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## **FDA Submission Completed for First-Ever Treatment for Rapid-Aging Disease Progeria**

**PEABODY, MA — March 23, 2020** – The Progeria Research Foundation (PRF) today announced the completed submission of a New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) by Eiger BioPharmaceuticals, Inc., seeking approval of lonafarnib for the treatment of Progeria and Progeroid Laminopathies. Lonafarnib, a farnesyltransferase inhibitor (FTI), would be the first-ever approved treatment for these diseases.

Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) is an ultra-rare and fatal disease that causes premature aging in children. Without lonafarnib therapy, children die of heart disease at an average age of 14.5 years. In 2018, a study based on information from the PRF International Patient Registry and PRF-funded clinical trials at Boston Children’s Hospital demonstrated extended survival with lonafarnib therapy. The 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.

Progeria is caused by a random genetic mutation that produces an overabundance of the progerin protein. Approximately 400 children worldwide have Progeria. The accumulation of progerin occurs with aging in the general population, in the cardiovascular system and elsewhere in the body such as the skin. In children with Progeria, the rate of accumulation is highly accelerated, resulting in progressive cellular damage and atherosclerotic heart disease.

“Approval of lonafarnib by the FDA as our first-ever treatment for Progeria would be absolutely tremendous,” said Audrey Gordon, President and Executive Director of The Progeria Research Foundation. “Being able to access lonafarnib by prescription, instead of through a clinical trial, will be a major achievement. This represents a huge step forward in our goal of treating and curing these beautiful children and young adults with Progeria.”

“This milestone is the culmination of twelve years of clinical data, throughout four clinical trials, treating children from over 30 countries and six continents,” said PRF Medical Director Leslie Gordon, MD, PhD. “The amount of hard work and dedication from our steadfast teams of researchers and staff that has gone toward furthering our mission to help these children has been incredible. This team of world experts on Progeria span multiple collaborative institutions, from Boston Children’s Hospital, Hasbro Children’s Hospital, Brigham and Women’s Hospital, Brown University, Boston University, and the National Institutes of Health. Most importantly, we are immensely grateful to all of the children with Progeria and their families, who’ve made this progress possible by participating in PRF’s research programs. Their courage inspires all of us, every day.”

## **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](http://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 ultrarare 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](http://www.eigerbio.com).