Progeria Research Foundation

QUICK FACTS as of January 2011

Mission: to discover the cause, treatment and cure for Hutchinson-Gilford Progeria Syndrome and its aging-related disorders.

PRF BY THE NUMBERS:

Known children living with Progeria: 78 in 31 countries

Children tested through PRF's Diagnostic Program: 88

Grants funded since PRF was established: 33

Cell lines in the PRF Cell & Tissue Bank: 151

Children participating in the PRF Medical & Research Database: 97

Total Dollars Raised

1999 through 2010: **$9,738,084**

85-90% of PRF's annual expenses are consistently dedicated to its programs and services.

The support we have received made the Progeria gene discovery, the Progeria clinical trials and all of our extraordinary progress possible. With the continued help of our supporters, we will win this race against time and find treatments and the cure for these special children – and perhaps also help millions who suffer from heart disease and other, aging-related conditions.

What is Progeria?

Progeria, also known as Hutchinson-Gilford Progeria Syndrome (HGPS), is a rare, fatal genetic condition characterized by an appearance of accelerated aging in children. All children with Progeria die of the same heart disease that affects millions of normal aging adults (arteriosclerosis), but instead of occurring at 60 or 70 years of age, these children may suffer strokes and heart attacks even before age 10. Remarkably, the intellect of children with Progeria is unaffected, and despite startling physical changes in their young bodies, these extraordinary children are intelligent, courageous, and full of life.

What is PRF?

The Progeria Research Foundation (PRF) was established in 1999 by the parents of a child with Progeria, Drs. Leslie Gordon and Scott Berns, and many dedicated friends and family who saw the need for a medical resource for the doctors, patients, and families of those with Progeria, and for funding of Progeria research. Since that time, PRF was the driving force behind the discovery of the Progeria gene, and has developed programs and services to aid both those affected by HGPS and the scientists that 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 model for disease-research organizations and a prime example of successful translational research, moving from creation, to gene discovery, to treatment trials in just 10 years.

PRF'S PROGRAMS & SERVICES

- **First-Ever Progeria Clinical Drug Trials for Progeria:** PRF funded and co-coordinated a $2 million, first-ever clinical drug trial for Progeria that took place in Boston with partners Children's Hospital Boston (CHB), Dana-Farber Cancer Institute and Brigham and Women's Hospital. The trial drug is called a farnesyltransferase inhibitor, or FTI, a drug that has shown great promise in the laboratory and in animal models. The trial enrolled 28 children from 16 countries, ages 3 to 15 years. If the drug proves effective, it will be a remarkable step forward in the pursuit of a cure.

  Since the start of the first trial, researchers identified two additional drugs that, when used in combination with the current FTI drug being tested, may provide an even more effective treatment for children with Progeria than the single drug. PRF moved quickly to explore these additional treatment options. PRF and CHB began a second clinical trial for Progeria in August 2009. The "Triple Drug Trial" is much larger than the first, involving 45 children from 24 countries, speaking 17 different languages.

- **PRF's 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:** The PRF Cell & Tissue Bank provides medical researchers with genetic and biological material from Progeria patients and their families, so that research on Progeria and other aging-related diseases can be performed to bring us closer to finding the cure. PRF has collected an impressive 90 cell lines from affected children worldwide (with ages ranging from 2 months to 17 years), and 61 lines from their immediate relatives.

❖**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 recommendations handbook on Progeria aimed at optimizing quality of life.

❖ **Diagnostic Testing**: PRF developed a diagnostic testing program for Progeria 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 6 scientific conferences that have brought together scientists and clinicians from all over the world to share their expertise and cutting edge scientific data, and foster collaboration in the fight against this devastating disease.

❖ **Research Grants:** PRF has awarded 33 research grants totaling over $2.5 million through peer review by our volunteer Medical Research Committee. Awards of up to $100,000, for up to two years, have allowed innovative new research in Progeria to thrive.

❖ **Publications and Research:** Both clinical and basic scientists have accessed 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 12 times that of the previous 50 years.

❖ **PRF Translation Program:** ***In touch with the world***. With a prominent global outreach, PRF eliminates barriers to communication for patients and their families around the world. This initiative has succeeded in translating PRF program and medical care materials into over 20 different languages.

❖ **Web Site/Public Awareness:** ProgeriaResearch.org provides access to the latest information on Progeria research, support, and education for families and caregivers. PRF's newsletters reach over 13,000 people in 60 countries. PRF's story has appeared on CNN, The Dr. Oz Show, Primetime Live, Dateline, and The Today Show, and in *Time* and *People* magazines, *The New York Times*, *The Wall Street Journal* (front page!) and dozens of other widely-read media outlets.

In October 2009, PRF and its partner GlobalHealthPR launched a global awareness campaign called **Find the Other 150,** to drive the search for unidentified children with Progeria worldwide. The campaign has helped PRF to discover new families and children with Progeria who need our help.

WHO'S WHO AT PRF?

Audrey Gordon, Esq., President and Executive Director, Co-Founder
Audrey Gordon, Esq. oversees all administrative aspects of The Progeria Research Foundation, including the Board of Directors' activities, fund-raising, grant submissions, medical research projects administration, and volunteer programs.

Leslie B. Gordon, MD, PhD, Medical Director, Co-Founder
Dr. Gordon co-founded The Progeria Research Foundation with friends and family after her son, Sam, was diagnosed with Progeria. Dr. Gordon oversees the Diagnostics Testing Program, Cell & Tissue Bank, and Medical & Research Database, and is a co-chair for the Progeria clinical drug trials. She is 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 Children's Hospital Boston and Harvard University 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 the 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 Senior Vice President of Chapter Programs at the March of Dimes.

Tina, Brandon, Brittany and Zach Pickard, PRF's Ambassador Family
In January 2010, the family of 3-year-old Zach Pickard was named PRF's Ambassador Family. The Pickards live in Lexington, Kentucky, and are an integral part of PRF's efforts to raise public awareness, involve other families in our programs, and raise funds for research. We appreciate the time and effort they put into these important activities.