



## The Progeria Research Foundation QUICK FACTS

***MISSION:*** *To discover the cure and effective treatment for Progeria and its aging related disorders.*

- ❖ Number of known children living with Progeria: 52 children in 29 countries
- ❖ Number of children tested through the PRF Diagnostic Program: 73
- ❖ Number of grants funded since PRF was established: 24
- ❖ Number of cell lines in the PRF Cell & Tissue Bank: 111
- ❖ Number of children participating in the PRF Medical & Research Database: 77

### 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 suffering at 60 or 70 years of age, these children may suffer strokes and heart attacks as early as 5 or 6 years of age. Remarkably, the intellect of children with Progeria is unaffected, and despite startling physical changes in their young bodies (e.g. heart disease, alopecia, osteoporosis), 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 a diagnostic test, cell & tissue bank, grassroots funding, and medical database to aid those affected by HGPS and those researchers that want to conduct Progeria research. Today, PRF is the only non-profit organization solely dedicated to finding treatments and the cure for Progeria.

### PRF'S PROGRAMS & SERVICES

- ❖ **First-Ever Progeria Clinical Drug Trial for Progeria:** PRF is funding and co-coordinating the first-ever clinical drug trial for Progeria, taking place at Children's Hospital Boston. 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 began on May 7, 2007 and is expected to last through October 2009. To date PRF has raised nearly 1.8 million of the 2 million dollars needed to fund this trial.
- ❖ **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 71 cell lines from affected children worldwide (with ages ranging from 8 months to 17 years), and 40 lines from their immediate relatives.
- ❖ **Medical & Research Database:** The database is a collection of medical records of Progeria patients from all over the world. The data is rigorously analyzed to determine the best course of treatments to improve the patient's quality of life. This information is invaluable for the attending physician to guide the family through the best course of action. To date, PRF has enrolled 77 children in the program, and published three formal healthcare recommendations: Cardiac Care, Nutrition, and Occupational Therapy/Physical Therapy. The database was critical in determining the primary clinical outcome parameter for the first-ever clinical drug trial for Progeria.

### Total Dollars Raised

1999 through 2008: **\$6,694,209**

Currently, nearly 90% of donations to PRF go directly to support our programs and services.

The support we have received made the Progeria gene discovery, the Progeria clinical trial 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 help millions who suffer from heart disease and other, aging-related conditions.

- ❖ **Diagnostic Testing:** PRF developed a diagnostic test for Progeria in the wake of the 2003 gene discovery so that children, their families and medical caretakers can for the first time be given 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 five 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, lethal disease.
- ❖ **Publications and Research:** A major goal of PRF is to promote awareness about Progeria and the progress being made in the field of Progeria research. Dozens of publications on Progeria, many of which acknowledge PRF grant support or PRF's support of providing cells, have been published in well-known, respected scientific journals read by thousands of researchers worldwide. The average number of scientific publications on Progeria since 2002 is more than 10 times that of the previous 50 years.
- ❖ **Research Grants:** PRF has awarded 24 research grants totaling \$1.7 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.
- ❖ **Web Site/Public Awareness:** ProgeriaResearch.org provides visitors with access to the latest information on Progeria research, support, and education for families and caregivers. PRF's Web site enjoys an average of approximately 15,000 visitors per month, and PRF's newsletters reach nearly 10,000 people in 49 countries. Since the gene discovery, PRF's story has appeared on CNN, Primetime Live, Dateline, The Today Show, in *Time* magazine, *People* magazine and *The New York Times*, and dozens of other media outlets that have a keen interest in Progeria and PRF's work. In the summer of 2006, PRF kicked off a national public awareness campaign featuring the voices of Ted Danson and Mary Steenburgen. The PSA was endorsed by the Advertising Council, and aired on the Astrovision in Times Square. In January 2007, PRF and the Progeria Clinical Drug Trial were featured on the front page of *The Wall Street Journal*, reaching millions of readers worldwide.
- ❖ **Progeria Family Outreach - The PRF Progeria Family Message Board and Chat Room:** In keeping with our mission of continuing to provide valuable resources for families of children with Progeria, PRF has created a private message board Web site. This on-line tool helps the families get to know each other, and develop a support network of people with whom they can share concerns and ideas on how best to care for their children.

## **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 events, grant writing, medical research projects administration, and volunteer programs.

### **Leslie B. Gordon, MD, PhD, Medical Director, Co-Founder**

Leslie B. Gordon, MD, PhD co-founded The Progeria Research Foundation with friends and family after her son, Sam, was diagnosed with Progeria. As PRF's medical director, Dr. Gordon oversees the Diagnostics Testing Program, Cell & Tissue Bank, and Medical & Research Database, and is a co-Chair for the Progeria clinical drug trial. She is Assistant Professor of Pediatrics Research at the Warren Alpert Medical School of Brown University and Hasbro Children's Hospital in Providence, RI

### **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 is Senior Vice President of Chapter Programs at the March of Dimes. In 2001, Dr. Berns completed a one-year White House Fellowship where he served as a Special Assistant to the U.S. Secretary of Transportation.

### **Steve, Sandy, Megan, Michaela and Josh Nighbor, PRF's Ambassador Family**

In April 2005, the family of 8-year-old Megan Nighbor was named PRF's Ambassador Family. The Nighbors live in Dalton, Wisconsin, 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.