



## **Eiger BioPharmaceuticals Announces FDA Acceptance of IND Application for Lonafarnib for the Treatment of Progeria and Progeroid Laminopathies**

**- Plans to File NDA in 2019**

PALO ALTO, Calif., Dec. 3, 2018 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare and ultra-rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has accepted the Investigational New Drug (IND) application for lonafarnib in the treatment of both Hutchinson-Gilford Progeria Syndrome (HGPS or progeria) and progeroid laminopathies. Eiger is collaborating with The Progeria Research Foundation and plans to submit a new drug application (NDA) to the FDA in 2019. There is no approved treatment for progeria or progeroid laminopathies. Lonafarnib is a first-in-class prenylation inhibitor in development for hepatitis delta virus (HDV) infection and also progeria and progeroid laminopathies.

"We had a positive pre-IND meeting with the Division of Gastroenterology and Inborn Errors Products at FDA in August and now plan to submit an NDA for lonafarnib in the treatment of progeria and progeroid laminopathies in 2019," said David Apelian, Chief Operating Officer and Executive Medical Officer of Eiger. "We are committed to bring the first approved therapy to market for patients suffering from these devastating disorders."

### **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 lamin A gene yielding the farnesylated aberrant protein, progerin. Lamin A protein is the structural scaffolding that holds the nucleus together. Researchers now believe that defective lamin A protein 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. It is estimated that in addition to the 400 children with Progeria, an additional 400 children worldwide have progeroid laminopathies.

### **About Lonafarnib**

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 multiple Phase 1/2 and Phase 2 studies. Lonafarnib has been granted Orphan Drug Designation for Progeria by the FDA. 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](http://www.progeriaresearch.org).

### **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](http://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, 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.

Investors: Ingrid Choong, PhD

Email: [ichoong@eigerbio.com](mailto:ichoong@eigerbio.com) / Phone: 1-650-619-6115

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