

Eiger BioPharmaceuticals Announces FDA Acceptance of NDA for Filing with Priority Review of Zokinvy™ (lonafarnib) for Treatment of Progeria and Progeroid Laminopathies

PALO ALTO, Calif. May 19, 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 U.S. Food and Drug Administration (FDA) accepted the New Drug Application (NDA) for accelerated review of Zokinvy™ (lonafarnib) for treatment of Progeria and Progeroid Laminopathies. The FDA granted Priority Review with a Prescription Drug User Fee Act (PDUFA) target action date of November 20, 2020. The FDA is not currently planning to hold an advisory committee meeting to discuss this application. Zokinvy for treatment of Progeria and Progeroid Laminopathies has been granted Rare Pediatric Disease Designation.

“The acceptance of our first NDA is a significant milestone for Eiger, and an important step toward bringing a treatment to children and young adults with Progeria and Progeroid Laminopathies,” said David Cory, President and CEO of Eiger. “We would like to thank The Progeria Research Foundation (PRF) for their commitment, persistence and dedication. Most importantly, we are grateful to all the children with Progeria and their families who have made this possible by participating in the lonafarnib clinical trials. We are preparing for the commercial launch of Zokinvy in the U.S and Europe.”

The Zokinvy NDA includes data from a study published by Gordon et al in *Journal of the American Medical Association (JAMA)* 2018 which demonstrated a survival benefit with 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.

“This milestone is the culmination of twelve years of clinical trials, treating children from over 30 countries and six continents with Progeria and Progeroid Laminopathies,” stated Leslie Gordon, MD, PhD, PRF Medical Director. “We are fortunate to have Eiger as a partner for the preparation and filing of the NDA and providing continuous Zokinvy (lonafarnib) drug supply to children and young adults with Progeria. We are grateful to all the children with Progeria and their families. Their courage inspires all of us, every day.”

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. Without lonafarnib therapy, 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 Zokinvy (lonafarnib)

Zokinvy 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.

Zokinvy 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, Zokinvy 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% (Gordon et al, *JAMA* 2018).

Zokinvy 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. Zokinvy (lonafarnib) is not approved for any indication, and is licensed by Eisai from Merck Sharp & Dohme Corp.

FDA has conditionally accepted the proposed brand name Zokinvy™ for lonafarnib for treatment of Progeria and Progeroid Laminopathies. Final approval of the Zokinvy brand name is conditional on FDA approval of the product candidate, lonafarnib.

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, conducted at Boston Children's Hospital, 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 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.

The NDA and MAA submissions for Zokinvy for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies have been accepted for filing. 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 Zokinvy product candidate in

the U.S. and E.U. 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 U.S. and E.U.; 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 Quarterly Report on Form 10-Q for the quarter ended March 31, 2020 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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