Proceeds from Priority Review Voucher Sale Will Fund Search for Cure for the Rapid Aging Disease Progeria

PEABODY, MA — November 23, 2020 – The Progeria Research Foundation (PRF) today announced that it will receive half of the net proceeds of the sale of a Priority Review Voucher (PRV). Earlier today, Eiger BioPharmaceuticals, Inc. (Eiger) announced that it has entered into a definitive agreement to sell its PRV for a lump sum payment of $95 million. PRF will receive 50 percent of the net proceeds, delivered under the terms of a Collaboration and Supply Agreement with Eiger.

The PRV was granted in conjunction with the recent approval by the U.S. Food and Drug Administration of Zokinvy™ (lonafarnib) as the first-ever approved treatment for Progeria and processing-deficient progeroid laminopathies (PL). Eiger's transaction remains subject to customary closing conditions, including anti-trust review.

Without Zokinvy, children with Progeria live an average of 14.5 years. Zokinvy is a farnesyltransferase inhibitor (FTI) that has shown survival benefit in children with Progeria. Data from the PRF International Patient Registry and PRF-funded clinical trials at 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.

According to a 2019 study published in The Orphanet Journal of Rare Diseases, average out-of-pocket clinical costs per approved orphan drug totaled $166 million. In addition to funding ongoing clinical development, PRF continues to fund crucial basic science grants to research laboratories worldwide and has provided millions to stimulate Progeria research around the world since its inception in 1999.

"Rare disease research organizations face inherent challenges because the market for any approved drug will always be a small one," said Audrey Gordon, President and Executive Director of PRF. "These funds will be used to accelerate the next generation of transformative treatments, and to pursue new groundbreaking opportunities to treat and cure children with Progeria worldwide."

"Since our founding in 1999, we've built a close-knit community with these children and their families. Now, with the continued involvement of our supporters and this tremendous resource, we can take full advantage of the current and future advances in science that will boldly accelerate our mission toward the cure for Progeria and its aging related diseases, including heart disease," said PRF Board Chair Scott D. Berns, MD, MPH, FAAP.

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is an ultra-rare, multisystemic, premature aging disease that accelerates mortality in young patients due to premature atherosclerosis, causing heart attacks and strokes.

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.

In addition to its pioneering achievements in research for this rare disease, PRF has achieved a coveted 4-star rating from Charity Navigator for the seventh year in a row, putting the organization in the top seven percent of reporting charities.

To learn more about research funded by The Progeria Research Foundation, please visit https://www.progeriaresearch.org/science-and-research/.

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'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.

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.

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