



First-Ever Treatment for Rare Rapid-Aging Disease Progeria Receives U.S. FDA Approval

The Progeria Research Foundation and Eiger BioPharmaceuticals Celebrate Historic Milestone with FDA Approval of Zokinvy™ (lonafarnib) for the Treatment of Progeria and Processing-Deficient Progeroid Laminopathies

PEABODY, MA — November 20, 2020 – The Progeria Research Foundation (PRF) today announced a historic milestone with the U.S. Food and Drug Administration (FDA) approval of Zokinvy™ (lonafarnib), for the treatment of Progeria and processing-deficient Progeroid Laminopathies (PL). Progeria is an ultra-rare, fatal pediatric rapid-aging disease. PRF, a pioneer in the rare disease research foundation space, has led Zokinvy clinical trial research since 2007.

Zokinvy is a farnesyltransferase inhibitor (FTI) that has shown survival benefit in children with Progeria. Data based on information from the PRF International Patient Registry and clinical trials co-coordinated by PRF and Boston Children's Hospital demonstrated that in patients with Progeria, Zokinvy reduced the incidence of mortality by 60% ($p=0.0064$) and increased average survival time by 2.5 years. Without Zokinvy treatment, children with Progeria die of heart disease at an average age of 14.5 years. Eiger BioPharmaceuticals (Eiger) began supplying Zokinvy for the Progeria clinical trials in 2015, and entered into a pioneering partnership with PRF in 2018 with the objective of leading Zokinvy through the FDA approval process.

“Today, we have achieved one important piece of PRF’s mission—the first-ever approved treatment for these beautiful children,” said Audrey Gordon, President and Executive Director of The Progeria Research Foundation. “Progeria is now one of the few rare diseases with an FDA-approved treatment. This momentous event is here, thanks in large part to PRF’s key partnerships, including the expert Progeria research teams from Boston Children’s Hospital, Hasbro Children’s Hospital, Brigham and Women’s Hospital, Brown University, Boston University, and the National Institutes of Health. Pharmaceutical partners that supplied lonafarnib free of charge to PRF-supported clinical trials were crucial as well, including Schering-Plough, Merck, and Eiger.”

Since its founding in 1999 by the family of Sam Berns after his diagnosis at age 2 years, PRF has made tremendous strides toward its mission to discover the cause, treatments and cure for Progeria. In partnership with the National Institutes of Health (NIH), PRF was the driving force behind the 2003 Progeria gene discovery; PRF’s core patient-focused programs include the Progeria International Patient Registry, Medical & Research Database, Cell & Tissue Bank, and Diagnostics Testing Program. In addition, PRF holds bi-annual scientific workshops, funds research grants and clinical drug trials,

and provides guidance for families in the Progeria community and their caretakers with its Clinical Care Handbook.

“Shortly after our son Sam was diagnosed with Progeria, my family and I founded The Progeria Research Foundation to find the cause, treatments, and cure for all children with this fatal disease. This first approved medication is a truly extraordinary milestone for the Progeria community as we forge ahead toward the cure,” said Leslie Gordon, MD, PhD, PRF Medical Director, and Zokinvy Clinical Trial Investigator. “We are thrilled to have Eiger as a partner in bringing Zokinvy to the approval finish line, and for their commitment to ensuring patients continuous access to Zokinvy. Most of all, the undaunted spirit and bravery of the children and their families have made all of this possible.”

“A therapy has been long overdue for this community,” said David Cory, President and CEO of Eiger. “We are very proud that the first drug approval at Eiger confers a survival benefit to patients with one of the most ultra-rare, and ultimately fatal, pediatric diseases. We are deeply appreciative of the commitment and scientific excellence from our partners at The Progeria Research Foundation (PRF) and Boston Children’s Hospital, and we are extremely grateful to all the children and young adults with Progeria and their families who have made this possible by participating in the Zokinvy clinical trials.”

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), and PL are ultra-rare, multisystemic, premature aging diseases that accelerate mortality in young patients due to accumulation of cellular progerin in HGPS or an abnormal lamin A protein in PL. Progeria is caused by a genetic mutation in the LMNA (“lamin A”) gene, and results in a disease-causing abnormal protein called *progerin*. There are approximately 400 children worldwide with Progeria. Thanks to PRF-funded research, we now know that progerin is produced in all of us as we age, but at a much lower rate than in children with Progeria. Due to this discovery of the biological connection between Progeria, heart disease and aging, finding the cure for one of the rarest diseases on earth could provide keys for treating millions of adults with heart disease and stroke associated with the natural aging process, as well as help the entire aging population.

“Today we celebrate the incredible milestone of an approved treatment for Progeria,” said Scott D. Berns, MD, MPH, FAAP, Co-Founder and Chairman of the Board, The Progeria Research Foundation. “And tomorrow we will press on until we have achieved our vision of a world in which every child with Progeria is cured.”

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 discovered the Progeria gene, a collaboration led by Dr. Francis Collins, who is currently Director of the National Institutes of Health (NIH). PRF has funded and

co-coordinated all Zokinvy-associated clinical trials for Progeria and Progeroid Laminopathies, conducted at Boston Children's Hospital, and supports scientists who conduct Progeria research worldwide. PRF's International Patient Registry includes over 300 children with Progeria in more than 65 countries.

PRF is the only non-profit organization solely dedicated to finding treatments and the cure for Progeria and its aging-related conditions, including heart disease. The organization fills a void, putting these children 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 commercial-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.

Zokinvy for the treatment of Hutchinson-Gilford Progeria Syndrome (HGPS or Progeria) and processing-deficient Progeroid Laminopathies is the Company's first FDA approval. A Marketing Authorization Application (MAA) has been accepted for filing and is under review by the EMA. Outside the U.S., Eiger's established global Managed Access Program, expected to span greater than 40 countries, ensures all children and young adults with Progeria and Progeroid Laminopathies have access to treatment.

Eiger's lead clinical programs target Hepatitis Delta Virus (HDV) infection, the most serious form of human viral hepatitis. Eiger is developing two complementary treatments for HDV. Lonafarnib is a first-in-class, oral prenylation inhibitor in a global Phase 3 trial. Peginterferon lambda is a first-in-class, well-tolerated type III interferon entering Phase 3.

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

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