Together, we will find the cure!

Progeria Research Foundation

2010 Annual Report

This is an extraordinary time for children with Progeria and their families, and for all those who support the work of The Progeria Research Foundation. After 11 solid years of hard work and phenomenal progress, PRF is well-positioned to seize opportunities to increase awareness and gain additional support. You are an essential part of the PRF team. You have helped make it possible for Progeria and PRF to be squarely in the spotlight, moving aggressively forward toward a cure, and for that we are deeply grateful.

Together, we WILL find the cure!

Audrey S. Gordon, Esq.

President, Executive Director

Progeria is a rare and fatal genetic disease characterized by an appearance of accelerated aging in children. Without the discovery of new treatments, all children with Progeria will die of heart disease at an average age of 13 years. The Progeria Research Foundation (PRF) was founded in 1999 in response to the complete lack of progress being made to help children with Progeria. We are a 501(c)(3), non-profit organization. Our mission: to discover the cause, treatments and cure for Progeria.[1] Today, PRF continues to be the only organization in the world solely dedicated to this mission. We have filled a void, taking these children out of the background where they had been for over 100 years and putting them and Progeria at the forefront of scientific efforts.

In just 11 years, we have achieved extraordinary progress towards our mission: the Progeria gene discovery in 2003, first-ever clinical drug trials initiated in 2007, extensive global awareness of the disease and PRF's work, and discovery of critical biological links between Progeria, heart disease and the aging we all experience.

All of this progress is due in large part to the creation of PRF's research-related programs and services. Developed with insightful determination, they provide the resources needed not only to advance the field of Progeria, but also to discover what Progeria can tell us about heart disease and aging.

With the support of dedicated staff and volunteers, a talented board of directors, courageous families, and thousands of generous people around the world, we are pushing the field of Progeria forward towards discovery, treatments, and cure. And along the way, we are learning a tremendous amount about ourselves.

Please enjoy this report, which details PRF's accomplishments in 2010 **(highlighted in blue)**. The tremendous success of our global campaign to identify all children with Progeria, and reaching the half-way point in the Triple Drug Trial contributed to an exciting year. Both of these initiatives will continue to be strong in 2011, along with the many other activities which have edged us ever-closer to our ultimate goal of a cure.

Your love and support for children with Progeria has made these exciting strides possible.

"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 clinical trials - Wow!"

- Francis Collins, MD, PhD
Director, National Institutes of Health (NIH)

In June 2010, Rafaely from Brazil and Ontlametse from South Africa were together in Boston for their Triple Trial visits. With 2-3 families coming in weekly, children with Progeria and their parents have a unique opportunity to meet in person.

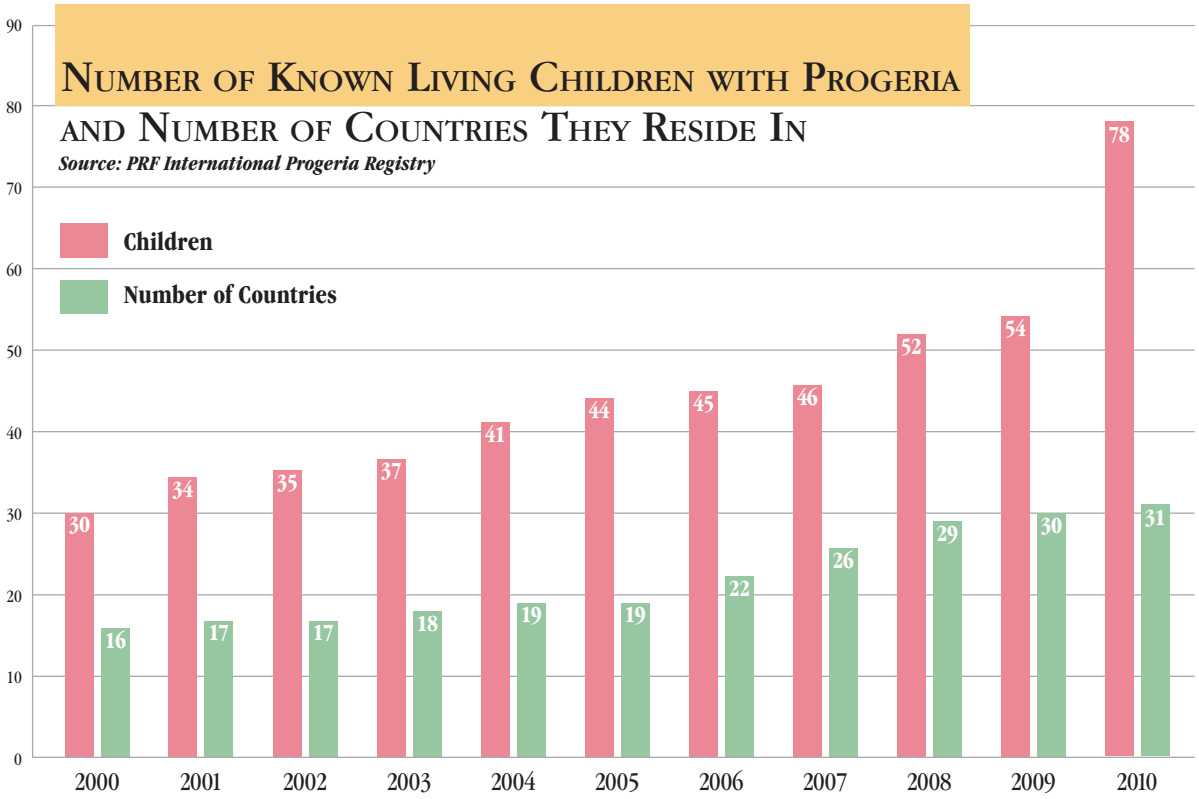
[1] In 2004, in response to the Progeria gene discovery and the growing scientific evidence linking Progeria to the heart disease and aging that affects us all, the mission was revised: To discover treatments and the cure for Progeria and its aging related disorders.

PRF operates an **international patient registry**, **cell and tissue bank**, **diagnostics testing program**, and **medical and research database**. PRF also organizes **scientific workshops**, funds and co-coordinates **clinical trials**, and translates important informational materials into over 20 languages. These programs work together to provide the core structure that has been essential in driving the field of Progeria forward. We are proud to collaborate with such prestigious academic institutions as Brown University and Harvard University to achieve these programs and services. All programs are thriving and expand annually, as the number of children identified and the number of researchers involved continue to climb.

The PRF International Progeria Registry

The Registry securely maintains centralized medical and contact information on children and families living with Progeria. This assures rapid distribution to families and their physicians of any new information that may benefit the children. Because of the ever-increasing worldwide awareness resulting from successful PRF outreach programs, more children with Progeria are being found and undergoing genetic diagnostic testing at earlier ages.

In 2010, we experienced a 44% increase in the number of children registered, with a record 78 children living with Progeria now identified.



Progeria Clinical Drug Trials: Forging Ahead Toward Treatments

Trial #1 update: Following the 2003 Progeria gene discovery, scientific progress in the field exploded. The genetic defect was intensely studied, mouse models were created so that potential treatments could be tested using animal models before moving into human trials, and a potential drug treatment for children with Progeria called farnesyltransferase inhibitors, or FTI's, was identified. PRF rose to the challenge by funding and coordinating a 2 ½-year, $2 million clinical trial that treated children with Progeria from across the globe. In May 2007, with our new partners at Harvard hospitals in Boston (Children's Hospital Boston, Dana-Farber Cancer Institute and Brigham and Women's Hospital), PRF made history when the first-ever clinical trial began. The clinical trial involved multiple visits for 28 children from 16 countries, which were completed in December 2009.

In October 2010, 6-year-old Niccolo and 14-year old Sammy, both from Italy, came to Boston for their third Triple Trial visit, marking the one-year point in their participation.

New discoveries like those that will come from this trial will help us to understand Progeria, its relationship to aging, and will help us define measurements of drug effect in future clinical treatment trials. **Throughout 2010 and presently, the trial team has been analyzing the data collected over these 2 ½ years. If the FTI drug proves effective, this will be a remarkable step forward in the quest to find a cure.**

Triple Trial update: In 2009, researchers from Spain identified two additional drugs that, when used in combination with FTI's, may provide an even more effective treatment than the single FTI drug. PRF moved quickly to explore these additional treatment options and worked with the Harvard hospitals to launch a new, Triple Drug Trial [2] for Progeria in August 2009, which will run through January 2012.

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[2] In October 2009, PRF, Children's Hospital Boston and Dana-Farber Cancer Institute received a highly competitive and prestigious NIH "Grand Opportunities" grant, funded by the American Recovery and Reinvestment Act. This exciting grant provides $3.1 million in funding over two years for the Triple Trial. PRF has also received $170,000 in private foundation grants to cover some of the additional trial expenses.

In January, 2010, enrollment in this new trial was completed. A total of 45 children from 24 countries enrolled in the Triple Trial. This included most of the original 28 children who participated in the first trial, as well as those who were either too young or not known to PRF during enrollment for that trial. These participants and their families are traveling to Boston every six months for two years for medications and study testing; **in 2010, 84 trips were successfully completed.**

We are thrilled to offer a second potential treatment option, and to a larger group of children, where just four years earlier none existed.

The PRF Diagnostics Testing Program

Immediately following the gene discovery in 2003, the first genetic test for Progeria was created. Used in conjunction with clinical information, the test provides a definitive, scientific diagnosis. Now earlier diagnosis, fewer misdiagnoses and earlier medical interventions make it possible to provide a better quality of life for the children. Since the gene discovery, 93 children have been tested.

In 2010, children from Brazil, Japan, Pakistan and the United States were diagnosed with Progeria through this program.

Research Grants

PRF's grants to basic scientists around the globe have propelled innovative research on Progeria. Thanks to these research studies, we now know more about Progeria and its biological link to heart disease and aging than ever before. To date, PRF has invested over $2.5 million to fund 33 basic science grants for Progeria-related research performed in 13 states and 3 other countries.[3]

Five new research grants were awarded in 2010, with seven active projects taking place throughout the year. We welcome our new research collaborators at Brunel University, Northwestern University Medical School, Massachusetts General Hospital, East Tennessee State University and University of Maryland.

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[3] This funding is separate from clinical trial funding, which brings the total to nearly $5 million.

" PRF is our lifeline, giving us help for Zoey today, and hope for a cure tomorrow."

- Laura and Ian,
Zoey's parents

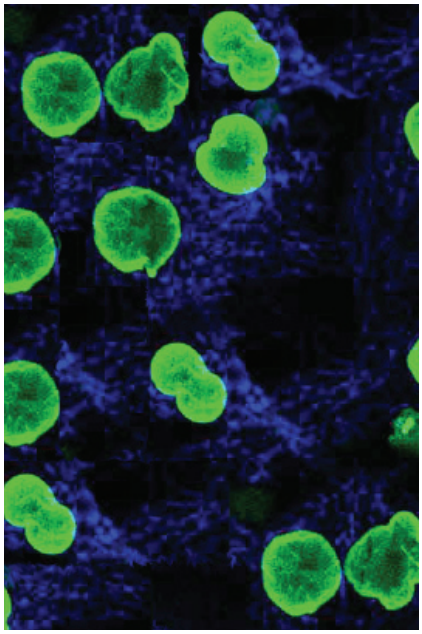
Zoey was diagnosed with Progeria in March 2010 at 5 months old, the age at which she is pictured here. Identification at such a young age was virtually unheard of 10 years ago.

The PRF Cell & Tissue Bank

In collaboration with Hasbro Children's Hospital and Brown University in Providence, Rhode Island, PRF established The PRF Cell & Tissue Bank. This essential resource provides precious biological materials to scientists throughout the world who are exploring the biology behind Progeria, conducting the science that will bring us to treatments and cure, and discovering the biological links between Progeria and aging. PRF now has an impressive 155 cell lines and various tissue samples from individuals with Progeria and their family members.

Since its creation in 2002 through the end of 2010, 254 cell lines have been distributed to 43 teams of researchers at 38 institutions in 16 states and 8 other countries: Germany, Sweden, Israel, Italy, Canada, Austria, China and England.

Cutting-edge, new stem cells now offered: In 2010, in collaboration with The University of Toronto, PRF funded the creation of a cutting-edge new research technology, expanding the PRF Cell & Tissue Bank to include Progeria "Induced Pluripotent Stem Cells" (iPSC). These cells are created from mature Progeria cells, such as skin cells or blood cells, which we already have in the PRF cell bank. The mature skin or blood cells are reversed in the laboratory to become Progeria stem cells. From this point, scientists can choose to explore stem cells in Progeria, or they can use these iPSC to create cells that are important for understanding heart disease in Progeria such as cells of the blood vessel wall, which are difficult to obtain by any other means. By using the bank's iPSC, scientists have a new tool for making new discoveries in the fields of Progeria and aging.



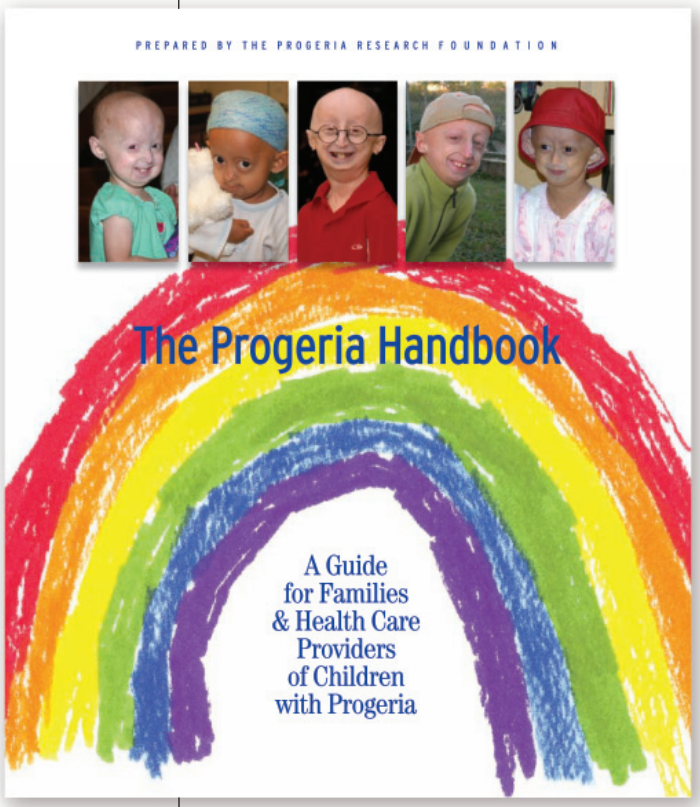
Progeria cells as seen in the laboratory.

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

- **Stephanie**, mother of Cameron, pictured at left with his parents and big sister. Cam was diagnosed in 2007 and is now enrolled in all of PRF's programs, including the clinical drug trials.

The PRF Medical & Research Database

This essential resource was developed in 2002, in collaboration with the Brown University Center for Gerontology and Healthcare Research. The program requires collection and analysis of medical records and radiology scans such as X-rays and MRIs, from individuals with Progeria worldwide. The data in the records is rigorously analyzed (and translated, if necessary) to understand clinical disease in Progeria, and inform general treatment guidelines to help optimize the children's quality of life. Importantly, the program also includes a "weighing-in" component where children are weighed routinely at home and report weights to program staff. These weights are used to prepare data for the clinical trials.



Progeria Healthcare Handbook published. 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 Medical & Research Database was vital to this handbook, as was the clinical expertise of all Progeria experts from Children's Hospital Boston who contributed chapters to the book.

In 2010, this Handbook has been distributed to nearly 250 families, physicians, teachers and others. Also in 2010, we began translating the handbook into Spanish, and we plan to make this resource available in other languages in the future as the global presence of PRF expands.

"We received our Handbook, read it cover to cover and absolutely love it - Thank you for providing so much important and helpful information!"

- From the parents of a child with Progeria

"The Handbook is phenomenal."

- From a family physician of a recently-diagnosed, 6-month-old girl

Workshops on Progeria

Since 2001, PRF has conducted six successful scientific conferences, all with support from the National Institutes of Health and The Ellison Medical Foundation. The depth and breadth of research into Progeria grows stronger with every meeting, as the number of participants and countries from which they originate increases each time. PRF is committed to this process of global medical collaboration.

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 gathering to hear expert oral presentations and view nearly 40 poster presentations. Doctors and scientists - who often work in separate worlds in the clinic or in the lab - inspired one another as they came together to share cutting-edge findings and collaborate on directions for future research. Speakers included leading scientists in the fields of heart disease, aging, genetics, and lamins.

"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.

Partnership with the National Institutes of Health (NIH)

PRF's early efforts led to first-ever NIH Progeria funding in 2002, through the National Institute on Aging. Since then, the PRF/NIH collaboration has resulted in government support that includes co-sponsorship of all six PRF scientific workshops, a natural history study of Progeria conducted in 2005-2006 at the NIH Clinical Center and published in the New England Journal of Medicine, and a 2009-2011 grant from the National Heart, Lung and Blood Institute for $3.1 million towards funding the Progeria Triple Drug Trial. Moreover, PRF's Medical Director Leslie Gordon is a frequent presenter for conferences sponsored and conducted by the NIH Office of Rare Diseases, where she discusses PRF's programs, how they have helped advance the field of Progeria, and how they may inspire other organizations. NIH Director Dr. Francis Collins often cites the Progeria story of progress from bench to bedside as an example of how a rare disease can be propelled towards cure when there is a non-profit group like PRF effectively collaborating with NIH and others.

Three-year-old Zach says "hi" to a captive audience during the 2010 Progeria Workshop's family panel presentation, as proud dad Brandon looks on.

Scientific Progress through Publications – Reporting new discoveries to the world

We strive to promote awareness within the scientific community about the phenomenal progress being made in the field of Progeria research, in order to energize the scientific community to continue, or become involved, in the field. Interest in Progeria has soared 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 Progeria publications has increased exponentially! Many of these studies were conducted using PRF grant funding, Cell & Tissue Bank materials and Medical & Research Database data, and are published in well-known, respected scientific journals and medical textbooks read by researchers and doctors worldwide.

The most notable of the 2010 publications, led by PRF's Medical Director and reported in the Wall Street Journal, describes a landmark study demonstrating that the Progeria-causing protein *progerin* increases in everyone with age, suggesting a possible new risk factor for heart disease.[4]

PRF's critical impact on Progeria related research is well-documented. In 52 years, from 1950-2002, there were just 104 peer review publications on Progeria, averaging 2 per year. **In the seven years from 2003-2010, 209 articles were published – an average of 26 per year - with a record high of 36 in 2010. This represents an extraordinary average annual increase of over 1,300%.**

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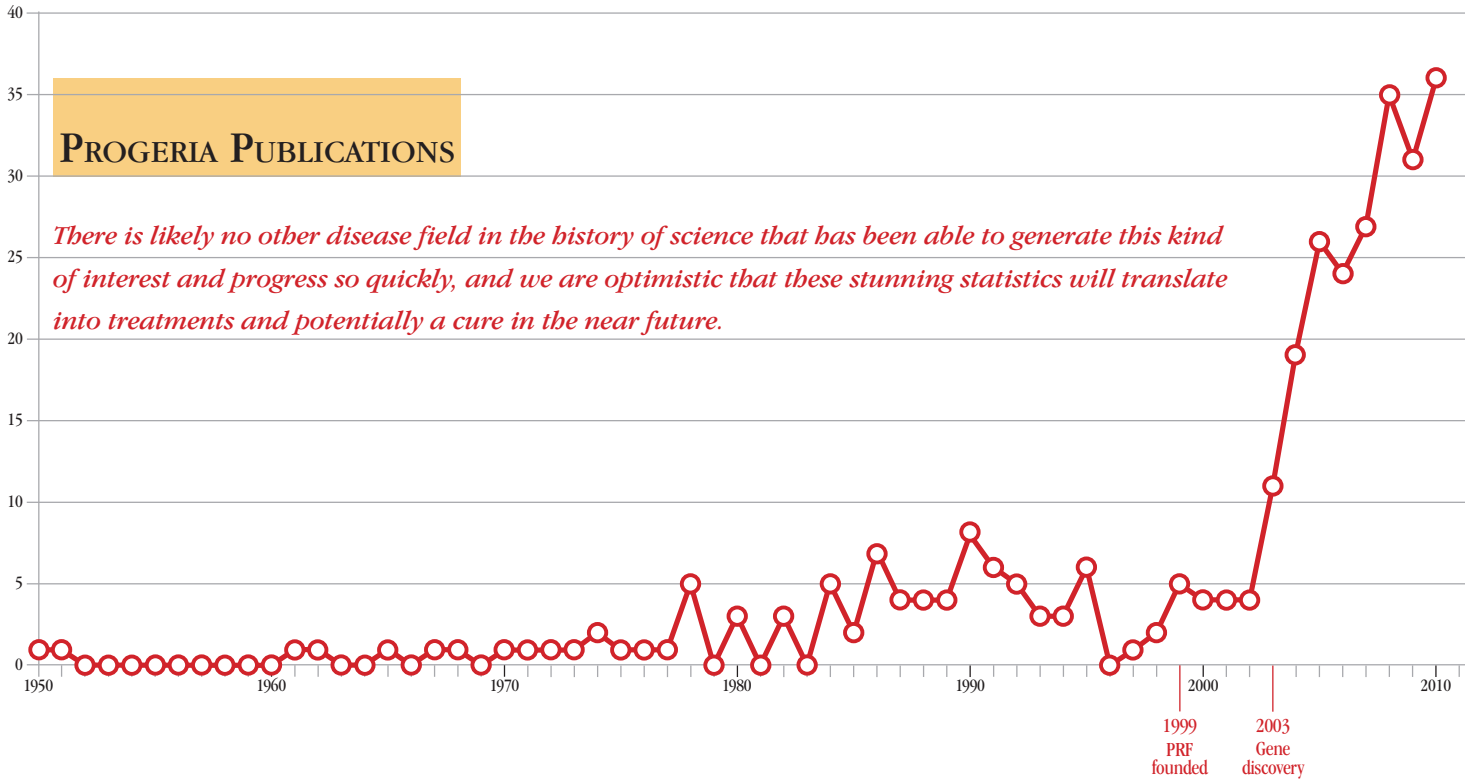
[4] Olive et. al., "Cardiovascular pathology in Hutchinson-Gilford progeria: correlation with the vascular pathology of aging." *Arteriosclerosis, Thrombosis, and Vascular Biology* 2010 Nov;30 (11):2301-9.



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 also help those with heart disease and other aging-related conditions – has reached millions through PRF's web site, newsletters, educational materials, and social and traditional 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.

In 2010, popular shows The Dr. Oz Show and 20/20 featured Progeria, and filming for future programs on Discovery and HBO took place.

As awareness continues to grow, more children come to PRF for diagnosis and care; more researchers apply for grants and cells to support their work; more scientists participate in PRF's workshops; more resources are needed to support this growth; and more individuals and organizations offer their support.

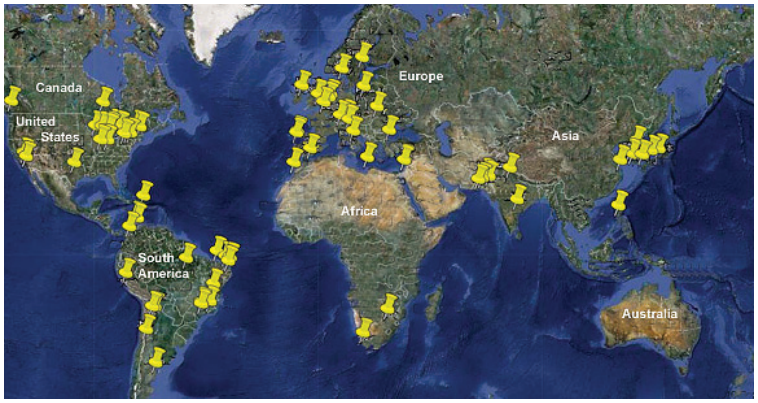
To Help The Children We Must Find The Children: At the end of 2009, and in response to the recognition that there were approximately 150 additional children living with Progeria that PRF had not found, PRF began a partnership with the worldwide health communications group GlobalHealthPR.[5] The partnership created an international awareness campaign called ***Find the Other 150***. Its goal: search globally for children with the extremely rare disease Progeria, so they would be able to receive the unique care they need and to help advance clinical science for Progeria. Through proactive outreach and a dedicated web site with information in six languages (*www.findtheother150.org*), this campaign – launched in the Americas, Europe, Russia, Asia and Africa – is succeeding far beyond our expectations.

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

Both girls are featured in a TLC documentary, "6 Going on 60", that premiered in December 2009 and re-aired frequently in 2010 due to its popularity, and a Barbara Walters, one-hour 20/20 special that aired in September 2010.

[5] Led by long-time PRF pro bono supporter Spectrum of Washington, DC, GlobalHealthPRF is the largest organization dedicated exclusively to healthcare communications worldwide.

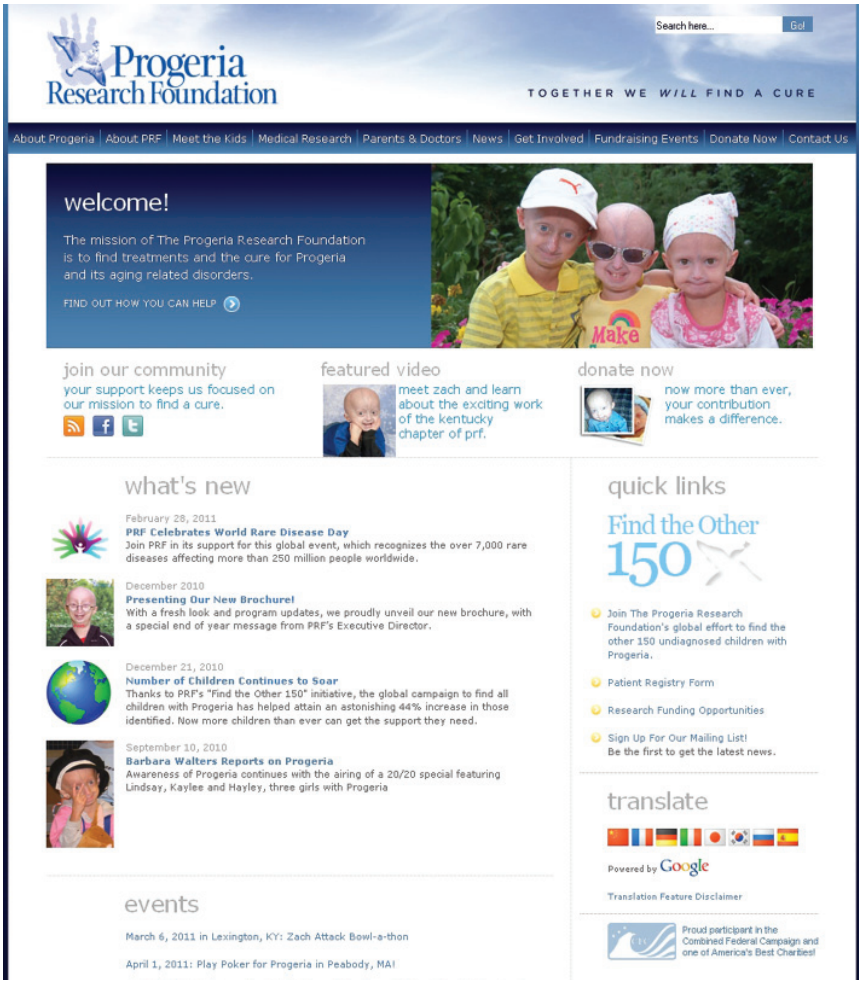
We are Finding More Children Than Ever Before: Throughout 2010, Find the Other 150 drove the search for previously unidentified children with Progeria worldwide, resulting in an unprecedented jump from 54 to 78 children – a 44% increase. The most recent 28 children live in Brazil, China, Colombia, India, Ireland, Japan, Korea, Mexico, Pakistan, Philippines, South Africa, and the United States.



Children with Progeria living around the world as of December 2010

In 2010 PRF also unveiled a new logo and a fresh look on our upgraded website, which now features rolling photos and videos. Our increased usage of social media is consistent with our burgeoning global influence. The ever-growing Facebook and Twitter presence to support worldwide awareness of Progeria – which increased in followers and fans three-fold in 2010! – is part of our efforts to communicate comprehensive, up-to-date news to the widest audience possible.

Progeria Research Foundation



Volunteers

PRF is incredibly fortunate to have such numerous and wonderful volunteers!* Students hold school events, companies have dress down days, athletes run marathons, relatives of children organize golf tournaments, dozens place Coins to Cure Progeria© cans in stores – the list of PRF's "Miracle Makers" goes on and on, and grows each year – look for them in our newsletters!

In 2010, many of PRF's dedicated chapters and other volunteers saw significant increases in fundraising revenue, with a total of over $600,000 raised by special events – a 20% increase from 2009.

The second annual *ONEpossible Campaign*, is an incredible example of PRF volunteerism. Led by 10 teams with a total of 59 people, the 2010 ONEpossible campaign raised $182,000 (a $75,000 increase from the year before) in just six weeks! With nearly 900 contributors (738 of whom were first-time donors), and most donations under $100, PRF supporters demonstrate once again that they are each ONE who will make a cure POSSIBLE.

PRF in 2011 and Beyond

In the years to come, we intend to live up to our reputation of rapid and considerable accomplishments, including the following:

- Continue to forge ahead to discover whether the current trial treatments are working to help the children.
- Work towards new treatments and treatment trials for children with Progeria.
- Continue our efforts to promote drug discovery and partner with drug companies, to develop potential treatments and the cure for Progeria.
- Expand the *Find the Other 150* Campaign globally, to increase awareness of Progeria so more children will be found and helped by PRF.
- Increase the number of participants in the International Registry, Cell & Tissue Bank and Medical & Research Database projects, so that scientists can continue to 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.
- Enlarge our volunteer base and revenue sources to support and expand PRF's programs.

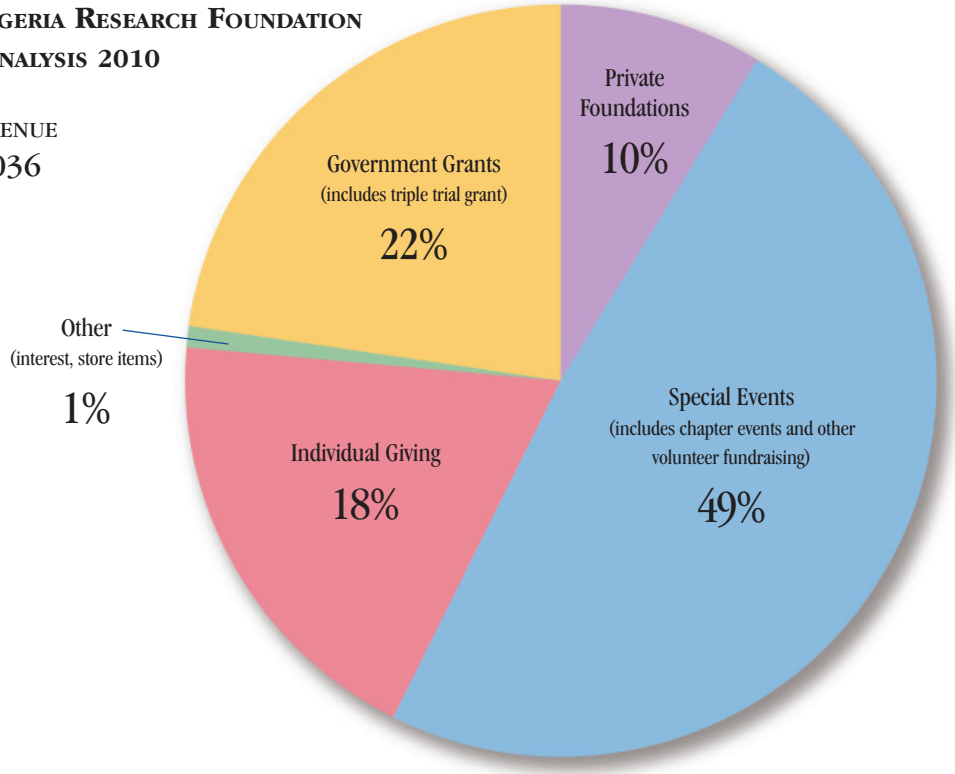
onepossible™

The Progeria Research Foundation

* 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 at no cost to PRF, to ensure less is spent on administrative costs and more on raising awareness, research, and finding a cure for Progeria. Our administrative/fundraising expenses are always 15% or below of total expenses.

The Progeria Research Foundation
income analysis 2010

TOTAL REVENUE
$1,736,036

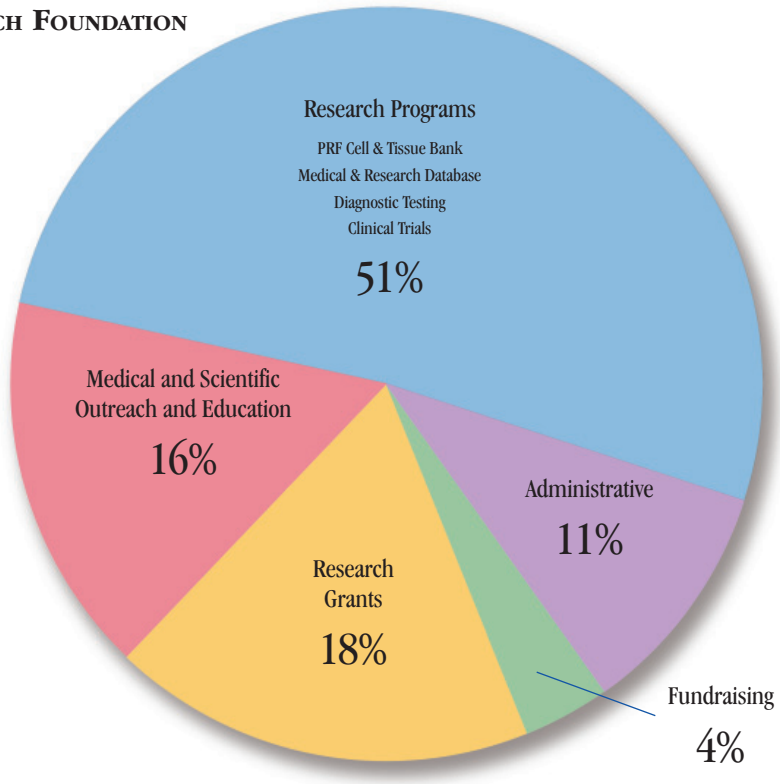


The Progeria Research Foundation
expense analysis 2010

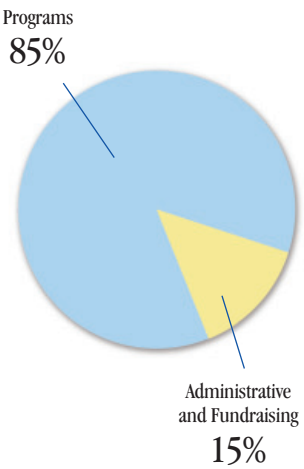
TOTAL EXPENSES
$1,241,035

NET ASSETS
$2,827,083

Much of these funds are designated for anticipated, future clinical trials and drug development costs



Expense Summary



"A year of tireless fight is gone, a year during which the Progeria kids have demonstrated that they are heroes... I want to tell you how happy we are for being part of this fight, part of The Progeria Research Foundation family, that day after day works for our son Mateo."

(Translated from Spanish)

- Sent to PRF December 2010 from the parents of 8-year-old Mateo from Argentina, who is enrolled in the Progeria Triple Drug Trial. Mateo and his parents were in Boston in August 2010 for his one-year trial visit.

Progeria Research Foundation

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