Eiger BioPharmaceuticals Receives FDA Rare Pediatric Disease (RPD) Designation for Lonafarnib for the Treatment of Progeria and Progeroid Laminopathies and Plans NDA Filing in 2019

- Designation Enables Priority Review Voucher Eligibility Upon NDA Approval

PALO ALTO, Calif., October 22, 2018 — Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for rare diseases, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease (RPD) designation to lonafarnib in the treatment of both Hutchinson-Gilford Progeria Syndrome (HGPS or progeria) and progeroid laminopathies. RPD designation for progeria and progeroid laminopathies enables priority review voucher (PRV) eligibility upon FDA approval of a rare pediatric disease product application for lonafarnib for these ultra-rare and fatal genetic conditions characterized by accelerated aging in children. Eiger is collaborating with The Progeria Research Foundation in this lonafarnib program and plans to submit a new drug application (NDA) to the FDA in 2019. There is no approved treatment for progeria or progeroid laminopathies.

The Priority Review Voucher Program is focused on encouraging development of therapies to prevent and treat rare pediatric diseases. If an NDA for lonafarnib is approved by the FDA for progeria and progeroid laminopathies, the RPD designation qualifies Eiger, as the NDA sponsor, for a PRV upon marketing approval. The voucher, which can be sold or transferred to another entity, can be used by the holder to receive priority review for a future NDA or BLA submission, which reduces FDA's target submission review time from ten months to six months. Eiger would share 50% of the proceeds from monetization of a Priority Review Voucher with PRF to support future progeria research.

"We are encouraged by the agency's support for lonafarnib and the opportunities provided by the Rare Pediatric Disease designation and the Priority Review Voucher Program," said David Cory, President and CEO of Eiger. "We plan to submit an NDA for lonafarnib in the treatment of progeria and progeroid laminopathies in 2019. In the meantime, we are committed to ensuring access to lonafarnib for all patients with these disorders, and plan to launch a global expanded access program by end of year."

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.

About Eiger

Eiger is a clinical-stage biopharmaceutical company focused on the accelerated

development and commercialization of targeted therapies for rare and ultra-rare diseases. We innovate by developing well-characterized drugs acting on newly identified or novel targets in rare diseases. Our mission is to systematically reduce the time and cost of the drug development process to more rapidly deliver important medicines to patients. Lonafarnib is our lead compound and is expected to advance into Phase 3 with a single, pivotal trial to treat hepatitis delta virus (HDV) to initiate by the end of the year. Lonafarnib is also advancing toward a potential NDA for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria), expected to be submitted in 2019. For additional information about Eiger and its clinical programs, 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, including whether lonafarnib receives approval for the treatment of progeria and progeroid laminopathies and the timing of an NDA submission; the timing of and our ability to initiate or enroll clinical trials; the timing for completion and potential filing for registration for our clinical candidates; our ability to make timely regulatory filings and obtain and maintain regulatory approvals for lonafarnib as a single agent or in combination and our other product candidates; 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 June 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.

SOURCE Eiger BioPharmaceuticals, Inc.

Investors: Ingrid Choong, PhD
Email: ichoong@eigerbio.com
Phone: 1-650-619-6115