



## **Eiger Announces Expanded License Agreement with Merck for Investigational Candidate Lonafarnib and Collaboration with The Progeria Research Foundation (PRF)**

### **- Plans to Seek FDA Guidance for Potential Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) Indication**

PALO ALTO, Calif., May 16, 2018 /PRNewswire/ -- Eiger BioPharmaceuticals, Inc. (NASDAQ: EIGR), focused on the development and commercialization of targeted therapies for rare diseases, announced today that it has expanded its licensing agreement with Merck, known as MSD outside the United States and Canada, to include rights to develop the investigational farnesyltransferase inhibitor lonafarnib for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria), a rare and fatal genetic condition characterized by accelerated aging in children. The expanded agreement provides Eiger with commercial and distribution rights to lonafarnib across the licensed and approved indications in the future. Concurrently, Eiger announced that it has completed a collaboration agreement with The Progeria Research Foundation (PRF). Eiger, at its sole cost and expense, will provide lonafarnib for ongoing clinical trials and expanded access in Progeria and be responsible for any potential filing of an NDA for the Progeria indication based on PRF data. Eiger plans to seek FDA guidance regarding a potential regulatory approval for lonafarnib in Progeria.

Merck previously provided lonafarnib free of charge for clinical studies supported by PRF in Progeria. Following the transfer of manufacturing technology for lonafarnib from Merck to Eiger in 2015, Eiger continued to provide lonafarnib for investigational use, and a collaboration with PRF emerged. Eiger plans to seek guidance from the FDA regarding data already generated in multiple clinical studies conducted and completed by PRF evaluating lonafarnib in Progeria. Under the agreement with Merck, Eiger will be responsible for regulatory execution, commercialization and distribution activities of lonafarnib for Progeria. Eiger is also preparing to evaluate lonafarnib in a Phase 3 clinical trial for the treatment of hepatitis delta virus (HDV) infection.

"Continued patient access to lonafarnib was the fundamental motivation for these agreements," said David Cory, President and CEO of Eiger. "Eiger will provide lonafarnib for ongoing clinical trials and expanded access in Progeria and work together with PRF to seek regulatory guidance on a pathway toward regulatory approval of lonafarnib for use in children with Progeria."

"Our mission at PRF is to discover treatments and the cure for Progeria, and its aging-related disorders, including heart disease," said Leslie Gordon, MD, PhD, Medical Director and Co-Founder of The Progeria Research Foundation. "In a relatively short time, we have achieved extraordinary progress towards our mission including the Progeria gene discovery in 2003, the first clinical trial in Progeria initiated in 2007, and clinical evidence of a survival benefit for children administered lonafarnib. We are indebted to Merck for supplying lonafarnib free of charge to PRF-supported clinical trials, and for facilitating our new partnership with Eiger. We look forward to collaborating with Eiger as we pursue pathways for regulatory approval of lonafarnib in Progeria."

### **About Progeria**

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is a rare and 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. 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 globally ~400 children have Progeria.

### **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. Lamin A and progerin are both farnesylated. However, farnesylated progerin 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 U.S. Food and Drug Administration (FDA). Lonafarnib is not approved for any indication, and is licensed by Eiger from Merck Sharp & Dohme Corp. Merck will not receive any milestone payments for the development of lonafarnib for the treatment of Progeria, and has waived royalty obligations from Eiger for a specified quantity of lonafarnib, estimated to supply the worldwide population of children with Progeria on an annual basis.

### **About The Progeria Research Foundation (PRF)**

The Progeria Research Foundation (PRF) was established in 1999 by Leslie Gordon, MD, PhD and Scott Berns, MD, MPH, parents of a child with Progeria. In 2003, the PRF Genetics Consortium, led by Francis Collins, MD, PhD, isolated the Progeria gene. PRF has also been the driving force behind studies to evaluate lonafarnib as a potential treatment for Progeria and support scientists who conduct Progeria research. Today, PRF is the only non-profit organization solely dedicated to finding treatments and a cure for Progeria. For more information, please visit [www.progeriaresearch.org](http://www.progeriaresearch.org).

### **About Eiger**

Eiger is a clinical-stage biopharmaceutical company focused on the development and commercialization of targeted therapies for rare diseases. We are committed to translational innovation and the development of well-characterized drugs acting on newly identified or novel targets. Our mission is to systematically reduce the time and cost of the drug development process to more rapidly deliver important medicines to patients with rare diseases. Our lead program evaluating lonafarnib in Hepatitis Delta Virus (HDV) infection is moving into Phase 3 with a single, pivotal trial planned to initiate by the end of the year. For additional information about Eiger and its clinical programs, 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 Eiger would be permitted to file an NDA based on PRF data and the timing and outcome of any FDA meeting with respect to lonafarnib and Progeria, 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; our ability to make timely regulatory filings and obtain and maintain regulatory approvals for lonafarnib as a single agent or in combination, ubenimex, PEG IFN lambda, exendin 9-39 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.

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 March 31, 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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