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First-ever treatment for rare, rapid-aging disease Progeria submitted in New Drug Application to the FDA

PEABODY, MA — December 17, 2019 – The Progeria Research Foundation (PRF) today celebrates a milestone with the initiation of a rolling submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) by Eiger BioPharmaceuticals, Inc., seeking approval of lonafarnib, the first-ever treatment for Progeria and Progeroid Laminopathies. Lonafarnib, a farnesyltransferase inhibitor (FTI), has demonstrated extended survival in children and young adults with Hutchinson-Gilford Progeria Syndrome (HGPS), or Progeria, an ultra-rare and fatal disease that causes premature aging in children. Without treatment, children with Progeria die of heart disease at an average age of 14.5 years.

Progeria is caused by a random genetic mutation that produces an overabundance of the progerin protein. Approximately 400 children worldwide have Progeria. In normal human aging, the accumulation of some progerin within a cell is common, but in Progeria, the rate of accumulation is highly accelerated, resulting in progressive cellular damage and atherosclerotic heart disease.

Lonafarnib inhibits farnesyltransferase, an enzyme that facilitates progerin production. The FTI is thought to prevent the mutant protein from absorbing into the cellular wall where it causes much of its damage, reversing instability of the nuclear structure.

Since 2007, PRF has funded and led four clinical trials to study lonafarnib's effect on Progeria, treating children from over 30 countries. Researchers from Boston Children's Hospital and Brown University have tracked more than 250 children from six continents, demonstrating a link between lonafarnib treatment and extended survival. Results from a 2018 study published in *The Journal of the American Medical Association (JAMA)*, demonstrated that treatment with lonafarnib alone compared with no treatment was associated with a significantly lower mortality rate (3.7% vs. 33.3%) after a median of 2.2 years of follow up.

"It is incredibly exciting to see twelve years of clinical data translate into the initiation of this NDA submission," said PRF Medical Director Leslie Gordon, MD, PhD. "Since day one of The Progeria Research Foundation, we have been working toward treatments and the cure for this rare, fatal condition. This submission—the first for the treatment of Progeria—is a big milestone in that mission. A big thank you to all of the children with Progeria and their families that have made this possible by participating in PRF's research programs."

A rolling NDA for the lonafarnib submission allows completed portions to be submitted and reviewed by the FDA on an ongoing basis. Eiger plans to complete the submission in the first quarter of 2020.

“Throughout our 20 year history, we’ve made tremendous progress in our understanding and treatment of Progeria, leading us to this historic achievement,” said Audrey Gordon, President and Executive Director of the Progeria Research Foundation. “PRF is grateful to the families and children with Progeria who have participated—some traveling thousands of miles to Boston—in our clinical trials, to the clinical investigators at Boston Children’s Hospital and Brown University, and to Eiger. We have worked tirelessly to make a meaningful impact on the lives of children and young adults with Progeria, and this submission brings us closer to that goal.”

About The Progeria Research Foundation

The Progeria Research Foundation (PRF) was founded in 1999 by the family of Sam Berns with an original mission of discovering the cause, treatments and cure for Progeria. In 2003, the PRF Genetics Consortium discovered the Progeria gene, a collaboration led by Dr. Francis Collins, who is currently Director of the National Institutes of Health (NIH). Today, PRF continues to be the only organization in the world solely dedicated to finding treatments and the cure for Progeria and its aging-related conditions, including heart disease. The organization fills a void, taking these children out of the background where they had been for more than 100 years and putting them and Progeria at the forefront of scientific efforts.

For more information and to support PRF’s mission, please visit www.progeriaresearch.org.

About Eiger

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

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 advancing peginterferon lambda, a first-in-class interferon, toward registration for the treatment of HDV. Eiger has initiated a rolling NDA submission for lonafarnib to treat Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and Progeroid Laminopathies with plans to complete NDA submission followed by an MAA submission in the first quarter of 2020.

For additional information about Eiger and its clinical programs, please visit www.eigerbio.com.