



The Progeria Research Foundation

President**Executive Director**

Audrey Gordon, Esq.

Treasurer

Carl Alviti, CPA

Clerk

Barbara F. Gordon, Esq.

Medical Director

Leslie B. Gordon, MD, PhD

Board of Directors

Carl Alviti, CPA

Karen N. Ballack, Esq.

Scott D. Berns, MD, MPH

Sandra A. Bresnick, Esq.

Rabbi Harold Kushner

Suzette Kushner, MS

Kim Paratore

John Seng

Elliott C. Wohlner, MD

Board of Advisors

W. Ted Brown, MD, PhD

Robert N. Butler, MD

Monica Kleinman, MD

George M. Martin, MD

Robert K. Morrison

Mary Oh, MD

William Oh, MD

Dr. William F. Schulz

Mel Zuckerman

P. O. Box 3453

Peabody, MA 01961-3453

Tel: (978) 535-2594

Fax: (978) 535-5849

info@progeriaresearch.org

www.progeriaresearch.org



February 3, 2010

Dear Friends,

Ten years ago, The Progeria Research Foundation (PRF) was founded to find a cure and effective treatments for Progeria and its aging related disorders. Today, PRF continues to be the only organization in the world dedicated to this mission. Our programs, developed with thoughtful determination over the past decade, continue to provide the resources needed not only to advance the field of Progeria, but also to discover more of what Progeria can tell us about heart disease and aging.

I am proud to provide you with this end of year report detailing PRF's 2009 accomplishments, which edged us ever-closer to our ultimate goal of a cure.

The biggest news for 2009 is that the first-ever Progeria clinical drug trial – funded and coordinated by PRF - ended in December, and the trial results will be available soon to let us know if we have found an effective treatment. Not wanting to wait until the first trial was complete to save precious time, PRF also started a second, larger clinical trial in August. The “Triple Drug Trial” is now testing two additional drugs that may help the children even more.

In the coming year, we will host our 6th scientific conference, fund and foster at least six new research projects, test more undiagnosed children, spread the word about Progeria and PRF's work, and do whatever else is needed to advance our quest for a cure.

You have helped make this all possible, and we are deeply grateful for your steadfast support.

Sincerely,

Audrey Gordon
President, Executive Director



2009 End of Year Report

Putting the spotlight on Progeria and the PRF programs that move us closer to treatments and a cure

Increased support for and interest in Progeria research around the world, the end of the first-ever Progeria drug trial, the launch of a second, much larger clinical trial to test more drugs, and a new global campaign to identify all children with Progeria -- these significant steps contributed to an exciting 2009 and the strengthening of our efforts to find a cure.

PRF's Programs and Services: *(2009 activities and updated information are italicized)*

PRF owns and operates its own international patient registry, cell and tissue bank, diagnostics testing program and medical and research database. PRF also organizes scientific workshops, provides start-up grants, and funds and co-coordinates clinical trials. This all-inclusive system centralizes the needs, and has proven extremely effective in driving the research forward for this rare, "rapid aging" disease. And while we work toward a future of treatments and cure, we also help the children with daily care issues. All programs are thriving and expand annually.

The First-Ever Progeria Clinical Drug Trial: Following the 2003 discovery of the Progeria gene, mouse models were created, the genetic defect intensely studied, and a potential drug treatment for children with Progeria called farnesyltransferase inhibitors, or FTI's was discovered. PRF rose to the challenge of coordinating the trial to test the drug with its new partners at Children's Hospital Boston, as well as committing to raise \$2 million to fund it – three times its then-current budget – and still continue to support its other important programs.

This first trial began in May 2007, involved 28 children (ages 3 to 15 years) from 16 countries and was just completed in December 2009. Trial results will be published in a scientific journal in 2010. PRF raised all funds needed to cover the costs of the trial.

Triple Drug Trial: *Since the start of the first trial, researchers identified two additional drugs that, when used in combination with the current drug being tested, may provide an even more effective treatment than the single drug. PRF moved quickly to explore these additional treatment options and, after a successful "mini trial" in April-May 2009 wherein 5 young children, ages 2-3 years, were given the 3 drugs to test the tolerability of side effects, PRF and Children's Hospital Boston launched this new clinical trial for Progeria in August 2009, which will run for 2 ½ years.*

The Triple Drug Trial is much larger than the first, involving 45 children from 30 countries: most of the 28 in the first trial, and those that were either too young for the first trial or not known to PRF during enrollment for that trial. As of last week, the trial is now fully enrolled.



Hayley, 10 years old, and Michiel, 9, in Boston in August for their final visit in the first clinical trial (for which they received trophies!) and their 1st visit for the new, triple drug trial.

***Funding:** In October 2009, PRF, Children's Hospital Boston and Dana-Farber Cancer Institute received the highly competitive and prestigious NIH "Grand Opportunities" grant, funded by the American Recovery and Reinvestment Act. This exciting grant will provide \$3.1 million in funding for the Triple Trial, thus the majority of costs are covered. PRF will be responsible for raising approximately \$100,000 of unfunded trial expenses.*



Adalia, now 3 years old, came to PRF when she was just 10 months old, and is now enrolled in the Triple Drug Trial. Being diagnosed at such a young age was virtually unheard of 10 years ago.

The PRF Diagnostics Testing Program: With the gene discovery came the ability to provide the children and their families a definitive, scientific diagnosis. This test makes earlier diagnosis, fewer misdiagnoses and early medical intervention possible to ensure a better quality of life for the children. *Since the gene discovery in 2003, 81 children have been tested, with most testing positive. In 2009, children from Brazil, Colombia, Portugal and the United States were diagnosed with Progeria, and tests for 5 children from India, Turkey, Brazil and the United States are in progress.*

Because of the ever-increasing worldwide awareness, more children are being found and tested at earlier ages. A record 54 children have been identified as living with Progeria today and we believe most, if not all, of the 5 pending will test positive. This number has steadily grown from 32 in 1999, to the low 40's in 2006, and now thanks to our efforts to reach all children with this disease, we are nearing the 60's.

The PRF Cell & Tissue Bank: After approval through a formal application process, this Bank provides medical researchers with precious biological material from Progeria patients and their families, so that research on Progeria and other aging-related diseases can be performed.

PRF now has an impressive 77 cell lines and various tissue samples from affected children from all over the world, whose ages range from 6 months to 17 years. Since its creation in 2002, cells have been sent to 41 researchers in 15 states and 9 countries. By the end of 2009, a total of 279 cell lines have been distributed.

The PRF Medical & Research Database: This labor-intensive program requires collection of medical records of Progeria patients from all over the world. The data in the records is rigorously analyzed to determine the best course of treatments to improve the children's quality of life, and to understand more about the basis of Progeria to assist with such vital information as clinical trial data. To date, 3 formal healthcare recommendations for Cardiac Care, Nutrition and Occupational Therapy/Physical Therapy have been published to help the children in their daily struggles with heart problems, weight/strength gain and stiff joints.

84 children have participated in this program, contributing their medical records to help develop treatment recommendations. In 2009, PRF began working on the Progeria Medical Assistance Booklet that will provide the most current information and resources for families, physicians and other caregivers about caring for children with Progeria. Information gathered from the database will be vital to this booklet, which is being funded by private foundation grants and will be completed in 2010.

Research Grants: PRF's grants of up to \$100,000 per year, for up to two years, have allowed innovative research in Progeria to thrive. Proposals are carefully evaluated by PRF's Medical Research Committee and Board of Directors. We now know more about Progeria and its biological link to heart disease and aging than ever before.

Five new research grants were funded in 2009. To date, PRF has invested over \$2 million to fund 28 grants for Progeria-related research projects performed in 13 states and 3 other countries.

Workshops on Progeria: PRF has organized five successful scientific conferences that have brought together scientists and clinicians from all over the world to collaborate, sharing their ideas and contributing their expertise in this lethal disease. The most recent workshop took place in Boston, MA in November 2007, bringing together 90 scientists from 8 countries for the largest conference gathering to date.

In 2009, PRF began planning the next workshop, "From Bench to Bedside in a Decade," scheduled for April 11-14, 2010 in Boston, MA with an estimated attendance of 125. Highlights include a plenary address by National Institutes of Health Director Dr. Francis Collins, a Progeria family panel and presentation of the first-ever Progeria clinical trial results.

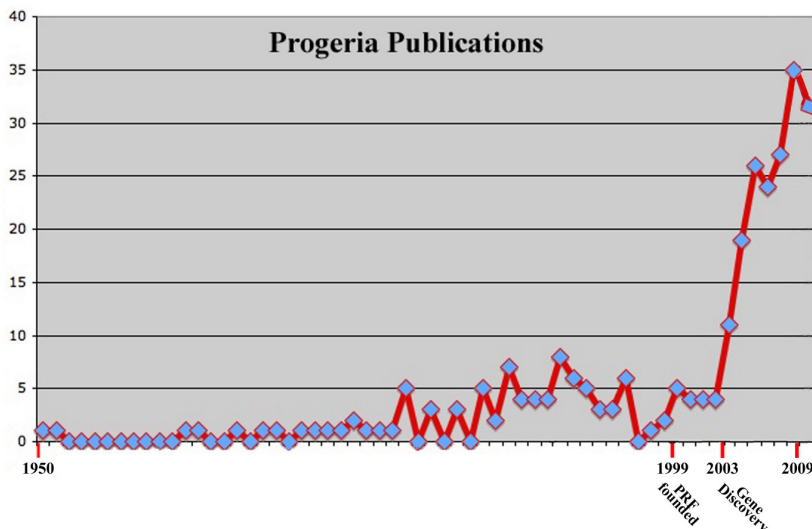
"The Progeria Research Foundation has made amazing strides: discovery of the genetic cause of the disease, testing of a possible drug treatment, and now the support of not just one but two clinical trials - Wow!"

- Francis Collins, MD, PhD, Director of the National Institutes of Health and co-discoverer of the Progeria gene.

Partnership with the National Institutes of Health (NIH): The partnership between PRF and NIH over the past nine years has resulted in more than \$323,000 in government grants to PRF, including NIH co-sponsorship of all five PRF scientific meetings. PRF's efforts also led to NIH funding of basic science research related to Progeria, for the first time in NIH history.

PRF's collaborations and interest in Progeria research continue with NIH; most recently NIH awarded a \$3.1 million grant for the recently launched Triple Drug Trial.

Publication of Scientific Papers: A major goal of PRF is to promote awareness about the progress being made in the field of Progeria research. Interest has flourished since the gene discovery, as more and more high-level scientists produce data that will help better understand Progeria and develop treatments. The number of publications continues to rise, many of which acknowledge PRF grant, cell bank or database support, and are published in well-known, respected scientific journals read by researchers worldwide.



From 1950-2002, there were 104 peer review publications on Progeria: an average of 2 per year. From 2003-2009, 173 articles were published: an average of 24.7 per year. Thus the average annual increase since 2003, the year the Progeria gene was discovered, is over 1,200%! We wonder if there is any other disease field in the history of science that has been able to generate this kind of interest and progress so quickly, and have great hopes this will translate into treatments and cure, the mission of PRF, in the near future.

Public Awareness: Before PRF was formed, virtually no one knew what Progeria was. Information about Progeria and our far-reaching message - that finding a cure may help those with heart disease and other aging-related conditions – has reached millions through PRF’s web site, newsletters, educational materials and the media. PRF’s story has appeared on CNN, Primetime, BBC, Dateline, Discovery, in Time and People magazines, The New York Times, The Wall Street Journal (front page!) and dozens of other widely-read media outlets. As awareness continues to spread throughout the world, more children come to PRF for diagnostic testing; more researchers apply to PRF for grant funding and cells to support their research; more scientists participate in PRF’s scientific workshops, and more volunteers offer needed support.

*PRF is now partnering with worldwide health communications group **GlobalHealthPR** in a global awareness campaign called **Find the Other 150**. This campaign, launched in October 2009 in the Americas, Europe, Russia, Asia and Africa, will drive the search for unidentified children with Progeria worldwide by raising awareness of the disease among both the general public and medical communities. GlobalHealthPR is the largest organization dedicated exclusively to healthcare communications worldwide. Its leader, Spectrum, based in Washington, DC, has been providing pro bono services to PRF since the 2003 Progeria gene discovery. The campaign is working! Launched just 3 months ago, we have found 3 more of the 150: 2 in Brazil, 1 in Turkey. Another 3 children also found their way to PRF, 2 from India and one, 10-month-old from the United States, mainly due to widely-viewed programs on Discovery’s TLC and ABC, and the release of an Indian movie about a child with Progeria, all in December 2009.*

*PRF also secured a partnership with **Baja Fresh**. In August 2009, the popular chain of Mexican-inspired food restaurants began a one-year fundraising and awareness campaign for PRF, collecting donations at 85 stores nationwide through coin boxes placed near the cash registers.*



Volunteers: PRF is so fortunate to have such wonderful volunteers!* Students hold school events, companies have dress down days, athletes run marathons, dozens place Coins to Cure Progeria© cans in stores – the list goes on and on, and grows each year.



PRF Executive Director Audrey Gordon and her nephew Sam celebrate Night of Wonder 2009.

*In 2009, our dedicated chapter and other volunteers worked harder than ever; many chapters saw significant increases in fundraising revenue, with a total of \$510,000 raised by outside events. A team of 40 volunteers spearheaded planning efforts for PRF’s 2009 **Night of Wonder** Gala, which brought together hundreds of passionate supporters and raised \$267,000 to help fund PRF’s programs. Many volunteers also supported the first **ONEpossible Campaign**, heading up 11 teams that brought in more than 800 donors (636 of which were first-time donors), raising \$107,000 in just two months!*



**PRF’s Board of Directors, committee members, corporate officers, lawyers, accountants, graphic designers and public relations representatives all devote their time, energy and talents to PRF for free to ensure less is spent on administrative costs and more on raising awareness and finding a cure for Progeria.*



PRF in 2010

PRF will continue working to find the cure by:

- ✓ Publishing results of the first-ever Progeria clinical drug trial and managing the Triple Drug Trial, with the hope that these two trials will support effective treatments for children with Progeria, and foster additional research directions.
- ✓ Keeping the Diagnostic Testing Program up and running continually, so that early and accurate diagnoses can be made and medical intervention can be implemented.
- ✓ Increasing the number of participants in the Cell & Tissue Bank and Medical & Research Database projects, and keeping those vital projects operating so that scientists can use them as research tools to understand Progeria, heart disease and aging.
- ✓ Funding additional research proposals that focus on studying the Progeria gene and how this defect can be corrected.
- ✓ Hosting the 2010 international scientific meeting, to continue to expand the collaborations and encourage novel ideas for treatments and a cure.
- ✓ Maintaining and fostering our partnership with the National Institutes of Health to encourage more funding of Progeria research and continued workshop and program support.
- ✓ Partnering with Spectrum and GlobalHealthPR to increase awareness of Progeria so children will be accurately diagnosed at earlier ages, more scientists will get involved in our efforts and more individuals will support our cause.
- ✓ Expanding our volunteer base and revenue sources to support and expand PRF's programs.

Conclusion

This is an exciting time for children with Progeria and their families, and all those who support the work of The Progeria Research Foundation. The announcement of the first-ever trial results may be the biggest news for the field since the gene discovery, and PRF, after ten solid years of hard work and phenomenal progress, is well-positioned to seize upon opportunities to increase awareness and support that could arise from the world-wide attention such news will receive. Your support has made it possible for Progeria and PRF to be squarely in the spotlight, moving aggressively forward toward a cure. *Thank you!*

Financial Profile

