

Eiger Announces Positive Meeting with FDA on Progeria Program

- Company advancing plans to file NDA for Lonafarnib in Progeria

PALO ALTO, Calif., September 4, 2018 – Today, Eiger BioPharmaceuticals, Inc. (NASDAQ: EIGR), a company focused on the development and commercialization of targeted therapies for rare diseases, announced it has received minutes from a pre-investigational new drug (pre-IND) meeting with the Division of Gastroenterology and Inborn Errors Products of the U.S. Food and Drug Administration (FDA) for lonafarnib in the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria). Eiger plans to submit a new drug application (NDA) in 2019. There is no approved treatment for Progeria, an ultra-rare and fatal genetic condition characterized by accelerated aging in children.

Eiger and the FDA engaged in a collaborative discussion regarding the analysis methodology for the survival data that was published in April 2018 [*Journal of the American Medical Association \(JAMA\)*](#) as potential support for submission of an NDA filing. This clinical study, which compared children with Progeria who received lonafarnib monotherapy with matched untreated children with Progeria, reported a primary outcome of mortality. The study found that children taking lonafarnib monotherapy (n=63) experienced a 77 percent reduction in the risk of mortality compared to a natural history, matched-control cohort of untreated children (n=63) after two years of study. Based on this meeting with the FDA, Eiger does not anticipate conducting additional clinical efficacy studies to complete the filing for registration.

Clinical studies with lonafarnib in Progeria were funded by The Progeria Research Foundation. Eiger has supplied lonafarnib for investigational use in Progeria clinical studies since 2015, and is now responsible for development, regulatory, commercialization and distribution activities for lonafarnib in Progeria.

“Eiger is preparing a regulatory submission for lonafarnib in Progeria in the U.S. with plans to file the NDA in 2019 and anticipates scientific advice from European Medicines Agency by end of 2018,” said David Cory, President and CEO of Eiger. “We are committed to ensuring access to lonafarnib for all children with Progeria, 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 350 children worldwide 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. 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 (PRF)

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 development and commercialization of targeted therapies for 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 with rare diseases. We plan to initiate D-LIVR, a Phase 3 study to investigate ritonavir-boosted lonafarnib in a single, pivotal trial in hepatitis delta virus-infected patients by end of 2018. We are preparing an NDA for lonafarnib in the treatment of Progeria 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, whether the FDA minutes confirm the understanding that existing data will support an NDA filing for lonafarnib in Progeria; our ability to meet the quality and documentation requirements for potential approval of an NDA; and the timing for filing of an NDA; our ongoing and planned clinical development, including whether 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; whether PREVENT Phase 2 study results will support further development of avexitide; 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, avexitide 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 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

Phone: 1-650-619-6115

Email: ichoong@eigerbio.com