# Eiger BioPharmaceuticals Reports on 2018 R&D Day

- **Late Stage Rare and Ultra-Rare Disease Pipeline Advancing**
- **>$100M in Cash Available to Achieve Key Milestones**

**PALO ALTO, Calif., December 11, 2018 –** Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare and ultra-rare diseases, today announced recent accomplishments reported at its R&D Day at the St. Regis Hotel in New York City.

Eiger management reported progress in all programs of its late stage pipeline, including clinical and regulatory program updates, market development, and expanded access.

*Key Announcements:*

- Hepatitis Delta Virus (HDV) Program
    - Phase 3 D-LIVR (Lonafarnib-based regimens) Study
        - First site initiated - University of Miami
        - First patient dosed expected Q1 2019
    - Phase 2 LIMT (PEG IFN-Lambda Mono) Study End of Treatment Data
        - Comparable anti-HDV activity to historical PEG IFN Alfa at Week 48
        - Well-tolerated overall
        - Opportunity for mono or combo Rx development with Lonafarnib
    - Phase 2 LIFT (PEG IFN-Lambda Combo with Lonafarnib) Study at NIH
        - 24 of 26 patients enrolled
        - Dosing expected to complete mid-2019

- Progeria and Progeroid Laminopathies (PL) Program
    - Rare Pediatric Drug (RPD) Designation granted by FDA for Progeria and PL
    - IND acceptance for Lonafarnib in Progeria and PL
    - Orphan Designation granted by EMA for Lonafarnib in Progeria
    - Expanded Access Program being implemented
    - NDA filing planned in 2019

- Post-Bariatric Hypoglycemia (PBH) Program
    - Topline data from Phase 2 PREVENT Study with Avexitide
    - 28-days of treatment demonstrated clinically meaningful improvements
        - Reductions in rates of hypoglycemia and severe hypoglycemia
        - Reductions in rate of rescue
        - Well-tolerated with no significant safety concerns
    - Regulatory guidance planned in 2019

“We are delivering on our founding commitment to rapidly advance only the most promising rare disease programs, adding strategic and complementary programs to the pipeline in the process,” said David Cory, President and CEO of Eiger. “We are now advancing a late stage pipeline and making progress toward bringing multiple first-in-class therapies to patients with rare and ultra-rare unmet medical needs.”

A replay of the webcast is accessible here.

**About Hepatitis Delta Virus (HDV)**
Hepatitis Delta is caused by infection with HDV and is considered to be one of the most severe forms of viral hepatitis in humans. Hepatitis delta occurs only as a co-infection in individuals harboring Hepatitis B Virus (HBV). Hepatitis delta leads to more severe liver disease than HBV alone and is associated with accelerated liver fibrosis, liver cancer, and liver failure. Hepatitis delta is a disease with a significant impact on global health, which may affect up to approximately 15-20 million people worldwide. The prevalence of HDV varies among different parts of the world. Globally, HDV infection is reported to be present in approximately 4.3% to 5.7% of chronic Hepatitis B carriers. The prevalence of HDV in patients infected with chronic HBV is even higher in certain regions, including certain parts of Mongolia, China, Russia, Central Asia, Pakistan, Turkey, Africa, Middle East and South America, with an HDV prevalence as high as 60% being reported in HBV-infected patients in Mongolia and Pakistan.

**About Lonafarnib in Hepatitis Delta Infection (HDV)**
Lonafarnib is a well-characterized, late-stage, orally active inhibitor of farnesyl transferase, an enzyme involved in modification of proteins through a process called prenylation. HDV uses this host cell process inside liver cells to complete a key step in its life cycle. Lonafarnib inhibits the prenylation step of HDV replication inside liver cells and blocks the virus life cycle at the stage of assembly. Lonafarnib has been dosed in over 120 HDV-infected patients across international academic centers and is moving into Phase 3 development for HDV with a single, pivotal trial planned to initiate by the end of the year. Lonafarnib has been granted Orphan Drug Designation by the U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA), and Fast Track Designation by U.S. FDA. Lonafarnib is not approved for any indication, and is licensed from Merck Sharp & Dohme Corp. (known as MSD outside of the United States and Canada).

**About Progeria**
Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is a rare and rapidly fatal genetic condition of accelerated aging in children caused by a point mutation in the LMNA gene, encoding the lamin A protein, yielding the farnesylated aberrant protein, progerin. Lamin A protein is part of the structural scaffolding that holds

the nucleus together. Researchers now believe that progerin makes the nucleus unstable, and that cellular instability leads to the process of premature aging in Progeria. Children with Progeria die of the same heart disease that affects millions of normally aging adults (arteriosclerosis), but at an average age of 14.5 years. Disease manifestations include severe failure to thrive, scleroderma-like skin, global lipodystrophy, alopecia, joint contractures, skeletal dysplasia, global accelerated atherosclerosis with cardiovascular decline, and debilitating strokes. It is estimated that 400 children worldwide have Progeria.

**About Progeroid Laminopathies**

Progeroid laminopathies are genetic conditions of accelerated aging caused by a constellation of mutations in the lamin A and/or Zmpste24 genes yielding farnesylated proteins that are distinct from progerin. While non-progerin producing, these genetic mutations result in disease manifestations with phenotypes that have overlap with, but are distinct, from Progeria. Collectively, worldwide prevalence of progeroid laminopathies is likely greater than Progeria.

**About Lonafarnib in Progeria and Progeroid Laminopathies**

Lonafarnib is a well-characterized, late-stage, orally active inhibitor of farnesyltransferase, an enzyme involved in modification of proteins through a process called prenylation. Progerin is a farnesylated protein that cannot be cleaved, resulting in tight association with the nuclear envelope, which in turn results in changes in nuclear envelope morphology and subsequent cellular damage. Lonafarnib blocks the farnesylation of progerin and has been dosed in over 80 children with Progeria at Boston's Children Hospital in Phase 1/2 and Phase 2 studies. Lonafarnib has been granted Orphan Drug Designation for Progeria by the FDA and EMA. Lonafarnib is not approved for any indication, and is licensed by Eiger from Merck Sharp & Dohme Corp.

**About The Progeria Research Foundation**

The Progeria Research Foundation was established in 1999 by the family of Sam Berns, a child with Progeria. Within four years of its founding, the PRF Genetics Consortium, led by Francis Collins, MD, PhD, discovered the Progeria gene. PRF has also been the driving force behind studies to evaluate lonafarnib as a potential treatment for Progeria and supports scientists who conduct Progeria research. Today, PRF is the only non-profit organization in the world solely dedicated to finding treatments and the cure for Progeria and its age-related conditions, including heart disease. For more information, please visit www.progeriaresearch.org.

**About Post-Bariatric Hypoglycemia (PBH)**

Approximately 150,000-200,000 bariatric surgical procedures are performed each year

in the United States, and another 100,000 are performed each year in Europe. The estimated prevalence of PBH is approximately 30,000 in the United States and approximately 25,000 in the European Union. As the number of bariatric surgeries to treat obesity and related comorbidities has increased, so too has the number of individuals who experience PBH, with symptoms typically developing one or more years following surgery. PBH can occur with a range of severity in post-bariatric surgery patients. Mild to moderate hypoglycemia may be managed largely through dietary carbohydrate restriction, whereas severe hypoglycemia results in neuroglycopenic outcomes (altered mental status, loss of consciousness, seizures, coma) which are unresponsive to diet modification. Severe PBH can be debilitating with a significant negative impact on quality of life. There is no approved pharmacologic therapy.

**About Avexitide (formerly exendin 9-39)**

Avexitide is a 31-amino acid peptide that selectively targets and blocks GLP-1 receptors, normalizing insulin secretion by the pancreas, and thereby reducing postprandial hypoglycemia. Avexitide is being investigated as a novel treatment for PBH. Avexitide has been granted orphan designation in the European Union by the EMA for the treatment of non-insulinoma pancreatogenous hypoglycemia syndrome (NIPHS) and orphan designation in the United States by the FDA for the treatment of hyperinsulinemic hypoglycemia. Both of these broad designations include PBH. Avexitide has never been approved or commercialized for any indication. More information on avexitide clinical trials may be found at www.clinicaltrials.gov.

**About Eiger**

Eiger is a late-stage biopharmaceutical company focused on the accelerated development and commercialization of a pipeline of targeted therapies for rare and ultra-rare diseases. The company's lead program is in Phase 3, developing lonafarnib, a first-in-class prenylation inhibitor for the treatment of Hepatitis Delta Virus (HDV) infection. The company is also preparing an NDA with plans to file in 2019 for lonafarnib in the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies. For additional information about Eiger, please visit www.eigerbio.com.

**Note Regarding Forward-Looking Statements**

This press release contains "forward-looking" statements that involve substantial risks and uncertainties. All statements other than statements of historical facts, including statements regarding our future financial condition, timing for and outcomes of clinical results, business strategy and plans and objectives for future operations, are forward looking statements. These forward-looking statements include terminology such as "believe," "will," "may," "estimate," "continue," "anticipate," "contemplate," "intend,"

“target,” “project,” “should,” “plan,” “expect,” “predict,” “could,” “potentially” or the negative of these terms. Forward looking statements are our current statements regarding our intentions, beliefs, projections, outlook, analyses or current expectations concerning, among other things, our ongoing and planned clinical development timing expectations and whether larger studies will support the earlier study results identified, including whether the D-LIVR Phase 3 study as a single, pivotal study will be initiated by the end of 2018; whether the D-LIVR Phase 3 study results, if successful, will be sufficient to support registration; the timing of and our ability to initiate or enroll clinical trials, including whether our D-LIVR study can be initiated by the end of this year; our ability to complete and achieve successful clinical study results with any or all of our product candidates in order make timely regulatory filings and obtain and maintain regulatory approvals based on our expected timelines; our ability to move lonafarnib into potentially pivotal clinical studies and file an NDA for progeria in a successful and timely manner; 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.

Various important factors could cause actual results or events to differ materially from the forward-looking statements that Eiger makes, including the risks described in the “Risk Factors” sections in the Quarterly Report on Form 10-Q for the quarter ended September 30, 2018 and Eiger’s periodic reports filed with the SEC. Eiger does not assume any obligation to update any forward-looking statements, except as required by law.


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