



The Progeria Research Foundation

www.progeriaresearch.org

August, 2009

news

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BACK COVER

Moving Ahead at Lightning Speed: New Progeria 'Triple Drug Trial' Begins!

As the first Progeria clinical trial draws to a close, PRF is funding continued drug treatment at Children's Hospital Boston.

In August, 2009, The Progeria Research Foundation and Children's Hospital Boston began a three-drug combination treatment trial. Researchers have identified two additional drugs that, when used in combination with the current FTI drug being tested, may provide an even more effective treatment for children with Progeria than FTI's alone.

See page 4 for details on this exciting and much larger trial that will include up to 45 children from 19 different countries.



Adalia, age 2, and Mom Natalia share a special kiss. Adalia was one of the first children to receive the new triple drug therapy.

PRF's Mission:

To discover the cure and effective treatments for Progeria and its aging-related disorders.

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Web Site:

Karen Gordon Betournay
Coordinator
Spectrum
Technical/Design

Graphic Designers:

Julie Pritchard
JCR Design, Foxboro, MA
Marie Migliaccio, Topsfield, MA
Regina Printing, Belleville, NJ

Accounting:

Carl Alviti, Matrix Financial, LLC,
Wellesley, MA

Progeria is a fatal, "premature aging" disease that afflicts children, who die of heart disease at an average age of 13 years - the same heart disease that affects millions of normal aging adults.

Finding the cure will help not only these special children, but perhaps also millions who suffer from heart attacks, strokes and other aging-related conditions.

Message from the President & Executive Director

On June 9th, 2009, I quietly celebrated ten years since PRF's very first board meeting. I found myself vividly remembering that gathering at my sister and brother-in-law Leslie and Scott's house, my notes in hand and my nerves in turmoil! It was a magical meeting, full of hope, determination and, of course, infinite passion – all of which remain the vital ingredients driving PRF's mission today.

Knowing this milestone will be shared later in 2009 with hundreds of supporters at the Night of Wonder and tens of thousands more through email blitzes, press releases, Facebook and our web site, I took some time to reflect privately on the remarkable journey so many others and I have been part of.

In just ten years, since we founded PRF in the face of no resources and no hope for the children, we have gone from gene finding to the first clinical trial in Progeria, and now a second drug trial has begun. We are now being hailed as a model for disease-research organizations and a prime example of successful translational research, moving from the lab to treatments at a pace virtually unheard of in the scientific community. And along the road towards helping to save the lives of children with Progeria, we've discovered brand new clues to heart disease and aging that affect us all. How absolutely astounding!

A mom recently wrote to me, "Sometimes I wonder how you continue to be motivated after 10 years... then I get a chance to talk to people who just want to help... and I get it." She's right on – compassion is tremendously inspiring!

My motivation comes from my nephew Sam and all children with Progeria, the families who put their trust in us to help their children, and the 10,000 of you who support PRF's work in dozens of ways. It is all of that – all of YOU – who will lead us to the cure.

Happy Anniversary to us all,

Audrey Gordon
President & Executive Director

Update on the First-Ever Progeria Drug Trial: We're almost there!

This first-ever Progeria drug trial began in May 2007 and involves 28 children from sixteen countries, ages 3 to 18 years. Beginning August 10th, children will spend a week at Children's Hospital Boston, undergoing tests for both their final, 2-year visit and their first visit as part of the 2-year Triple Drug Trial.



Sam Berns is all smiles as he receives his trophy for completing the First-Ever Progeria Trial this month.

By December 2009, all currently enrolled children will have completed their hospital visits, and trial results are expected in 2010. These courageous families have traveled to Boston 7 times from as far away as India, Israel, Pakistan and Japan to join the fight against Progeria.

"Kids Helping Kids"

In the search for markers of disease and disease improvement in Progeria, researchers must compare various physical aspects of children with Progeria and "control" children who do not have Progeria. At Rhode Island Hospital in Providence,

RI, volunteers donated blood and urine to help complete one control study. And at Children's Hospital Boston, members of the clinical trial team worked with 58 children to look at normal pediatric structure of bones and blood vessels. By comparing these studies of age and gender matched control children, researchers will understand how the blood vessels and bones of children with Progeria are affected by the disease, and measure whether drug treatments have an affect on disease status.



Sam's cousin Syetta (and her monkey) look on while giving blood for the Progeria control study at Rhode Island Hospital. Dr. Jim Linakis made the experience painless.

Clinical Trial Campaign: 95% There and Counting Down

Thanks to the tremendous response to our 1st Annual ONEpossible™ Campaign (see page 10) and many other generous donations directed to the first-ever Progeria trial, we have raised \$1.9 of the \$2 million trial campaign goal – **AMAZING!**



Jason, 8 years old, undergoes a carotid duplex ultrasound at Brigham and Women's Hospital to measure his major arteries as Drs. Marie Gerhard-Herman and Leslie Gordon review the results.

Announcing The Progeria Triple Drug Trial

Scientific Basis for Treatment

Progeria is caused by an abnormal protein named progerin. The Progeria research team at Children's Hospital Boston will add two drugs, called pravastatin and zoledronate, to the current treatment with FTI. **Strategy:** All three drugs will target different points along the pathway leading to production of the disease-causing progerin. In exciting laboratory studies presented by Dr. Carlos Lopez-Otin of Spain at the 2007 Progeria Research Foundation Scientific Workshop, the two new drugs improved disease in Progeria cells and extended lifespan in mouse models of Progeria*. **Goal:** If the three drugs administered in this trial can effectively block this farnesyl group attachment, then progerin may be "paralyzed" and Progeria may be improved. We hope that the drugs will work as partners, to complement each other so that the progerin protein is affected more by combining the three drugs than using any one drug alone.

Who Will Enroll In The Triple Drug Trial?

The Feasibility Trial: The team has already conducted a mini-trial for 5 children with Progeria. The short, one month "feasibility" trial asked whether the three-drug combination would be well-tolerated, prior to embarking on a larger international trial. Side effects were acceptable, and the team has moved ahead to the larger efficacy trial.

The Efficacy Trial: We anticipate up to 45 children will enroll in this trial, from 19 different countries, speaking 11 different languages. This includes children participating in the FTI-only trial, the 5 in the feasibility trial, and other children that were either too young to participate in the first trial or children that we've discovered over the past 2 years. Children currently enrolled in the FTI-only trial will have the opportunity to enroll in the triple trial when they arrive for their last visit for the current trial. This allows the children to continue taking FTI without any missed doses.

The Treatment/Progeria Relationship

How did we get from gene discovery to drug therapy for children with Progeria? Finding the gene for Progeria was the key. This gene is called *LMNA*, and it normally encodes a protein called prelamin A (this protein is further processed and becomes lamin A). Children with Progeria have a mutation in *LMNA* which leads to the production of an abnormal form of prelamin A called "progerin." Many years' worth of basic research on prelamin A and lamin A gave us the ability to understand that the drugs administered in this trial may affect disease in Progeria. Over the past six years, research has focused on systematically testing these drugs on Progeria cells and Progeria mice.

The Clinical Trial Team

Since May 2007, a 28-member team has treated children with Progeria from around the globe. Members of the team have expertise not only in Progeria, but also in the three drugs administered in this trial.

Trial Medications At a Glance

Pravastatin (marketed as Pravachol or Selektine) is a member of the drug class of statins. It is usually used for lowering cholesterol and preventing cardiovascular disease.

Zoledronic acid is a *bisphosphonate*, usually used as a bone drug for improving osteoporosis, and to prevent skeletal fractures in people suffering from some forms of cancer.

Lonafarnib is an *FTI* (Farnesyltransferase inhibitor), a drug that can reverse an abnormality in Progeria cells in the laboratory, and has improved disease in Progeria mice.

All 3 drugs block the production of the farnesyl molecule that is needed for progerin to create disease in Progeria.

Timing

Patients will travel to Boston for testing and examinations lasting 4-7 days, every 6 months for a period of 2 years. For the FTI-only trial, Boston visits occur every four months.

Cost:

We estimate the trial will cost PRF nearly \$1.7 million over a 2 1/2-year period for drugs, clinical testing, translators, travel, food, lodging and staff.

* *Combined treatment with statins and aminobisphosphonates extends longevity in a mouse model of human premature aging*, by Varela et. al. *Nature Medicine*, 2008. 14(7): p. 767-72.



Cam and Miles, age 2

This is the new generation of children PRF is helping...

Adalia, age 2



"Paving the Way"

In March, 2009, these five children participated in a one-month feasibility study to determine if the side effects of the three drugs taken together were tolerable. The results were positive, paving the way for the full, two-year, Triple Drug Trial to enroll up to 45 children with Progeria. **Hats off to these amazing families!**

"EVERYONE has been so wonderful. To us you are ALL GOD SENT and we APPRECIATE all that you do for these little angels. Our family is so overwhelmed with excitement and all sorts of emotions with Adalia's trip to Boston this weekend, I can't even begin to type the words of how we are feeling."

- Adalia's grandmother; just before Adalia's first trip to Boston for the 1-month tolerability trial.

Nicholas, age 3



"Thank you all at PRF so much for all you have done! We would have been lost in a world of confusion and grief without you. Instead, we live in a world of hope and purpose. Thank you again and again! With much love and respect."

- Family of Cam, another new triple trial patient.



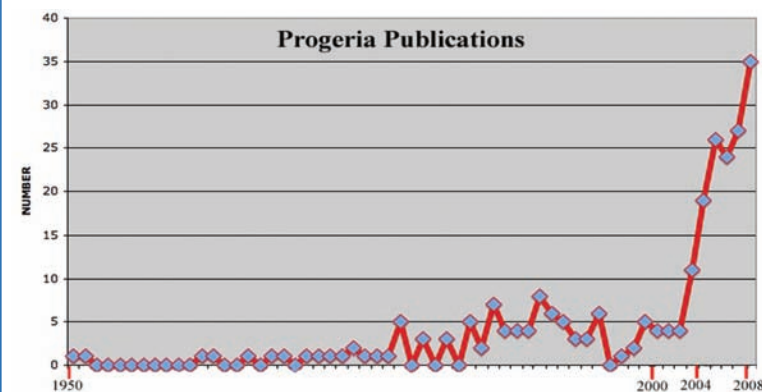
Zach, age 2

Refresher: What is a Clinical Trial?

A clinical drug trial is a type of research study designed to determine if a new drug is safe and effective in humans. To learn all about clinical trials, go to www.clinicaltrials.gov

What's News in Progeria Research? Plenty!!

The number and variety of scientific publications on Progeria continue to grow. There were a record 35 articles in 2008. This chart shows a 1,000% annual increase in the 6 years since the Progeria gene discovery, compared to the 53 years prior – WOW!



Swedish team's new findings strengthen our understanding of the connection between normal aging and Progeria*

Dr. Maria Eriksson, author of the Progeria gene discovery in 2003, has now invented a new, powerful technique to quantitatively measure the expression of the Progeria gene. Dr. Eriksson's laboratory at the Karolinska Institute in Sweden used the technique to measure the amount of progerin RNA in both normal and Progeria cells. RNA is the blueprint molecule in our cells for making protein. The Swedish group found that both normal and Progeria cells make larger and larger amounts of progerin RNA as they age. Eriksson's result shows that the RNA signal for making progerin quickly builds in the cells of children with Progeria, and builds slowly over a lifetime in us all. The new technique is expected to be widely used in experiments that address the mechanism of progerin action.

Increased expression of the Hutchinson-Gilford Progeria syndrome truncated lamin A transcript during cell aging, by Rodriguez S, Coppède F, Sagelius H and Eriksson M, *Eur. J. Hum. Gen.* (2009),1-10.

In a second article, Dr. Eriksson's group examined the expression of lamins A and B in different types of skin cells and during the hair cycle. The research team found strong production of these proteins at all stages of the hair cycle. Comparisons with normal and Progeria mice tell us more about how hair and skin are affected in Progeria and normal aging.

Differential expression of A-type and B-type lamins during hair cycling, by Hanif M, Rosengardten Y, Sagelius H, Rozell B, Eriksson M, *PLoS One*. 2009;4(1):e4114.

Detailed Dental Analysis Emerges from NIH Natural History Study

From 2005-2006, PRF collaborated with the National Institutes of Health on a natural history study of Progeria. Fifteen children flew to NIH for clinical examinations, as the research team studied all organ systems affected by the disease. Novel oral-craniofacial phenotypes and quantification of previously reported features were examined. This detailed analysis of dental characteristics of Progeria, led by **Dr. Thomas Hart's dental team**, helps us to understand much more about how Progeria affects the mouth structure and function.

Hutchinson-Gilford progeria syndrome: oral and craniofacial phenotypes, by Domingo DL, Trujillo MI, Council SE, Merideth MA, Gordon LB, Wu T, Introne WJ, Gahl WA, Hart TC. *Oral Dis*. 2009 Apr;15(3):187-95

PRF Research Grantees Devise New Method to Study Progerin in Cells

Dr. Stephen Young's laboratory at UCLA has set up an experimental system in which the amount of progerin in normal cells can be increased or decreased. This method will allow investigators to sort out the direct effects of progerin from secondary ones, and better understand the damage caused by varying amounts of progerin in cells.

Activating the synthesis of progerin, the mutant prelamin A in Hutchinson-Gilford progeria syndrome, with antisense oligonucleotides, by Fong LG, Vickers TA, Farber EA, Choi C, Yun UJ, Hu Y, Yang SH, Coffinier C, Lee R, Yin L, Davies BS, Andres DA, Spielmann HP, Bennett CF, Young SG. *Hum Mol Genet*. 2009 Jul 1;18(13):2462-71.

Article breaks new ground on HGPS affect on cellular functions

Progeria has previously been shown to affect many fundamental cellular functions including replication, gene expression, and DNA repair. **Dr. Hubner** and coworkers from Germany's University of Würzburg, Institute of Anatomy and Cell Biology have added the transport of proteins from the cytoplasm (outside of the nucleus) into the nucleus (which contains our DNA) to this list.

All proteins are synthesized in the cytoplasm, and those that end up in the nucleus have to get across the nuclear membrane. The transport is accomplished through channels in the nuclear membrane called "nuclear pores". Many proteins are too large to simply diffuse through the nuclear pores, but are "ushered" through them by special proteins that have evolved for this purpose. Dr. Hubner's group shows that Progeria cells have globally decreased protein transport from the cytoplasm into the nucleus.

Nuclear protein import is reduced in cells expressing nuclear envelopathy-causing lamin A mutants, by Busch A, Kiel T, Heupel WM, Wehnert M, Hubner S. *Exp Cell Res*. 2009 Aug 15;315(14):2373-85.

Italian Researchers Study Lamin A Biochemistry

Progerin is the abnormal protein that causes Progeria. Its normal protein counterpart is lamin A. Both progerin and lamin A go through a series of processing steps in order to function. Current treatment trials for progeria are based on an understanding of how we can affect progerin processing.

Dr. Giovanna Lattanzi and her research group from the University of Urbino, Italy have better defined these processing steps, and the protein forms produced by cells under several different conditions.

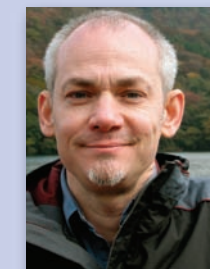
Different prelamin A forms accumulate in human fibroblasts: a study in experimental models and Progeria, by Dominici S, Fiori V, Magnani M, Schena E, Capanni C, Camozzi D, D'Apice MR, Le Douar C, Auclair M, Caron M, Novelli G, Vigouroux C, Maraldi NM, Lattanzi G, *Eur J Histochem* 2009 53(1):43-52.

26 Grantees and Counting: PRF Awards Two More Research Grants

William L. Stanford, PhD, University of Toronto, Canada

Induced-Pluripotent Stem Cells (iPSC) from HGPS patient fibroblasts to elucidate the molecular mechanism associated with diminishing vascular function

iPS cells, or Induced pluripotent stem cells are cells that started out as a mature cell type easily obtained and grown in the laboratory, and are treated with biochemical "cues" that signal the cells' genetic machinery to turn them into immature stem cells. These stem cells are then given additional biochemical "cues" to mature once again, but not into their original cell type. For example, a skin cell (mature) can be first turned into a stem cell (immature) and then turned into a vascular cell (mature). This cutting edge technology is intensely important for Progeria research, where we cannot obtain live human blood vessel, heart and bone cells of children with Progeria for study. Dr. Stanford will develop multiple Progeria iPSCs to model Progeria vascular disease stem cells, in order to study heart disease in Progeria in brand new ways.



Dr. Stanford

Dr. Stanford is a Canada Research Chair in Stem Cell Bioengineering & Functional Genomics, and Associate Professor & Associate Director of the Institute of Biomaterials & Biomedical Engineering at the University of Toronto. He is also the co-Scientific Director of the Ontario Human iPSC Cell Facility.

Jakob Tolar, University of Minnesota, Minneapolis, MN

Correction of human progeria induced pluripotent cells by homologous recombination

Dr. Tolar's lab has shown that allogeneic cellular therapy with mesenchymal stem cells can prolong survival in the Progeria mouse model, suggesting that cellular therapy can be of benefit to children with Progeria. However, the children have abnormal DNA repair and as such are expected to experience significant toxicities with the chemoradiotherapy needed for engraftment of cells from unrelated donors. Therefore, Dr. Tolar will limit such toxicity by developing genetically corrected cells from the Progeria children themselves, combining the novel concept of iPSCs from Progeria patients with the emerging technology for gene correction mediated by zinc finger nucleases. In this manner he aims to establish a platform for clinical translation of safer stem cell gene therapy with progeny cell types of iPSCs as a definitive treatment for children with Progeria.



Dr. Tolar

Dr. Tolar is an Assistant Professor and attending physician at the University of Minnesota in the Divisions of Pediatric Hematology-Oncology and Pediatric Blood and Marrow Transplantation. Dr. Tolar's research focuses on the use of bone marrow-derived stem cells and gene therapy for correction of genetic diseases and improving outcome of blood and marrow transplantation.

Progeria and PRF: Hot Topics at National Meetings

Word of the impressive advancements in Progeria research continues to spread throughout the scientific and general community. As a result, speaking invitations from prestigious organizations to PRF's leadership have helped reach thousands who attend to learn more about Progeria and PRF's work.

PRF's Chairman Dr. Scott Berns Speaks at Milken Global Conference



Scott Berns makes a point, as fellow panelist Dr. Pinchas Cohen of UCLA Children's Health Center listens.

Beverly Hills, CA: This annual event brings together 3,000 extraordinary people – Nobel laureates, CEOs from the world's top companies, first-rate journalists, and leaders in education, health care and philanthropy – to discuss today's most pressing challenges. Dr. Berns participated in a fascinating panel discussion on longevity, bringing a scientific perspective of the link of Progeria research to normal aging. He received a rousing applause from the audience after describing PRF's progress toward a cure for Progeria.

Many thanks to panel moderator and Milken Institute senior fellow Howard R. Soule, PhD, for inviting Dr. Berns to speak. "Nothing that I am aware of in the history of modern medicine comes close to a trial of this complexity, with children coming from all over the world", Dr. Soule remarked toward the end of the presentation, "Hats off to you and your foundation."

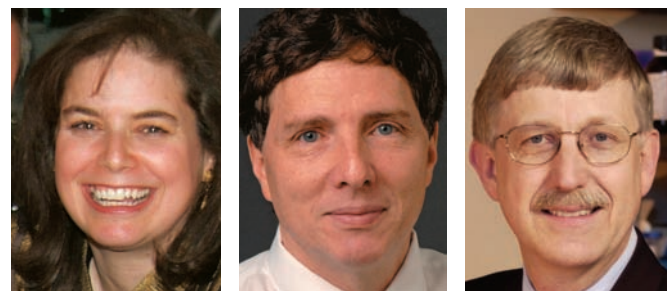
PRF's Medical Director Dr. Leslie Gordon Addresses Rare Disease Community at NIH Meeting

Bethesda, MD: Dr. Leslie Gordon gave a captivating presentation on how successful collaborations have helped PRF move from clinical research to clinical practice. "In ten years we have gone from obscurity to drug trial, and at every step along the way we have partnered with others who have helped make such rapid progress possible."



Dr. Steve Graft, Director of the Office of Rare Diseases at the National Institutes of Health, invited Dr. Gordon to speak at the "Advancing Rare Diseases Research through Networks and Collaboration" conference.

PRF's Medical Director Speaks at ASCB Meeting with NIH Director Nominee Dr. Francis Collins and Progeria Trial Investigator Dr. Mark Kieran



Dr. Leslie Gordon

Dr. Mark Kieran

Dr. Francis Collins

San Francisco, CA: The American Society for Cell Biology (ASCB) is the world's largest professional organization advancing cell biological research. At its 48th annual meeting in December 2008, this prestigious panel of Progeria researchers conducted a special session detailing the story of Progeria research advances. The one hour session, entitled "Translating Progeria: A Bench-to-Bedside Story" was chaired by ASCB President and pioneering nuclear lamin researcher Dr. Robert Goldman. It was attended by 1,000 scientists.

April 2010: PRF To Hold Its 6th International Workshop on Progeria "From Bench to Bedside in a Decade"



With the expected presentation of the first-ever Progeria clinical trial results, this 6th Progeria scientific meeting promises to be the most exciting and informative conference yet.

The meeting will promote scientific discussions within formal and informal settings. The main focus is to bring basic and clinical science together, to promote collaboration and progress in Progeria research.

Organizers:

- Leslie Gordon, MD, PhD, Warren Alpert School of Medicine, Brown University
Medical Director, The Progeria Research Foundation
- Frank Rothman, PhD, Brown University
- Christine Harling-Berg, PhD, Brown University

Advisory Committee:

- Robert Goldman, PhD, Northwestern University
- George Martin, MD, University of Washington
- Tom Misteli, PhD, National Cancer Institute

Location and accommodations: Seaport Hotel, Boston, MA

Dates and times: Sunday evening, April 11, 2010 through Tuesday late afternoon April 13, 2010

Agenda: Plenary Session, Oral Presentations, Posters and a Progeria Family Panel

Poster presentations are encouraged. Several posters will be elevated to oral presentations.

Registration information and Abstract forms: <http://www.progeriaresearch.org/workshop2010.html>

Workshop coordinator: Susan Rosenblatt, workshop@progeriaresearch.org or 978-535-2594



Normal Nucleus HGPS Nucleus FTI-Treated Nucleus



2007 Workshop attendees

PRF's 1st Annual Campaign Surpasses \$100,000 Goal!!



With just 6 months left until the first-ever Progeria clinical drug trial was to be completed, our goal was to raise \$100,000 to help fund it. Mission accomplished! From February 1 to March 31, over 800 people donated anywhere from \$10 to \$5,000, raising an **astonishing \$107,000 – amazing!**

We know these are tough financial times, so it is especially heartwarming that so many donated. It all made a difference – \$25, \$30, \$50 or more – every ONE of you and your donations got us to goal.



Thank you to campaign chair Kim Paratore, and to all the teams who worked so hard to make this event a success



Wendy Nelson and Paula Kelly smile triumphantly after completing the 2009 Boston Marathon and raising nearly \$10,000 for PRF! They are surrounded by their children, and in the middle is their neighbor Sam Berns.

TEAM BALLACK, led by PRF Board member Karen Ballack from California

TEAM BRESNAHAN, led by PRF Board member Chuck Bresnahan and his daughters from Ohio

TEAM FACEBOOK, for all those who have Facebook pages and wanted to show how powerful the online world can be in making the dream of a cure POSSIBLE.

> PRF now has Facebook Cause and Group pages – join today!

TEAM FOOSE, led by Terry Foose from California, in loving memory of her daughter Amy

TEAM KUSHNER, led by Ariel Haber Kushner in loving memory of her brother Aaron

TEAM NIGHBOR, led by PRF's Ambassador family with Megan Nighbor

TEAM PARATORE, led by PRF Board member and campaign chair Kim Paratore from Massachusetts

TEAM PONN, led by long-time PRF supporter and former Night of Wonder chair Debbie Ponn from MA

TEAM SCHOONOVER, led by Cam's grandparents from Pennsylvania

TEAM ZACH ATTACK, led by Zach's parents from Kentucky

TEAM BOSTON MARATHON, led by the committed athletes who ran in this year's marathon

Thank you for being ONE who will make a cure POSSIBLE

Check out these statistics!

Number of Donors: **845**

New Donors to PRF: **636**

Average Donation: **\$96.21**

Most Common Donation: **\$25**

announcing night of wonder 2009!

tickets are now available at \$200 participant level and \$300 benefactor level...
 be a corporate or individual sponsor – levels from \$2,500 to \$20,000 and come with great “perks”...
 place an ad in our beautiful and well-distributed program book – ads range from \$100 to \$1,000...
 donate an auction item... so many ways to ride the wave!



Surf's Up
 for PRF's 10th anniversary
night of wonder 2009!

it's time once again for PRF's amazing, signature gala – you won't want to miss it...!

saturday, november 14, 2009 at 6:30 pm
 the royal sonesta hotel, cambridge, massachusetts



this beach party promises...

top-down cruising through silent and live auctions with paul zekos, auctioneer...
 surfside picnic with gourmet food and umbrella drinks – try our special “bikini-tinis”!...
 dancing “inside the curl” to music by the zaitchik brothers... and much, much more!

call 978-535-2594, email prfnw09@yahoo.com or visit progeriaresearch.org today!

PRF ON THE MOVE!

PRF Comes to Chicago!

It was a night to remember in the windy city on June 8th, with 85 guests attending a champagne reception for The Progeria Research Foundation at the home of hosts **Linda and Bob Mendelson**. Linda and Bob learned of PRF's work through attending the most recent two *Night of Wonder* galas co-chaired by their daughter Debbie Ponn, and wanted to do something to bring awareness to their hometown of Chicago.

In addition to heartfelt speeches by PRF's Chairman of the Board Scott Berns, Executive Director Audrey Gordon, Linda, Bob and Debbie, the captivated audience listened to two honored guests:

- Dr. Robert Goldman, distinguished researcher from around the corner at Northwestern University's Feinberg School of Medicine, and
- Rabbi Harold Kushner, founding PRF board member and renowned writer of inspirational books, the most famous being *"When Bad Things Happen to Good People"* written in 1981.

"I wrote that book because I made a promise to my son that he would be remembered," said Rabbi Kushner, "Who would have imagined the couple that started PRF 22 years later would live 20 minutes from my wife and me, and we would be so deeply involved in their efforts to find a cure for their son?"



(l to r) Scott Berns, Audrey Gordon, Dr. Goldman, Linda and Bob Mendelson, Rabbi Kushner and Debbie Ponn

Hear from the guests...

"Last night was really an over the top educational experience. Sad, informative, hopeful, warm, gracious, passionate...truly a lovely evening. I came away with hope – this grass roots effort seems to be gaining momentum."

"Many thanks for including me in such a wonderful event! The good spirits from the talks inspired all of us - what a wonderful set of speakers! Everybody who came wanted to help the cause in some way."

"What an inspiration. Working together, we can make a difference – and find a cure."

Baja Fresh Gives Fresh Hope to Children with Progeria

We are thrilled to announce a new corporate partnership with **Baja Fresh!** This month the popular chain of Mexican-inspired food restaurants kicks off a one-year fundraising and awareness campaign for PRF, collecting donations from customers at 85 stores nationwide, through coin boxes placed near the cash registers. For a list of participating locations, visit www.progeriaresearch.org.



"The Progeria Research Foundation is a cause we're proud to support," said Baja Fresh owner David Kim. Mr. Kim met PRF's board chairman Scott Berns in April at the Milken conference (see page 8) and, after Scott's stirring presentation, said, "You really moved me, and I want to help."

Such spontaneous generosity!

Public Service Announcement Airs Nationwide

Viamedia, the leading independent provider of Cable TV advertising sales, donated \$500,000 in advertising time for PRF's Public Service Announcement this spring. The PSA ran nationwide over a 3-month period on networks such as TLC, Discovery and Fox News.

Raising awareness about Progeria and PRF's work is one of our major goals, as we strive to find all children with this rare disease throughout the world, so we can help each and every one. *Thanks to partners like Viamedia, we will accomplish that mission!*

PRF Keeps Up with the "Social Media" Times

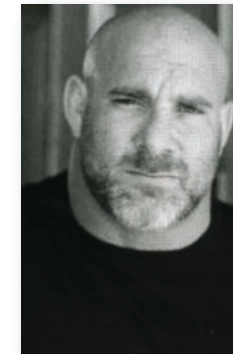
We're on **Facebook** and **Twitter!** Visit our Cause and Group pages on Facebook, featuring PRF's logo and named: *Support Progeria Research: The Official Cause /Group for The Progeria Research Foundation*. Combined, the pages have nearly 2,000 friends following discussions, events and the latest PRF news – Join in at http://apps.facebook.com/causes/183613?m=92eea645&recruiter_id=39018947

We're also Tweeting under the name "Progeria", so log in and Tweet us, too!



PRF is "Smarter than a 5th Grader" charity

On July 24, 2009, millions tuned in to the FOX channel as professional wrestler **Bill Goldberg** braved the classroom to test his knowledge of elementary school subjects on **ARE YOU SMARTER THAN A 5th GRADER? - Celebrity Charity Edition**.



Bill learned about Progeria and PRF's work through car designer guru Chip Foose and YearOne President Kevin King at the 2007 Braselton Bash car show supporting PRF. Shortly thereafter, he was approached by FOX and generously chose The Progeria Research Foundation to benefit from his appearance. How exciting to hear PRF's name on a national TV show!

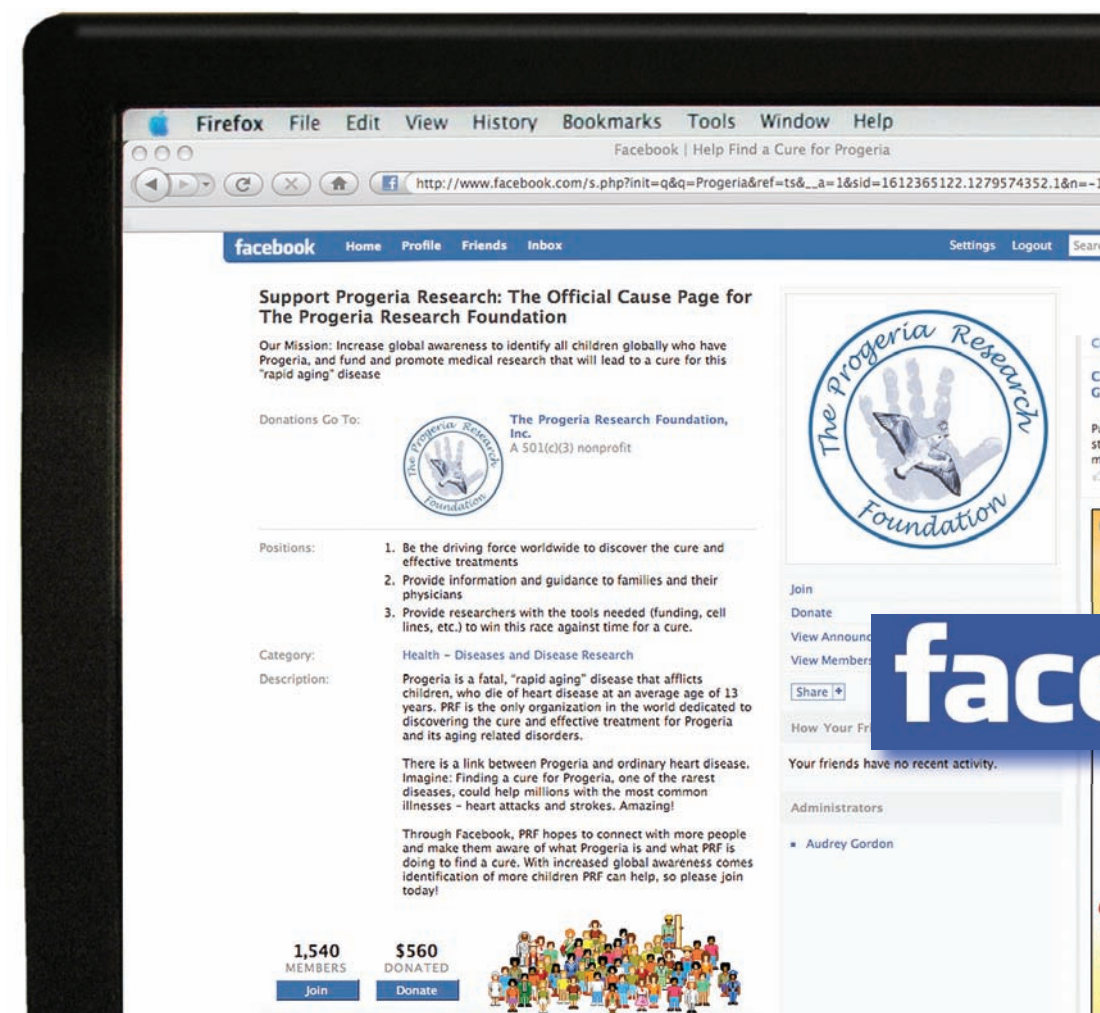
ARE YOU SMARTER THAN A 5th GRADER? is an award-winning, unique game show measuring adults' lack of knowledge as they tackle school subjects learned decades ago. They can "cheat" through advice from real grade-schoolers. Contestants compete for cash that is then donated to their chosen charity. PRF received \$25,000 - *Thank You Bill, we think you made the SMART choice to support PRF!*

New Charity Donate Games Supports PRF

In March, **Donate Games** was launched, its mission to support orphan disease research through "gamers" - players of video games. It was started by seasoned technology executive **Jim Carol** after his son **Taylor** was diagnosed with a rare form of Leukemia. Taylor made a full recovery, but he and his family were left with an unshakable urge to make a difference in the lives of others.

Donate Games collects and resells used video games from gamers, then donates the proceeds to organizations promoting research on rare diseases. The Progeria Research Foundation was chosen as one of the first to receive funding.

"With their thoughtful, planned vision of what is needed to propel the research forward, The Progeria Research Foundation has set the gold standard for what can be done when a family faces a rare disease," says Jim. "We hope by spreading the word to the gaming community, it will inspire others to support their work and promote research for rare diseases." *Thank you, Jim and Taylor - this is one game PRF is guaranteed to win!*



Annual Events

They get better and better every year

Our chapters, families and other volunteers continue to devote their time and energy to events that help support PRF's work, and new events start up each year. These FUNdraisers make it possible for us to forge ahead toward a cure – THANK YOU ALL!

Dozens Deal In for 4th Annual Poker for Progeria Tournament

One hundred Texas Hold 'em players gambled the night away in PRF's hometown of Peabody, Massachusetts, while dozens more came to enjoy the silent auction, raffles and Black Jack, raising nearly \$9,000.

In keeping with the gambling theme, our signature "Pocketbooks for Progeria" auction was a hit, where the high bidders on designer purses receive a surprise inside: lottery tickets, gift certificates, or cash. **SUCH A DEAL FOR ALL WHO CAME!**



These 3 men (pictured with their dealer) were all smiles as they picked up the top cash prizes.

A Father/Grandfather's Love Lights Up the Indiana Fairways

Chris Kempf is a busy man – not only does he run his chain of bakeries, but he also devotes dozens of hours each year to organizing the annual **Evansville Kennedy Golf Scramble** in honor of his daughter Cheryl and granddaughter Sarah. This year brought out a full roster



of players, raising \$14,000 and loads of local support. **CHRIS, YOU ROCK!**

Chris, Sarah and Cheryl together right before tee time.

Holy Cow – I mean Pelican! Pickard Pelican Run Raises \$24,000

Indiana's Brownstown Electric Supply Company (BESCo) once again organized the 2nd Annual **Zach Pickard Pelican Run** in May. 180 walkers and runners participated, and Zach was on hand to meet every one of them. The highlight of the race was when Zach crossed the finish line while everyone cheered him on.



Zach enjoys a photo opp with parents Tina and Brandon by the race's signature statue.

Determined to raise more than last year, they did so with some creative side events: selling angel food cakes and shaving their heads! "It seems in many ways the whole town is behind our efforts" says race organizer Brett Turner. We're not surprised, BESCo – your passion for children with Progeria is evident and contagious – **WE CAN'T WAIT TO SEE WHAT YOU COME UP WITH NEXT YEAR!**



11 members of BESCo's warehouse staff shaved their heads and raised \$1,230 – thanks for the sacrifice, guys



BESCo employees Diana Beedie and Debbie Sweeney teamed up to bake 178 angel food cakes as part of the Pelican Run, raising \$1,430 – now that's a lot of "dough"!

Talented Pinstripers Paint for Progeria

Pinstriping is a beautiful art form of fine lines, found mainly on cars and motorcycles. **The Pinstripers Reunion** is held annually at the Grand National Roadster Show in Pomona, CA, where show organizer and famous pinstriper Von Hot Rod invites the hottest pinstripers from around the country to showcase their art and participate in a charity auction. This is the 2nd year they have chosen to benefit PRF, thanks to the efforts of CA chapter president Terry Foose and her son, legendary car designer Chip Foose. They raised over \$13,000 this year – **NOW THAT'S A TRUE WORK OF ART!**

Miles for Miracles Continues its Miraculous Success

It was another banner year for the MI chapter as over 300 hundred people came out to walk for Progeria at the 4th annual event, raising \$28,000 – phenomenal! Kids had a blast with a balloon man and a "bubble station", chasing crazy-shaped bubbles around the park! "Once again it was a very family-oriented event and everyone really enjoyed themselves" said Lindsay's mom Kristy. **EACH YEAR, WITH EACH MILE, WE COME CLOSER TO THE MIRACLE OF A CURE!**

Lindsay and best friend Sophia are all giggles as they get ready to start the walk.



Our Latest Miracle Makers - PRF's Pride and Joy!

PowerBlockTV Sells its Unique Cars for Progeria

The PowerBlock is a series of four automotive how-to programs: *Horsepower, Trucks!, MuscleCar* and *Extreme 4x4*. They made room for the 2009 projects by auctioning six favorites. The fans went wild – bids for the one-of-a-kind vehicles totaled \$100,000! RTM Productions' owners Joe and Patty St. Lawrence (pictured here) donated 20% of the proceeds to PRF, after learning of Progeria through PRF supporter Chip Foose. **Now THAT'S Power!**



More and more athletes are running for Progeria!

In addition to our Boston Marathon runners, this spring two dedicated women ran marathons in their communities, raising thousands for PRF. **Now THAT'S racing for a cure!**



Patti (at right) with friend and fellow runner Paula

Kira Brady ran the Newport Beach, CA Avia OC Marathon – a "Marathon for Miles" – for her 2-year-old god-nephew, raising over \$5,000. "It is Miles' wonderful spirit, kindness, sense of humor and joy that define him in a way that his disease never will," she says. We applaud Kira's own spirit!

Patti Tanaka ran her very first marathon in Colorado, in loving memory of her daughter Rachel. Patti's shirt had Rachel's picture on it, keeping her close to her heart the whole route. **Way to go, Patti!**

Kids Helping Kids - it's the BEST!

Charlotte, Lauren and Savannah of Boxford, MA are industrious young ladies! They ran a "Pennies for Progeria" campaign at their elementary school in Boxford, MA and raised \$188! Many thanks for your hard work and dedication to children with Progeria, girls.



We hope you inspire others your age to do the same!

Graduating Girls Perform Community Service for Cameron

Rachel and Alanna raised over \$1,400 in honor of local child Cameron. Students must complete community service hours to graduate, so these industrious young ladies organized a Coins to Cure Progeria® contest: homerooms competed and the one who raised the most got a pizza party donated by Domino's. They also held a casual Friday, with students paying to wear jeans instead of uniforms. Good luck in college, girls, **something tells us your futures are very bright!**

Many schools, clubs and religious organizations require students or members to perform community service – why not choose PRF to benefit from it?!

Hot Wheels are Hot for PRF

Car enthusiast Craig Pike held a "Home Runs, Hot Dogs and Hot Rods" show in Mesa, Arizona to benefit PRF. For a donation, dozens raced their toy cars on 50-foot tracks. Craig, inspired by the story of Amy Foose, held this event in her honor, raising \$365 for Team Foose and the ONEpossible campaign. **Thanks for helping drive the cure, Craig!**

Our First Miracle Makers from China!

Another example of how finding a cure for Progeria is truly a global effort: 8th graders at Shanghai Community International School, quite moved when they learned about Progeria, held a dance with games, raffles and a bake sale, raising \$1,130! "They used their free time during lunch and after school organizing the event", said teacher and event advisor Izumi Tsurumi, "These generous students represent the community spirit and outreach that is important to the school." **We agree completely!**



Senior Bakes to Make a Cure POSSIBLE

Jennifer, a Woburn (MA) High School senior, learned about the ONEpossible campaign through PRF's website and wanted to help, so she organized several school bake sales, and the sweets-craving students cleaned her out! Jennifer raised \$350 and handed out brochures to help educate her classmates about Progeria. **Thanks to Jennifer and all the students who enjoyed the goodies for a good cause**

Student "kicks butt" to raise money and awareness for PRF!

Another successful bake sale – and for the first time, at a Tae Kwon Do school! Laurie of Green, RI, raised \$100 at the New England Tae Kwon Do Center in nearby Coventry, selling out of her goods and telling fellow students about Progeria. Thanks for demonstrating your skills in and out of the dojo, Laurie, and showing that **bake sales can be held in all kinds of places!**

Who else wants to join in and bake for a cure?!

Kentucky Go-Cart Racers Go Wild for PRF!

J & M Motorsports Park in Columbia hosted a Benefit Race for Progeria. After Biff Campbell met Zach, he and his family wanted to support PRF by organizing the event. With sponsors, raffles, entry fees and concession sales, they raised over \$3,000 - **Way to GO, Campbell family!**

Go-cart racer Hannah Campbell with Zach. Hannah also raised \$5,000 for Team Pickard's ONEpossible efforts – go, Hannah, go

Many people comment on how fun this section is to read and be inspired by – see even more photos and events at www.progeriaresearch.org

Lots more events planned for 2009 – go to www.progeriaresearch.org for the latest listings, or start one of your own!

The Progeria Research Foundation

P.O. Box 3453
Peabody, MA 01961-3453

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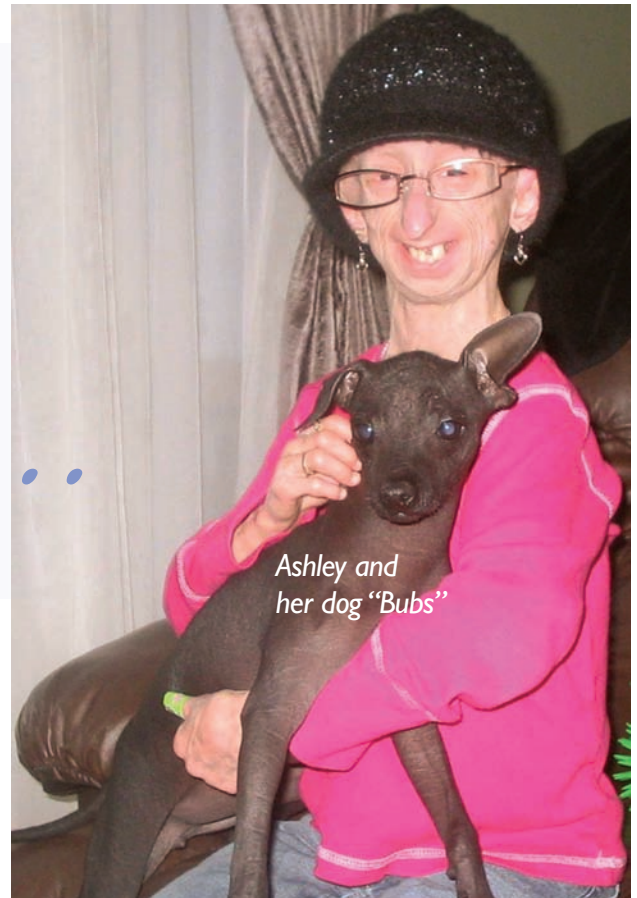


RETURN SERVICE REQUESTED

*In Loving
Memory
of Ashley...*

Ashley, from Canada, passed away in April, one month before her 18th birthday. Ashley's popular YouTube video was viewed by millions who enjoyed seeing her bright personality and insightful advice to other children with Progeria: "when people stare at you, don't let it bother you...you're one of a kind".

*Ashley is gone from our sight, but never our memories
Gone from our touch, but never our hearts.*



Ashley and
her dog "Bubs"