



## **First-Ever Treatment for Ultra-Rare Rapid-Aging Disease Progeria Receives Approval in Japan**

The Progeria Research Foundation Marks Significant Achievement as Zokinvy™ (lonafarnib) Receives Japanese Approval for the Treatment of Progeria and Processing-Deficient Progeroid Laminopathies

**PEABODY, MA — January 20, 2024** – The Progeria Research Foundation (PRF) today announced a new milestone with the approval of Zokinvy™ (lonafarnib) by the Japanese Ministry of Health, Labor and Welfare (MHLW), 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. Eiger BioPharmaceuticals (Eiger) and PRF began a partnership in 2018 to bring this drug to market. Approval from the U.S. Food and Drug Administration (FDA) for Zokinvy was granted in November, 2020 and the European Medicines Administration (EMA) in July, 2022.

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 (BCH) demonstrated that in patients with Progeria, Zokinvy reduced the incidence of mortality by 72% and increased average survival time by an average of 30% (4.3 years). Without Zokinvy treatment, children with Progeria die of heart disease at an average age of 14.5 years. Prevalence data from PRF’s International Patient Registry indicate an expected 6 children with Progeria are living in Japan today.

“We’re thrilled by this wonderful news,” said Audrey Gordon, President and Executive Director of The Progeria Research Foundation. “On the heels of the 2020 FDA approval and the 2022 EMA approval for Zokinvy, now children and young adults with Progeria and PL in Japan will be able to access this life-extending, heart-strengthening treatment through the convenience of a prescription.”

“Since co-founding The Progeria Research Foundation in 1999, we have poured our hearts and souls into the most promising research toward treatments and the cure for every child with Progeria,” said Leslie Gordon, MD, PhD, PRF Medical Director and Zokinvy Clinical Trial Investigator. “After more than a decade of Zokinvy research funded by PRF and co-conducted by PRF and BCH, Zokinvy’s authorization in Japan is a win for the worldwide Progeria community. Thank you to the courageous children, their families, and our many expert Progeria research teams and pharmaceutical partners who made this key milestone possible.”

Over the course of its 24-year existence, 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 holds bi-annual scientific workshops, funds research grants breaking new ground in areas such as RNA therapeutics and Genetic Base Editing, and funds clinical drug trials. PRF's core patient-focused programs include the Progeria International Patient Registry, Medical & Research Database, Cell & Tissue Bank, Diagnostics Testing Program, and a Clinical Care Handbook for families and physicians.

"After conducting 16 years of Progeria clinical trials at Boston Children's Hospital, this milestone demonstrates the positive impact of a dedicated research team, rigorous testing, and the steadfast bravery of the wonderful children and their families," said Dr. Monica Kleinman, Principal Investigator for the Progeria clinical trials at Boston Children's Hospital. "Being a part of this achievement for these children is not only professionally rewarding but also a testament to the transformative potential of scientific advancement in Progeria research."

## **About Progeria**

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 400-450 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.

## **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, Acting Science Advisor to the President of the United States and former 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 370 children with Progeria in 70 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](http://www.progeriaresearch.org).

**Media Contact:**

Eleanor Maillie

[emaillie@progeriaresearch.org](mailto:emaillie@progeriaresearch.org)

P: (978) 879-9244