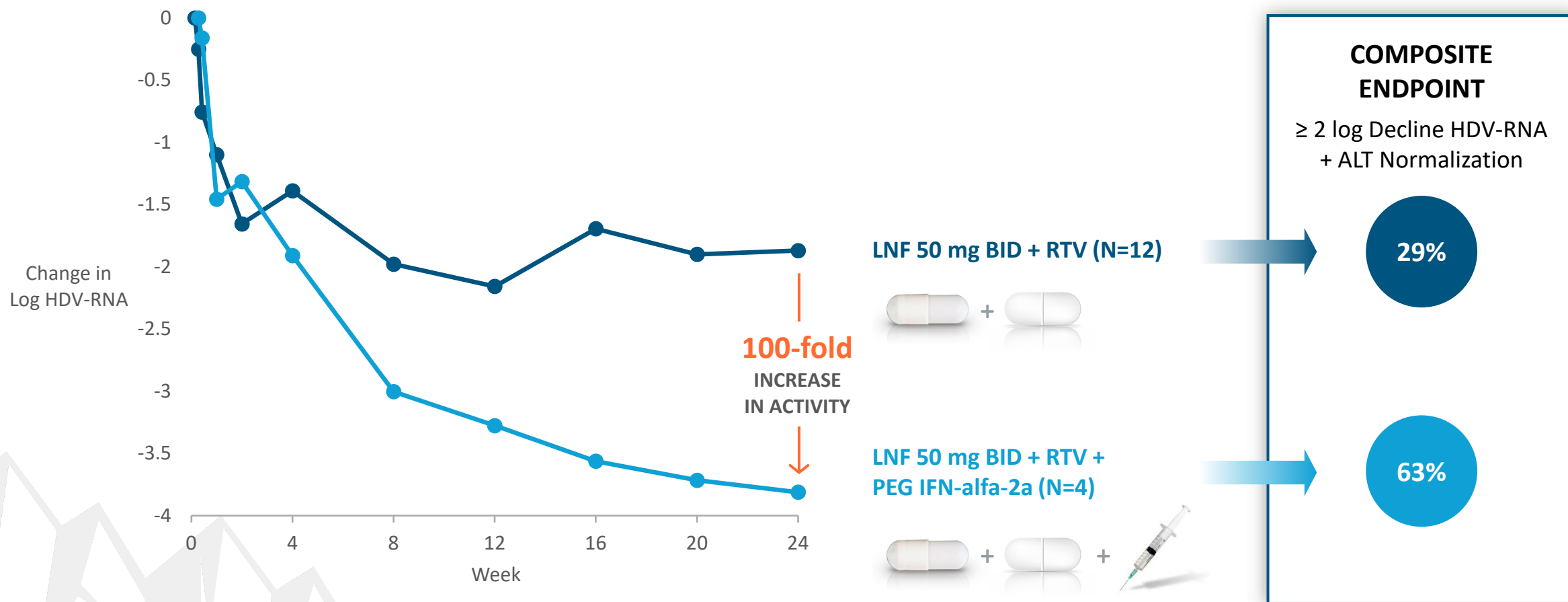
- Well-characterized in patients
  - > 2,000 patients dosed in oncology program by Merck (Schering)
  - > 90 children dosed in Progeria program by Boston Children's Hospital
  - > 170 patients dosed in HDV program
  - Longest duration of dosing > 10 years
- Most common AEs are GI related
- **Orphan Designation** U.S. and EU
- **FDA Breakthrough Therapy** Designation
- **EMA PRIME** Designation
- Patent estate covers broad range of lonafarnib + ritonavir doses and durations





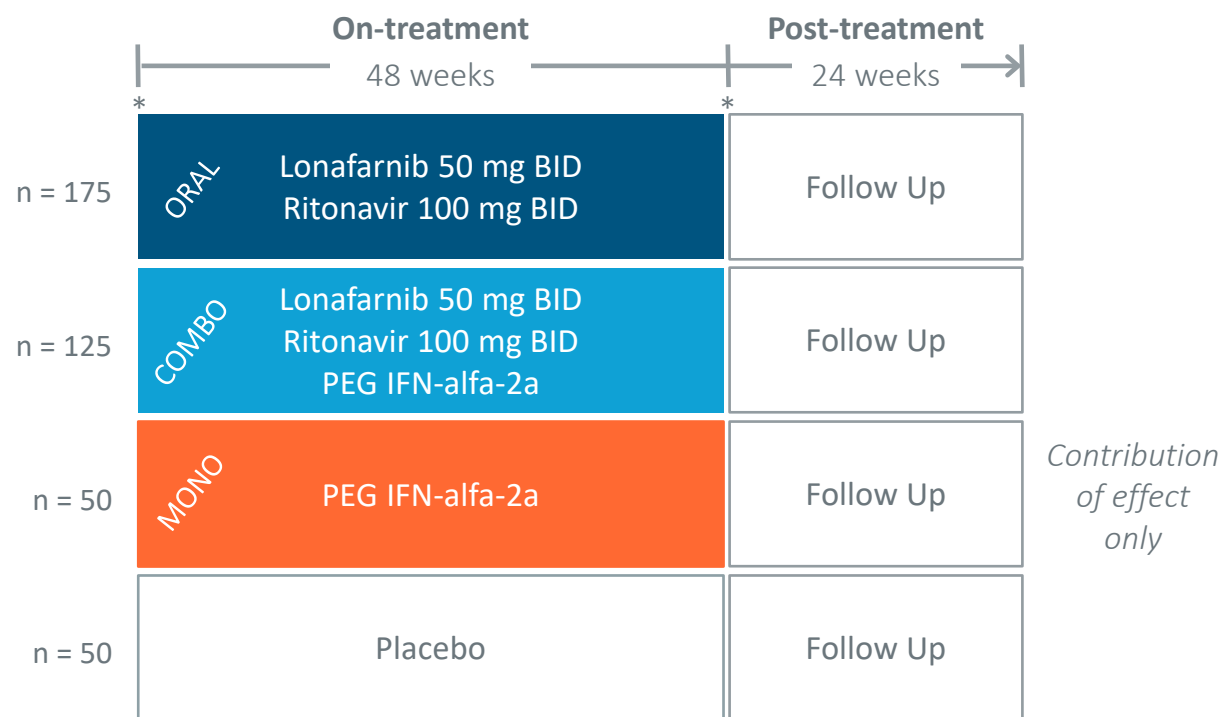
# Lonafarnib Phase 2 Data

## TWO LONAFARNIB-BASED REGIMENS IDENTIFIED FOR REGISTRATION



# D-LIVER Phase 3 Global Study

## MULTIPLE PATHWAYS TO APPROVAL



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

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

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

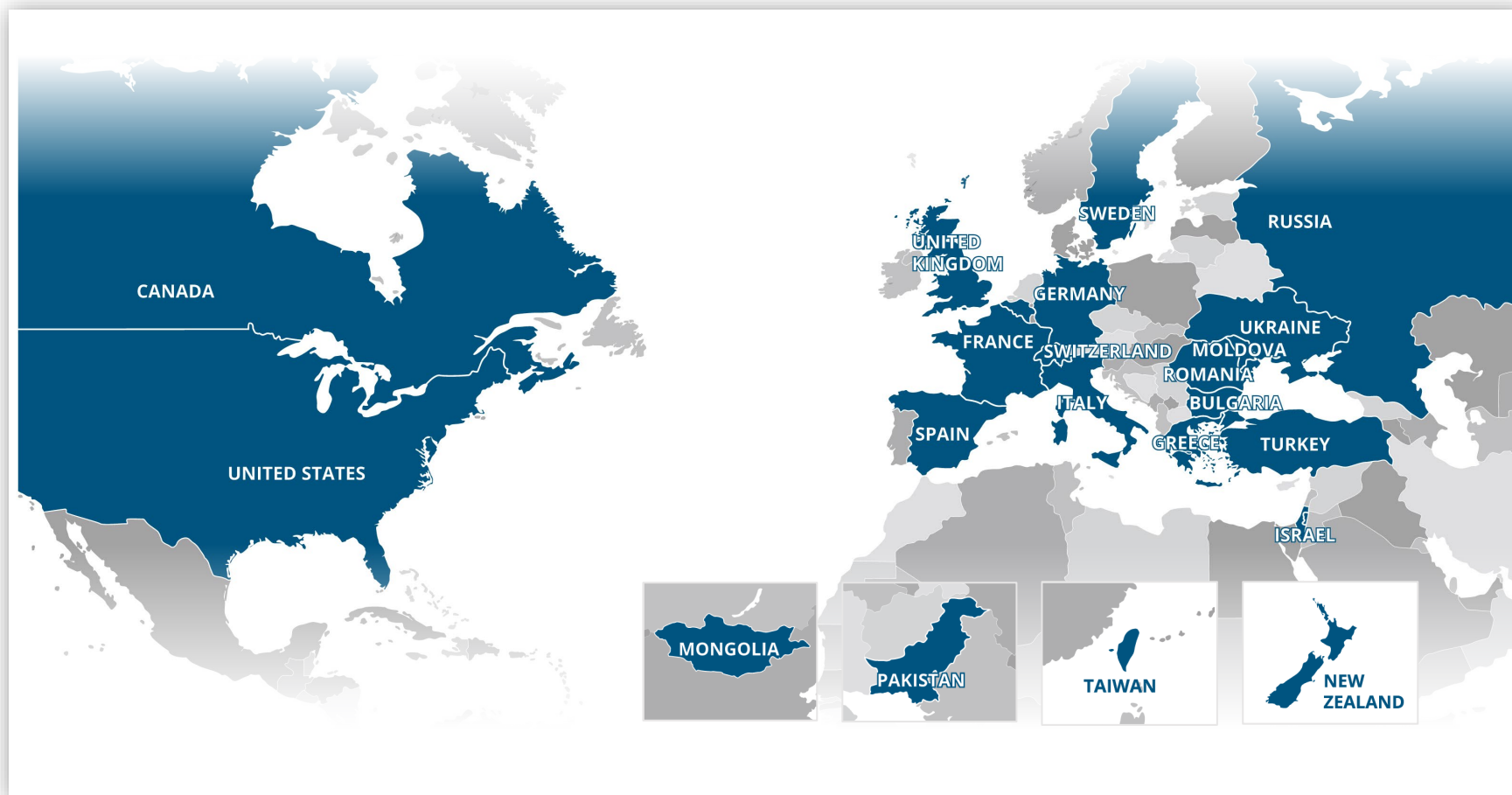
# D-LIVR Phase 3 Global Study



>90% Enrolled\*

Full Enrollment in 2021

20+ 100+  
COUNTRIES SITES







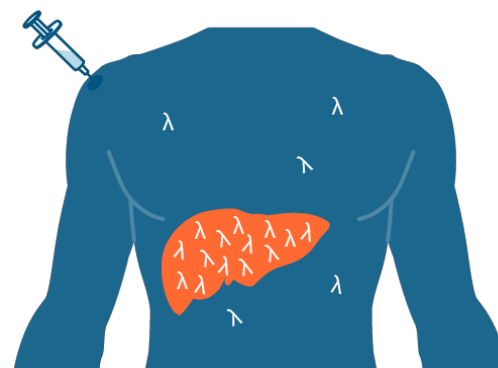
# Peginterferon Lambda (Lambda) for HDV

## A WELL TOLERATED TYPE III INTERFERON

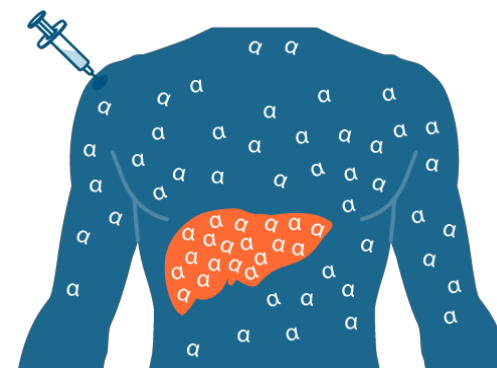
- Binds to a unique receptor vs type I IFN- $\alpha$ 
  - Highly expressed on hepatocytes
  - Limited expression on hematopoietic and CNS cells
- Uses similar downstream signaling pathway to IFN- $\alpha$
- 3,000+ patients in 19 clinical trials (HCV / HBV / HDV)
- **Orphan Designation** in U.S. and EU
- **FDA Breakthrough Therapy Designation**
- Composition of matter and method of use patents

### Lambda Receptors Highly Expressed in the Liver

LAMBDA RECEPTORS NOT WIDELY  
DISTRIBUTED THROUGHOUT BODY



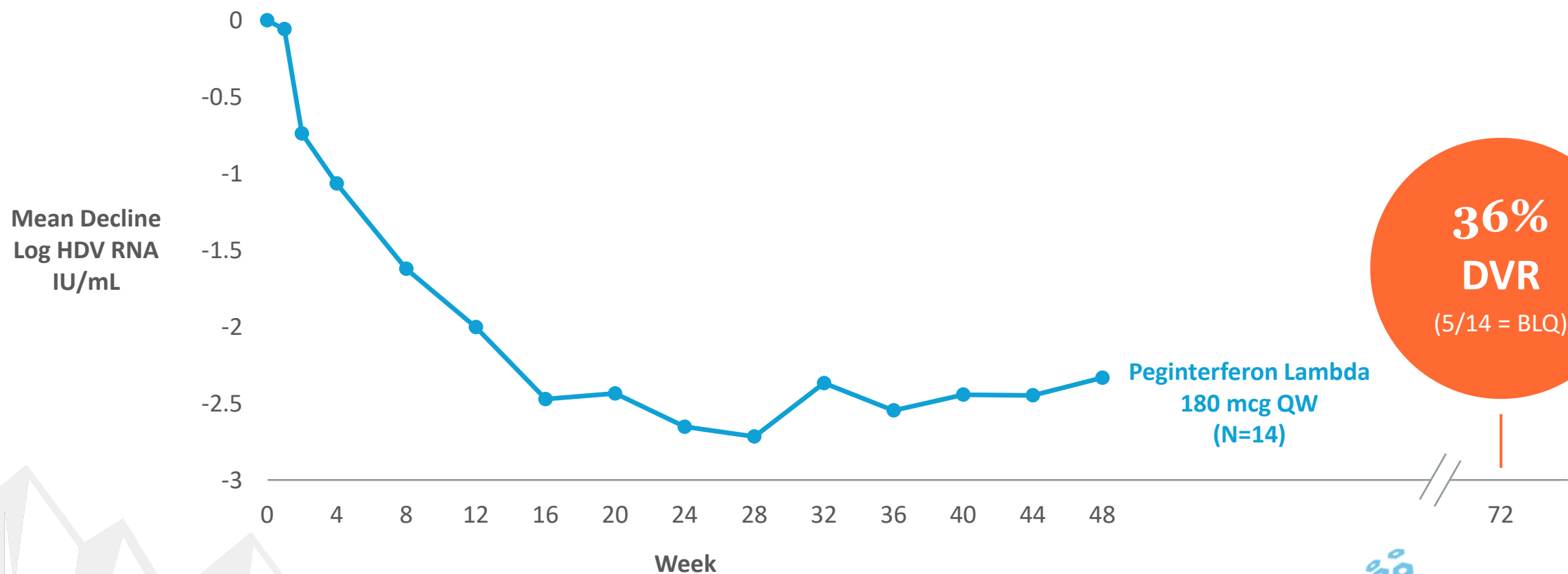
IFN- $\alpha$  RECEPTORS WIDELY  
DISTRIBUTED THROUGHOUT BODY





# Phase 2 Peginterferon Lambda Study Results

**36% DURABLE VIROLOGIC RESPONSE (DVR) WITH PEGINTERFERON LAMBDA**

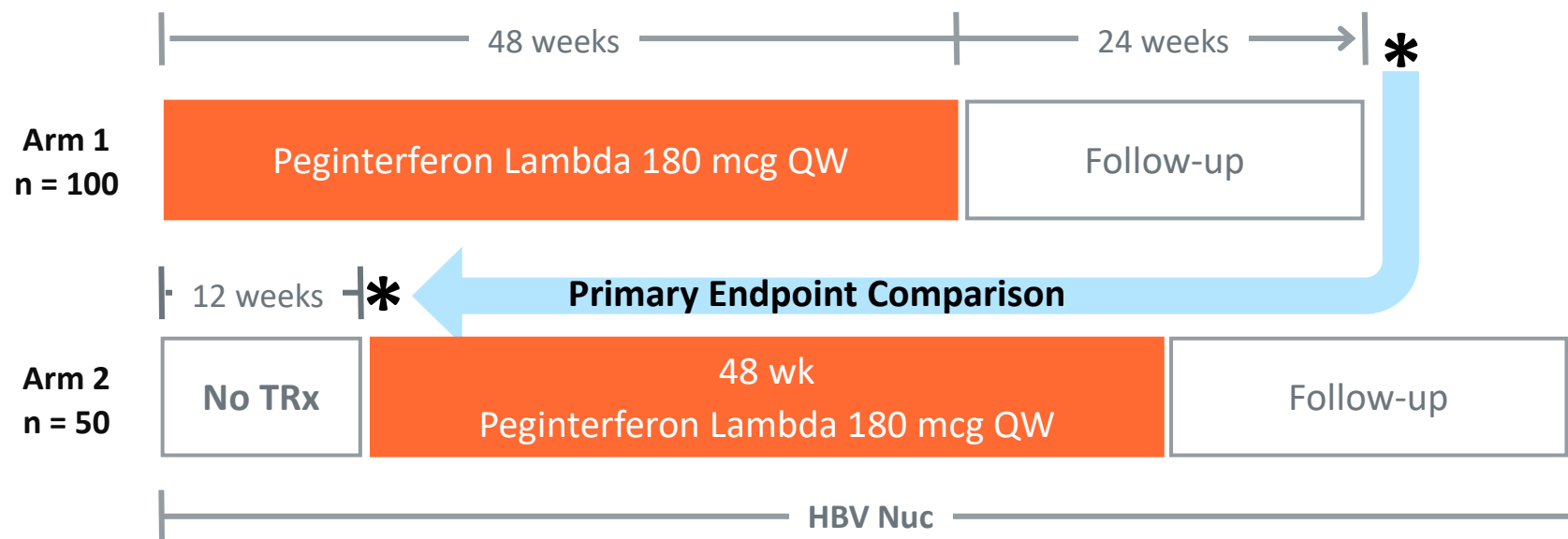
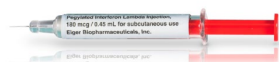




# Peginterferon Lambda Phase 3 Study of HDV



FIRST PATIENT ENROLLED BY YEAR END 2021



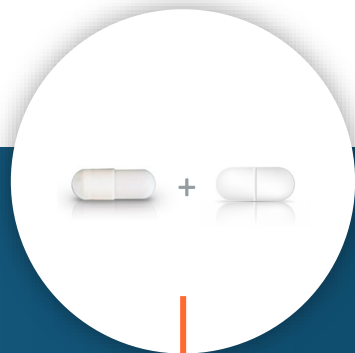
## Primary Endpoint\*

DVR at 24 Weeks Post-TRx (Arm 1) versus 12 Weeks Post-No TRx (Arm 2)



# Eiger HDV Platform in Phase 3

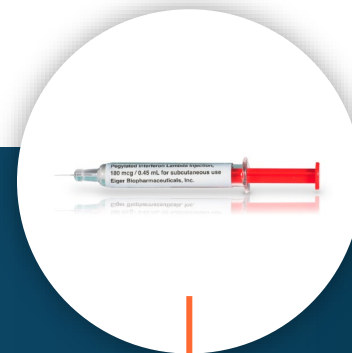
## FOUNDATIONAL THERAPIES FOR FUTURE COMBINATIONS



Lonafarnib/Ritonavir

ORAL

**D-LIVR**



Peginterferon Lambda

WEEKLY SUB Q

**LMT-2**

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Potential HDV cure and maintenance therapies



# Peginterferon Lambda for COVID-19

## POTENTIAL AS A CONVENIENT, OUTPATIENT THERAPY FOR NEWLY DIAGNOSED PATIENTS

- Positive Phase 2 *ILIAD* results in newly diagnosed COVID-19 outpatients
- Potential to improve clinical outcomes and curb community spread
- Treatment well tolerated\*
- Resistance due to variants of SARS-CoV-2 may not be an issue due to mechanism of action
- Provided in a prefilled syringe as a convenient, single dose, outpatient treatment
- Phase 3 *TOGETHER* investigator sponsored study enrolling in Brazil

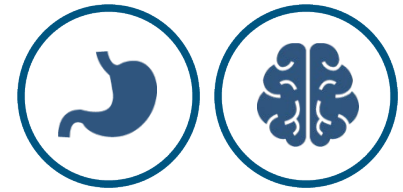




- Multi-center, investigator-sponsored, randomized, placebo-controlled Phase 3 study
- Evaluating multiple therapeutics in newly diagnosed, non-hospitalized COVID-19 patients
- **TOGETHER** includes an investigational arm of Peginterferon Lambda as a single subcutaneous dose
- Peginterferon Lambda arm enrolling up to 800 high-risk, non-hospitalized patients
- Primary endpoint is reduction of emergency room visits and hospitalizations
- Recruiting at eleven sites in Brazil
- Interim analysis for futility expected by year-end



# Avexitide Is a First-in-Class GLP-1 Antagonist



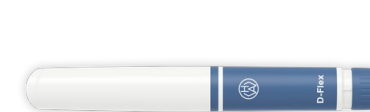
## TARGETED MOA FOR POST-BARIATRIC HYPOGLYCEMIA AND CONGENITAL HYPERINSULINISM

- Novel Liquid Formulation Developed
- Sub-cutaneous Delivery
- Targeted Mechanism of Action
- Differential Device Strategies for PBH & HI
- Patent Protection Will Provide Market Exclusivity Through at Least 2039



# Avexitide for PBH

PHASE 3 READY IN 2022



## POST-BARIATRIC HYPOGLYCEMIA (PBH)



- Complication of bariatric surgery
- Dangerously low blood sugar after meals
- ~**5-10%** of Roux-en-Y Gastric Bypass
- ~**2.5%** of Vertical Sleeve Gastrectomy
- ~150K PBH patients in U.S. & EU5
- Other procedures: esophagectomy, gastrectomy, Nissen fundoplication

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

## CLINICAL & REGULATORY STATUS

- Proof of concept demonstrated in 54 patients dosed across 4 completed Phase 2 studies
- Most recent Phase 2 data published in *JCEM* 2021
- FDA **Breakthrough Therapy** Designation for Hypoglycemic Hypoglycemia
- Concurrence with FDA and EMA on single pivotal study (N=90) with end points previously demonstrated in Phase 2

Phase 3 Ready in 2022:  
Manufacturing and device development ongoing

# Avexitide for HI

## FDA DISCUSSIONS ONGOING FOR PHASE 3 STUDY DESIGN



### CONGENITAL HYPERINSULINISM (HI)



- Ultra-rare pediatric metabolic disorder
- Most frequent cause of persistent hypoglycemia in neonates and children
- >3,300 patients in U.S. & EU5
- Well-defined market; multiple therapies in development
- Near-total pancreatectomy is indicated

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

### CLINICAL & REGULATORY STATUS

- Proof of concept demonstrated in 39 patients across 3 Phase 2 studies (neonates, children, adolescents/adults)
- Clinically significant endpoint (glucose infusion rate) demonstrated
- FDA **Rare Pediatric Disease Designation**; PRV eligible
- FDA **Breakthrough Therapy Designation**
- Differentiated from PBH based on dose and device

FDA discussions ongoing for Phase 3 neonate/child studies



# FDA Approved to Reduce the Risk of Mortality in Hutchinson-Gilford Progeria Syndrome (Progeria)



NORD®  
National Organization  
for Rare Disorders

2021 INDUSTRY INNOVATION AWARD

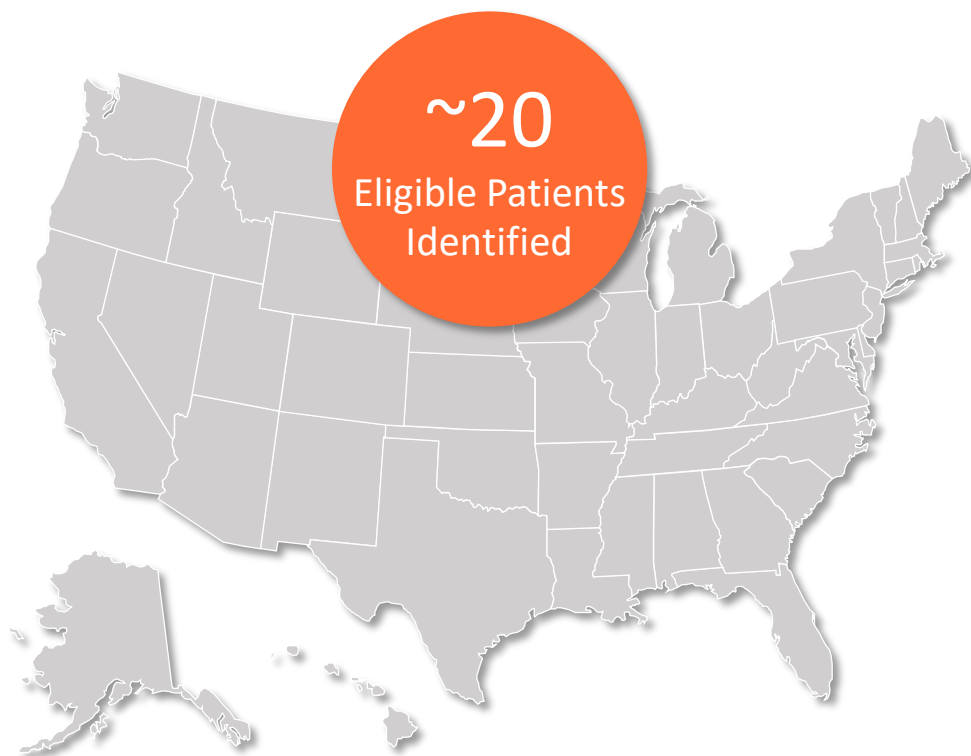


Photos courtesy of The Progeria Research Foundation and Progeria Family Circle

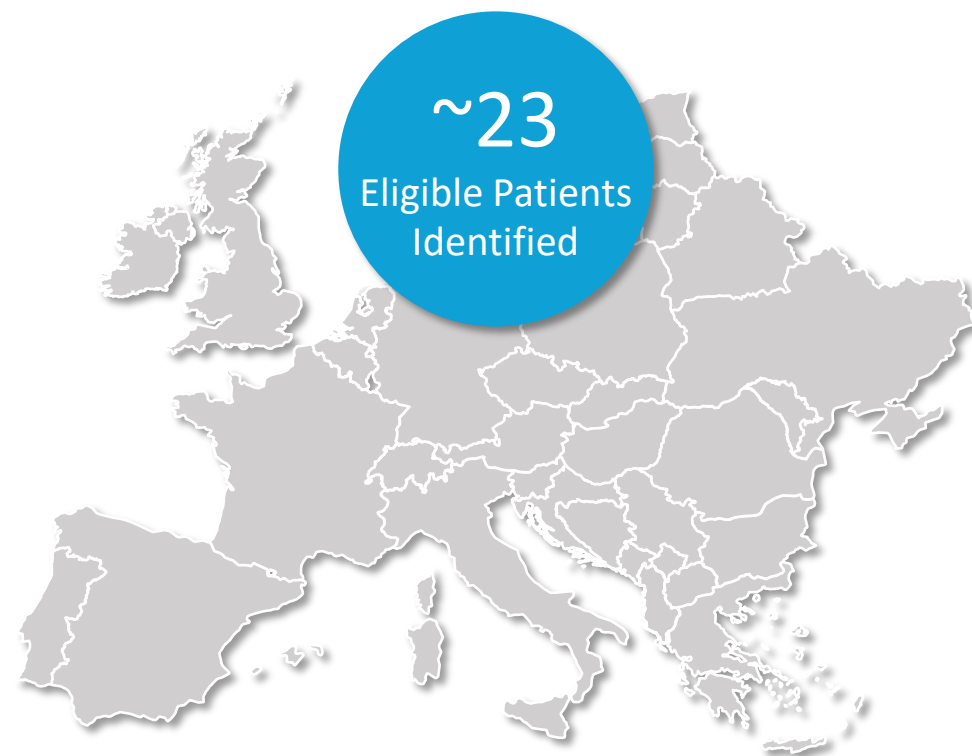




## Initial Commercial Focus in U.S. and Europe



**U.S. – Commercially Launched in Jan 2021**



**Europe – Preparing for Commercial Launch**



- Phase 3 *D-LIVR*: >90% enrolled\*; full enrollment by end of 2021
- Phase 3 *LIMT-2* study first patient by end of 2021
- Avexitide manufacturing and device development in 2021
- Phase 3 *TOGETHER* study interim futility analysis by end of 2021
- Zokinvy EMA approval expected in 2021
- Strong cash balance: planned operations funded into 4Q 2023