Eiger BioPharmaceuticals Announces EMA Validation of Lonafarnib MAA for Treatment of Progeria and Progeroid Laminopathies

- **Accelerated Assessment of MAA Previously Granted by EMA in March**

PALO ALTO, Calif. April 27, 2020 -- Eiger BioPharmaceuticals, Inc. (Nasdaq:EIGR), focused on the development and commercialization of targeted therapies for serious rare and ultra-rare diseases, today announced that the European Medicines Agency (EMA) has completed its validation of the Marketing Authorization Application (MAA) for lonafarnib to treat Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies. Validation of the MAA confirms that the submission is accepted and starts the formal review process by the EMA's Committee for Human Medicinal Products (CHMP). The EMA previously granted an accelerated assessment of the MAA.

Lonafarnib is a well-characterized, first-in-class, oral inhibitor of farnesyltransferase. A study published in *JAMA* 2018 demonstrated an 88% reduction in the risk of mortality in patients with Progeria treated with lonafarnib monotherapy. The most commonly reported adverse events are gastrointestinal in nature. Many patients with Progeria have received continuous lonafarnib therapy for greater than 10 years. There is currently no approved therapy for Progeria or Progeroid Laminopathies.

"Validation of the MAA combined with accelerated assessment from the EMA represents another major step toward the anticipated approval of lonafarnib in Progeria and Progeroid Laminopathies," said David Cory, President and CEO of Eiger. "We look forward to working with the Progeria community, including The Progeria Research Foundation (PRF) and Progeria Family Circle (PFC), to bring the first approved treatment to children and young adults with Progeria and Progeroid Laminopathies."

About Progeria

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is an ultra-rare and fatal genetic condition of accelerated aging in children. Progeria is caused by a point mutation in the *LMNA* gene, which encodes the lamin A protein, yielding the farnesylated aberrant protein, progerin. Lamin A protein is part of the structural scaffolding that holds the nucleus together. 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 similar to 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. Progerin is a farnesylated, aberrant protein that researchers believe cannot be cleaved, resulting in tight association with the nuclear envelope, which leads to changes in nuclear envelope morphology and subsequent cellular damage.

Lonafarnib blocks the farnesylation of progerin and has been dosed in over 90 children with Progeria at Boston Children's Hospital in Phase 1/2 and Phase 2 studies. In patients with HGPS, lonafarnib monotherapy was associated with a lower mortality rate after 2.2 years of follow-up compared with no treatment (3.7% vs 33.3%, respectively) with a hazard ratio of 0.12 or a reduction in risk of mortality of 88% (*JAMA* 2018).

Lonafarnib has been granted Orphan Drug Designation for Progeria by the FDA and EMA and Breakthrough Therapy Designation and Rare Pediatric Disease Designation 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 (PRF) was established in 1999 by the family of Sam Berns, a child with Progeria. Within four years of its founding, the PRF Genetics Consortium, in collaboration with Francis Collins, MD, PhD, discovered the Progeria gene. PRF has funded and co-coordinated all lonafarnib-associated clinical trials for Progeria and Progeroid Laminopathies and supports scientists who conduct Progeria research worldwide. PRF is the only non-profit organization solely dedicated to finding treatments and the cure for Progeria and its age-related conditions, including heart disease. PRF's International Patient Registry includes over 300 children with Progeria in more than 65 countries. For more information, please visit www.progeriaresearch.org.

About Progeria Family Circle
The Progeria Family Circle (PFC) is a European patient network, founded in 1997, supporting children and young adults with Progeria and their families on a European level. The PFC consists of a network of parents and experts in Europe, visiting medical

congresses to keep informed about research developments in Progeria. The PFC goal is to support better and faster recognition of symptoms of Progeria, raise awareness for a better acceptance of Progeria children and young adults in public communities, provide opportunities for families to meet each other through many annual reunions throughout Europe, and offer other necessary and permanent support for families with children with Progeria. For more information, please visit www.progeriafamilycircle.blogspot.com.

About Eiger

Eiger is a late-stage biopharmaceutical company focused on the development and commercialization of first-in-class, well-characterized drugs for serious rare and ultra-rare diseases for patients with high unmet medical needs, for which no approved therapies exist.

Eiger has completed an NDA and MAA submission for lonafarnib for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies. Eiger has also established a global Managed Access Program, expected to span greater than 40 countries, to ensure all children and young adults with Progeria and Progeroid Laminopathies have access to treatment.

The company's lead program is in Phase 3, developing lonafarnib, a first-in-class oral prenylation inhibitor for the treatment of Hepatitis Delta Virus (HDV) infection. The company is also advancing peginterferon lambda, a first-in-class interferon, toward registration for the treatment of HDV. 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, the timing of our ongoing and planned clinical development, including the potential for approval of our lonafarnib product candidate in the US and EU for Progeria and Progeroid Laminopathies; our progression and enrollment of our Phase 3 D-LIVR study in HDV; our plans to advance peginterferon lambda in HDV in the US and EU; our ability to transition into a commercial stage biopharmaceutical company; our ability to finance the continued advancement of our

development pipeline products; that the company's expectations regarding the effects of COVID-19 on the Company's trials and development may be incorrect, and the potential for success of any of our product candidates.

These statements concern product candidates that have not yet been approved for marketing by the U.S. Food and Drug Administration (FDA). No representation is made as to their safety or effectiveness for the purposes for which they are being investigated.

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 Annual Report on Form 10-K for the year ended December 31, 2019 and Eiger's subsequent filings with the SEC. Eiger does not assume any obligation to update any forward-looking statements, except as required by law.

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