Eiger Announces Breakthrough Therapy Designation Granted by FDA for Lonafarnib in Progeria and Progeroid Laminopathies

- **Survival Benefit in Children with Progeria Published in JAMA 2018**
- **NDA Filing Planned in 2019**

PALO ALTO, Calif., December 19, 2018 — Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare and ultra-rare diseases, today announced that the Food and Drug Administration (FDA) has granted Breakthrough Therapy designation for lonafarnib for the treatment of Hutchinson-Gilford progeria syndrome (HGPS or Progeria) and progeroid laminopathies. FDA Breakthrough Therapy designation is designed to expedite the development and review of medicines intended to treat serious or life-threatening diseases. Progeria and progeroid laminopathies are ultra-rare, genetic conditions characterized by accelerated aging, with no approved treatment. Eiger is collaborating with The Progeria Research Foundation and plans to submit a new drug application (NDA) to the FDA in 2019. Lonafarnib is a first-in-class prenylation inhibitor for the treatment for Progeria and progeroid laminopathies.

“There is an urgent unmet medical need for an approved treatment for children with Progeria as well as those with progeroid laminopathies,” said Leslie Gordon, MD, PhD, Medical Director of The Progeria Research Foundation. “Lonafarnib has demonstrated a survival benefit in Progeria and truly represents a breakthrough therapy for these children. We look forward to continued collaboration with Eiger toward NDA filing and ensuring access to this treatment to patients worldwide.”

“Eiger is advancing only the most promising, targeted therapies for rare diseases,” said Lisa Porter, MD, Chief Medical Officer of Metabolic Diseases at Eiger. “Progeria is an ultra-rare, fatal, pediatric disease with no approved treatment. We are very pleased with FDA Breakthrough Therapy designation for lonafarnib in Progeria and progeroid laminopathies as we prepare for NDA filing in 2019.”

This Breakthrough Therapy designation is supported by data from clinical studies of lonafarnib, demonstrating statistically significant improvements in survival (77% reduction in the risk of mortality for patients treated with lonafarnib monotherapy relative to matched untreated controls) in children with Progeria where the average survival age without treatment is 14.5 years.

About Progeria

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is a rare and rapidly fatal genetic condition of accelerated aging in children. Progeria is 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 may make the nucleus unstable, and that cellular instability may lead 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 researchers believe cannot be cleaved, resulting in tight association with the nuclear envelope, which is believed to lead to 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 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 we can timely file in 2019 our planned NDA for Progeria and Progeroid Laminopathies; whether our filing package will be sufficient to support regulatory approval in either or both of Progeria and Progeroid Laminopathies; 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, including our D-LIVR Phase 3 study of lonafarnib for the treatment of HDV; 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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