SEPTEMBER 2010: MID-YEAR REPORT ON ACTIVITIES

Progeria is a fatal, genetic condition characterized by an appearance of accelerated aging in children. The Progeria Research Foundation (PRF) was founded in 1999 to discover treatments and a cure 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 detailing of PRF's accomplishments over the past year, which edged us ever-closer to our ultimate goal of a cure. The biggest news of 2009 is that in December all patients completed their visits for the first-ever Progeria clinical drug trial – funded and coordinated by PRF - and the trial results will be available soon to let us know if we have found an effective treatment. In addition, the launch of a second, much larger clinical trial to test more drugs, and a new global campaign to identify all children with Progeria contributed to an exciting 2009; Both initiatives are going strong in 2010, along with many other activities. We greatly appreciate the support of thousands who have helped make all of this possible.

Lindsay, 5 years old and Kaylee, 6, in Boston September 2009 for their final visit in the first clinical trial (for which they received trophies!) and their 1st visit for the new, triple drug trial.

Both girls are featured in a TLC documentary, "6 Going on 60", that premiered in December and re-airs frequently due to its popularity.

PRF's Programs and Services 2009-2010

PRF owns and operates its own international patient registry, cell & tissue bank, diagnostics testing program and medical & research database. PRF also organizes scientific workshops, provides treatment guidance for families and their physicians, offers research 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. 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 identified. 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 patient visits were completed in December 2009. The trial team is analyzing the data and will announce the results in 2010. If the drug proves effective, it will be a remarkable step forward in the quest for a cure. PRF raised all funds needed to cover the trial costs.

Triple Drug Trial: Since the start of the first trial, researchers identified two additional drugs that, when used in combination with FTI's, 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 fully enrolled and much larger than the first, involving 45 children from 24 countries, including most of the 28 children who participated in the first trial and those that were either too young or not known to PRF during enrollment for that trial. We are thrilled to move forward with testing this second treatment option, where just four years earlier none was available.

"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, in November 2009

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 has already received $170,000 in private foundation grants to cover most, if not all, of the unfunded trial expenses.

9-month-old Zoey was diagnosed with Progeria in March 2010. Identification 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, 90 children have been tested, with most testing positive. In the past year, children from Brazil, Colombia, Japan, Pakistan, Portugal, South Africa and the United States were diagnosed with Progeria, and tests for children from Brazil, India, and Peru are in progress.

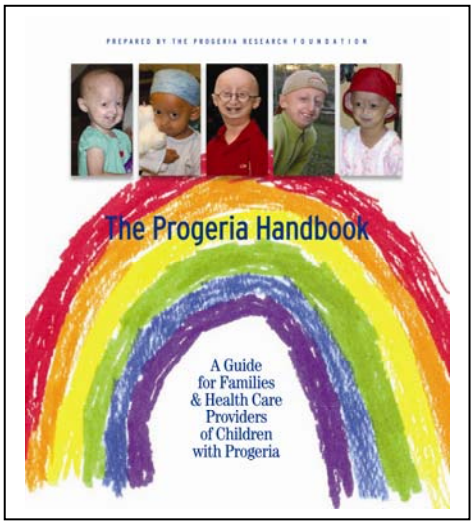
Because of the ever-increasing worldwide awareness, more children with Progeria are being found and tested at earlier ages. As of July 2010, a record 67 living children have been identified. This number has steadily grown from 32 in 1999, to the low 40's in 2006, and now thanks to our efforts to reach all children, we are in the high 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 86 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 44 researchers in 15 states and 9 countries. To date, a total of 283 cell lines have been distributed.

PRF is now embarking on cutting-edge technology, expanding the bank to include "Induced-Pluripotent Stem Cells" (iPSC). These cells are created from Progeria patient cells and transformed into Progeria vascular smooth muscle and other cell types that are difficult to explore naturally. They will be valuable for researchers' basic studies and for drug development.

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. 94 children have participated in this program, contributing their medical records to help develop treatment recommendations.

In April 2010, PRF published The Progeria Handbook, a portable book that provides families, researchers, and caregivers such as physicians and teachers easy access to information ranging from basic health facts and daily care recommendations to detailed medical treatment guidelines. With removable pages, the handbook can be easily updated as new treatment information becomes available. The book is also readily available to download, in whole or by chapter, at www.progeriaresearch.org/patient_care Information gathered from the database was vital to this handbook, which was funded by private foundation grants and has been distributed to nearly 200 thus far.



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. Thanks to these studies, we now know more about Progeria and its biological link to heart disease and aging than ever before. Five new research grants were awarded in 2009, and one thus far in 2010. PRF has invested over $2 million to fund 29 grants for Progeria-related research performed in 13 states and 5 countries.

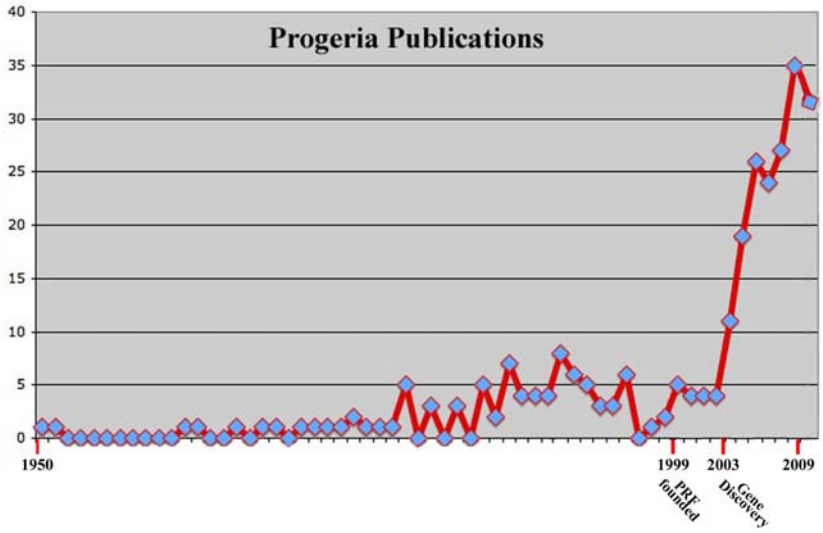
"This year's workshop clearly set a new benchmark. It was one of the most interactive and informative meetings of its kind I have ever attended. The spirit of the meeting was remarkably collaborative, upbeat and inspiring."

- Michael Gimbrone, MD, Professor of Pathology at Harvard Medical School, Chairman of the Dept. of Pathology at Brigham & Women's Hospital, Boston, MA.

Workshops on Progeria: PRF has organized six 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, *From Bench to Bedside in a Decade*, took place in Boston in April 2010, with a record 140 participants from 10 countries.

Partnership with the National Institutes of Health (NIH): The relationship between PRF and NIH over the past nine years has resulted in several government grants to PRF, including NIH co-sponsorship of all six workshops. PRF's efforts also led to first-ever NIH funding of basic science research related to Progeria. PRF's Medical Director is periodically invited to present at workshops sponsored by NIH's Office of Rare Diseases, where she discusses PRF's programs and how they have helped advance the field. PRF's collaborations with NIH continue to build and strengthen government interest in this field, as evidenced by the recent grant for the 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. The most recent publication, led by PRF's Medical Director, reports on a landmark study that demonstrates the Progeria-causing protein Progerin increases in everyone as we age, suggesting a possible new risk factor for heart disease. (August 26, 2010: *Arteriosclerosis, Thrombosis, and Vascular Biology)*



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, representing an average annual increase of over 1,200% since 2003, the year the Progeria gene was discovered. There is likely no other disease field in the history of science that has been able to generate this kind of interest and progress so quickly; the hope is that this will translate into treatments and cure 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 scores of other widely-read media outlets. As awareness continues to spread, more children come to PRF for diagnosis; more researchers apply for grant funding and cells to support their work; more scientists participate in PRF's scientific workshops, and more supporters offer help.

In October 2009, PRF began a partnership 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, is driving the search for unidentified children with Progeria worldwide by raising awareness of the disease. 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. *And it's working!* We are thrilled that since the campaign began 11 months ago, we've seen an unprecedented jump from **54 to 67 children – a 24% increase.** The most recent 13 live in Brazil, India, Japan, Peru, Portugal, South Africa, Turkey and the United States.

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

In addition, PRF's new, sharper logo appears on the recently upgraded website, which now features rolling photos and videos. We are also using our ever-growing FaceBook and Twitter presence to support worldwide awareness of Progeria.

Volunteers: PRF is so fortunate to have such numerous and 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.

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 (held every other year), our signature event that brought together hundreds of passionate supporters and raised $267,000 to help fund PRF's programs.

PRF co-founders Drs. Scott Berns and Leslie Gordon celebrate Night of Wonder 2009 with their son Sam.

Many volunteers also supported the first **ONEpossible Campaign**, led by 11 teams that brought in over 800 donors (636 of which were first-time donors), raising $107,000 in just two months! The 2010 ONEpossible campaign took place May-June, and raised an astonishing $182,000 with hundreds of additional, new supporters joining the effort to find a cure.

onepossible™
The Progeria Research Foundation

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* *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. Our administrative/fundraising expenses are always 15% or below.*

PRF in 2010 and Beyond

We plan to live up to our reputation of speedy and considerable accomplishments for the remaining months of 2010 and the coming years, including the following:

- ✓ Publish results of the first-ever Progeria clinical drug trial and complete the first half of the Triple Drug Trial, with the hope that these two trials will support effective treatments for children with Progeria, and foster additional research directions.

- ✓ Continue our partnership with Spectrum and GlobalHealthPR to increase awareness of Progeria so more children will be found and accurately diagnosed at earlier ages.

- ✓ Increase the number of participants in the Cell & Tissue Bank and Medical & Research Database projects, and keep those vital projects operating so that scientists can use them as research tools to understand Progeria, heart disease and aging.

- ✓ Fund additional research proposals that focus on studying the Progeria gene and how this defect can be corrected.

- ✓ Maintain and foster our partnership with the National Institutes of Health to encourage more funding of Progeria research and continued workshop and program support.

- ✓ Expand 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.

You have helped make it possible for Progeria and PRF to be squarely in the spotlight, moving aggressively forward toward a cure and we are deeply grateful for your kind support.

Sincerely,

Audrey Gordon, Esq.
President, Executive Director