QUICK FACTS as of August 10, 2016

MISSION: To Discover Treatments and the Cure for Hutchinson-Gilford Progeria Syndrome and its Aging-related Disorders, Including Heart Disease

PRF BY THE NUMBERS:

- Identified children living with Progeria: 134* in 45 countries
- PRF-sponsored Progeria Clinical Drug Trials: 3
- Grants funded: 62, totaling $6.7 million
- Cell lines in the PRF Cell & Tissue Bank: 211
- Children in PRF's Medical & Research Database: 149
- International Scientific Meetings on Progeria: 11
- Number of languages into which PRF's program and medical care materials are translated: 31
- Increase in scientific publications since the Progeria gene discovery: 2,200%

*Includes 29 children in the Progeroid Laminopathy category who have a mutation in the Lamin pathway, but do not produce progerin.

Total Dollars Raised

1999 through July 31, 2016: **$22,709,071**

More than 80% of PRF's annual expenses are consistently dedicated to its programs and services – one factor in our achieving a coveted 4-star rating from Charity Navigator.

The support we have received made the Progeria gene discovery, the Progeria clinical trials, the first-ever treatment for Progeria, and all of our other extraordinary progress possible. With the help of current and new supporters, we *will* win this race against time and find treatments and the cure for these special children. Moreover, Progeria treatment discoveries may also help millions with heart disease and the entire aging population.

What is Progeria?

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is a rare, fatal genetic condition of accelerated aging in children. Children with Progeria die of the same heart disease that affects millions of normally aging adults (arteriosclerosis), but at an average age of just 14 years. Remarkably, their intellect is unaffected, and despite significant physical changes in their young bodies, these extraordinary children are intelligent, courageous, and full of life.

About PRF

The Progeria Research Foundation (PRF) was established in 1999 by Drs. Leslie Gordon and Scott Berns, the parents of a child with Progeria, along with many dedicated friends and family who saw the need for a medical research resource for the doctors, patients, and families of those with Progeria. Since that time, PRF has been the driving force behind the Progeria gene discovery and the first-ever Progeria drug treatment. PRF has developed programs and services to aid those affected by Progeria and the scientists who conduct Progeria research. Today, PRF is the only non-profit organization solely dedicated to finding treatments and the cure for Progeria. PRF is hailed as a prime example of a successful translational research organization, moving from creation, to gene discovery, to first-ever drug treatment in just 13 years.

PRF'S PROGRAMS & SERVICES

- **First-Ever Progeria Clinical Drug Trials and Treatment:** PRF-Sponsored Clinical Drug Trials bring children from around the world for promising treatments that may help to improve disease and extend the lives of children with Progeria. ***In 2012, history was made with the discovery that lonafarnib, a farnesyltransferase inhibitor or FTI, is the first-ever treatment for Progeria, improving many aspects of the disease including the vital vascular system. More recent studies conclude that lonafarnib extends estimated lifespan.*** In 2016, PRF initiated a two-drug trial, adding everolimus, with the hope that the two drugs together will be even more effective than lonafarnib alone. ***These are remarkable steps forward in the pursuit of a cure.***

- **International Progeria Registry** maintains centralized information on children and families living with Progeria. This assures rapid distribution of any new information that may benefit the children.

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- **Cell & Tissue Bank:** PRF's Bank provides researchers with genetic and biological material from Progeria patients and their families so research on Progeria and other aging-related diseases can be performed to bring us closer to the cure. PRF has collected an impressive 211 cell lines from affected children worldwide (ages 2 months to 17 years) and their immediate relatives, including 10 Induced Pluripotent Stem Cell (iPSC) lines.

- **Medical & Research Database:** The Database is a centralized collection of medical information from Progeria patients worldwide. The data is rigorously analyzed to help us understand more about Progeria and devise treatment recommendations. In 2010, this analysis contributed to PRF's comprehensive healthcare Handbook on Progeria aimed at optimizing quality of life. The Handbook is available in English, Spanish, Portuguese, Russian, and Italian.

- **Diagnostic Testing**: This program was developed in the wake of the 2003 gene discovery so that children, their families, and medical caretakers can get a definitive, scientific diagnosis. This can translate into earlier diagnosis, fewer misdiagnoses, and early medical intervention to ensure a better quality of life for the children.

- **Scientific Workshops on Progeria:** PRF has organized 11 conferences that have brought together scientists and clinicians from all over the world to share their expertise and cutting edge scientific data. These workshops foster collaboration in the fight against this devastating disease. The most recent meeting was May 2-4, 2016 in Boston, MA.

- **Research Grants:** Through peer review by our volunteer Medical Research Committee, PRF has funded projects throughout the world that have led to important discoveries about Progeria, heart disease, and aging. Awards of up to $100,000 per year, for up to three years, have allowed innovative new research in Progeria to thrive.

- **Publications and Research:** Both clinical and basic scientists have utilized the PRF grants, cells and tissues, and database; their discoveries are published in top-notch scientific journals. The average annual number of scientific publications on Progeria since 2002 is more than 20 times that of the previous 50 years!

- **PRF Translation Program:** ***In touch with the world***. With a prominent global presence, PRF eliminates barriers to communication for patients and their families from around the world. This initiative has succeeded in translating PRF program and medical care materials into 31 languages.

- **Public Awareness: progeriaresearch.org** provides access to the latest information on Progeria research and support for families. Through **Facebook**, **Twitter,** and other mediums, PRF's direct social media reach is over 1 million. PRF's story has appeared on CNN, ABC News, Primetime, Dateline, The Katie Couric Show, and The Today Show, in *Time* and *People* magazines, *The New York Times*, *The Wall Street Journal* and many other widely-read media outlets. In addition, the award-winning 2013 HBO film ***Life According to Sam*** has raised awareness in a unique and inspiring way. PRF also manages **Find the Other 150** (www.findtheother150.org), **a global awareness campaign** to find children with Progeria worldwide, so they can get the unique help they need.

WHO'S WHO AT PRF?

Audrey Gordon, Esq., President and Executive Director, Co-Founder
Working closely with the Board of Directors, officers, staff, and volunteers, Ms. Gordon is responsible for day-to-day management and for ensuring The Progeria Research Foundation's financial growth and program development.

Leslie B. Gordon, MD, PhD, Medical Director, Co-Founder
Dr. Gordon co-founded PRF with friends and family after her son, Sam, was diagnosed with Progeria. Dr. Gordon oversees PRF's research-related programs, and is a co-chair for the Progeria clinical drug trials. She is an Associate Professor of Pediatrics Research at the Warren Alpert Medical School of Brown University and Hasbro Children's Hospital in Providence, RI, and a Staff Scientist at Boston Children's Hospital and Harvard Medical School.

Scott D. Berns, MD, MPH, FAAP, PRF Chairman of the Board, Co-Founder
Dr. Berns, Sam's father, is a co-founder of The Progeria Research Foundation and serves as Chairman of the Board. He is a Board Certified Pediatrician and Clinical Professor of Pediatrics at the Alpert Medical School of Brown University. He is also President and CEO of the National Institute for Children's Health Quality, an independent, nonprofit organization working to improve children's health.

Meghan Waldron, PRF's Youth Ambassador
Meghan serves as a spokesperson for PRF, from the perspective of a youth with Progeria. Accomplished cellist and violinist, high school cross country and track team athlete, published poet – Meghan is a talented and busy 15-year-old living in Massachusetts.

Tina, Brandon, Brittany, and Zach Pickard, PRF's Ambassador Family
The family of 9-year-old Zach lives in Lexington, Kentucky, and is an integral part of PRF's efforts to raise public awareness and raise funds for research.

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