The Progeria Research Foundation

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A note of thanks…

The Progeria Research Foundation (PRF) continues to be the only organization in the world dedicated to discovering the cure and effective treatment for Progeria and its aging related disorders through medical research, outreach and education. Since its founding in 1999, and with relentless passion and perseverance, we are making tremendous strides toward our ultimate goal of a cure. The year 2008 is no exception. I am delighted to provide you with this end of year report detailing PRF's accomplishments of the last twelve months.

In 2009 PRF will celebrate ten years of existence, and what a decade it has been! From gene discovery to clinical trial, we are forging ahead at an unparalleled pace. The 2½-year trial – funded and coordinated by PRF - will end in late 2009, and soon thereafter we will know if we have found an effective treatment for the children. Such an exciting time!

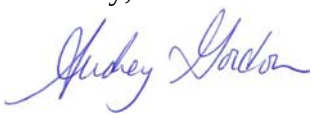
We continue to run all of our programs with worldwide outreach. Researchers receive funding and the use of cells and tissues from our Bank, and attend the scientific workshops; children are being tested for Progeria; families and their doctors continue to receive the medical information they need. These essential programs have made the fantastic progress possible, and they are thriving thanks to your support.

The year 2008 ends with the exciting news that farnesyltransferase inhibitors (FTIs – the drug being tested in the current clinical trial) may reverse the heart disease experienced by those with Progeria. The study also provides further confirmation of the link between Progeria and common heart disease. Imagine: Finding a cure for Progeria, one of the rarest diseases, could help millions with the most common illnesses – heart attacks and strokes. Amazing!

We started from ground zero and quickly and efficiently did what was needed to bring Progeria into the spotlight and on the road to a cure. We could not – and cannot – do it without you, so please know how much all of us at PRF appreciate your support.

May the New Year bring you much happiness and good health, and bring us that much closer to a cure.

Sincerely,



Audrey Gordon
President, Executive Director

2008 End of Year Report

Adding to the ever-growing list of historic milestones that bring us closer to treatments and a cure.

The first full year of the Progeria clinical drug trial, three new PRF chapters launched, and **new research studies that show Progeria may be reversible**! All this and lots more contributed to a terrific 2008 and an intense eagerness to continue our quest for a cure in 2009.

PRF's Programs and Services: *(2008 activities and updated information are italicized)*

Unlike most medical research organizations, PRF created, owns and operates its own cell and tissue bank, diagnostics testing program and medical and research database. Recognizing the historical lack of Progeria research funding and structured promotion of research, 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 for this "rapid aging" disease forward. All programs are thriving and have expanded with each year.

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 FTI drug with its new partners at Children's Hospital Boston, as well as committing to raise $2 million to fund the trial – three times its then-current budget – and still continue to fund its other important programs.

"This could mark a major breakthrough for FTI drugs to treat Progeria and potentially other forms of heart disease."

- Dr. Elizabeth Nabel, Director of the National Heart Lung and Blood Institute and co-author of an October 2008 study that found FTI's actually reversed the heart disease in Progeria mice. What encouraging news for the current Progeria clinical drug trial!

2008 was the first full year of the trial; it began in May 2007 and is scheduled to end in October 2009. *To date, all 27 participating children have completed their 1-year visit, and most have just 2 more visits to go. As of December 2008, 90% ($1.79 million) of the total funds needed to cover the costs of the trial have been raised.*

The drug trial is the best hope today to prolong and improve the lives of children with Progeria. Its further potential to help millions with heart disease is truly astounding.

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, 73 children have been tested, with most testing positive, and 3 are in progress. In 2008, children from Brazil, South Africa, Egypt, Colombia, Argentina, Japan and the United States were diagnosed with Progeria.*

Because of the ever-increasing worldwide awareness, more children are being found. A record 52 children have been identified as living with Progeria today; 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 worldwide with this disease, we have reached the 50's.

It was a relief to get the diagnosis, just to finally have that part over with and to know how to deal with it and move forward

- Stephanie, mother of Cameron (pictured here and diagnosed in March 2007 at age 6 months)

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. *Presently, PRF has gathered an impressive 71 cell lines and various tissue samples from affected children from all over the world and whose ages range from 6 months to 17 years.* Since its creation in 2002, cells have been sent to dozens of researchers in 11 states and 7 other countries. *In 2008, a record 55 lines were distributed to scientists.*

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 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. *77 children have participated in this program, and in late 2008, PRF received grant funding to create a comprehensive booklet for families and doctors to use for treatment purposes. Information gathered from the database will be vital to this booklet, which will be completed in 2009.*

Research Grants: PRF's grants of up to $50,000 per year, for up to two years, have allowed innovative research in Progeria to flourish. Proposals are carefully evaluated by PRF's Medical Research Committee and Board of Directors. *As of December 2008, PRF has invested $1.7 million to fund 24 grants for projects performed in 11 states and 3 countries. In 2008, PRF increased its solicitation efforts by disbursing information on PRF grant opportunities to several online searchable databases such as Infoed, Community of Science, IRIS, and internal research institutions' listings.*

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 ideas and expertise on this lethal disease. The most recent workshop took place in Boston, MA in November 2007, bringing 90 scientists from 8 countries together for the largest conference gathering to date. *The next workshop will likely be held in 2010, after the clinical trial results have been published.*

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, including NIH co-sponsorship of all five PRF scientific meetings. PRF's efforts also led to NIH funding basic science research related to Progeria for the first time in NIH history. *Collaborations continue, as leaders from NIH's National Heart, Lung and Blood Institute and Human Genome Research Institute are involved with the clinical trial and several other Progeria research projects.*

Publication of Scientific Papers: Through its grants, cell distribution and other resources, PRF has helped facilitate a tremendous increase in scientific publications. This increase promotes awareness about the progress being made in the field of Progeria research, and more and more high-level scientists are producing data that will help to better understand Progeria and develop treatments. The average number of scientific publications on Progeria since 2002 is more than 10 times that of the previous 50 years. *In 2008, 14 scientific papers on Progeria, and dozens more on related fields of lamins, progeroid syndromes, aging, etc. were published in well-known, respected scientific journals read by thousands of researchers worldwide. Also in 2008 a new section, "What's News in Progeria Research," was added to the PRF website to provide easy access to and increased awareness of the most current Progeria research.*

Web Site/Public Awareness: It's hard to imagine that just nine years ago, virtually no one knew what Progeria was. Now, facts about Progeria and our far-reaching message - that finding a cure may help millions with heart disease and other aging-related conditions – has reached millions worldwide, mainly through PRF's web site 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 and dozens of other widely-read media outlets. *The 2008 PRF Media Results Report on media coverage of PRF and Progeria showed that an estimated 106 million people were reached in 20008 alone, through print and the internet. With this vast outreach, more children are being helped than ever before.*

Lindsay, 4 years old from Michigan, enjoys a laugh and hug with her mom while interviewing with the Detroit Free Press in August 2008. *Photo courtesy of the Detroit Free Press*

PRF has recently formed a partnership with GLOBALHealthPR, an international consortium of successful healthcare public relations firms in Europe, the United States and Asia. Staffed by more than 175 communication specialists, they are the largest independent public relations group dedicated exclusively to health and medical communications worldwide, and have generously offered their services pro bono.

PRF and GLOBALHealthPR will work together to create the largest worldwide presence possible, to find and assist all children with Progeria.

Volunteer Expansion: PRF is so fortunate to have such wonderful volunteers![*] Students hold events at their school, companies have dress down days, athletes run marathons, hundreds of Coins to Cure Progeria© cans are in stores – the list goes on and on, and grows each year. *2008 also saw the launch of three new chapters in Kentucky, Michigan and Pennsylvania, led by families of newly diagnosed children, bringing the total number of chapters to seven. These dedicated supporters raised nearly $500,000 and awareness to tens of thousands in 2008. And all year, a team of 30 translators have helped PRF effectively communicate in 13 different languages with our trial families.*

Hundreds joined Cameron Howard's family at the first annual "Kilometers for Cam" race in September, run by PRF's new Southwest Michigan chapter.

PRF in 2009 and Beyond: PRF will continue working to find the cure by

- ✓ Completing the first-ever Progeria clinical drug trial, with the hope that FTIs will prove an effective treatment, and the trial results will pave the way for 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.
- ✓ Holding additional scientific meetings to continue to expand the collaborations and 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.
- ✓ Increasing public awareness so more children will be properly 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

For nine years, PRF supporters have worked tirelessly to propel Progeria research forward, with fantastic results that have now brought us to the brink of a potential treatment. And as awareness of Progeria, its link to heart disease and aging, and the resources PRF provides spreads throughout the world, we move closer to saving the lives of these special children.

[*] The 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.

Financial Profile