



Introducing PRF's Ambassador Family

In April 2005, the family of 5-year-old **Megan Nighbor** was named PRF's Ambassador Family. The Nighbors are an integral part of PRF's efforts to raise awareness, provide support to other families and involve them in our programs, and fundraise for research. We appreciate the time and effort the Nighbors put into these important activities. They are a busy family, spending lots of their time at activities like Michaela's Pee-Wee Baseball games, Josh's football games, and just trying to keep up with Megan's boundless energy! "We try to make everything a family gathering", says Sandy.

Megan's dad **Steve** works at Wisconsin's Alto Dairy Cooperative and mom **Sandy** works at Ted's Piggly Wiggly. **Joshua** will be a Sophomore at Markesan High school this fall, and **Michaela** will enter 2nd grade at Markesan Elementary School. Megan loves going to Sunshine Nursery School. All three have made lots of friends at school and through the many sports and hobbies they enjoy.

To learn more about the Nighbors, go to <http://www.progeriaresearch.org/spokespeople.shtml>



Sandy, Megan, Josh, Steve and Michaela Nighbor

The Progeria Research Foundation

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The Progeria Research Foundation

www.progeriaresearch.org

Summer/Fall 2005

Workshop 2005

PRF Continues to Promote Scientific Collaborations in its Push Toward Treatments

PRF is assembling a group of internationally-recognized scientists in Boston, Massachusetts on November 3-5, 2005 for its fourth workshop. With speakers that include leading scientists in the fields of aging, heart disease, genetics, and laminopathies, this meeting promises to influence this fast-growing field of study and help us to understand more about the biological basis of disease in Progeria, atherosclerosis and cellular aging.

This workshop is expected to set the stage for the next round of progress in translating bench research on Progeria into treatment.

And for the first time, researchers will have the opportunity to meet with some of the children and families living with Progeria.

See page 4 for more details.



This photo of Zach and his dog Hobbes was posted on the message board by mom Molly, who wanted to introduce her son to the other Progeria families.

Because it's wonderful to be able to talk to someone who understands what you're going through....

Introducing the PRF Progeria Family Message Board

We are excited to announce the creation of a private message board web site created just for the families of children with Progeria. We have created this message board in response to requests from families who want to connect with other families, and in keeping with our mission to provide valuable resources for our many families around the world. Our hope is that this on-line tool will help them get to know each other, and develop a support network of people with whom they can share concerns and ideas on how best to care for their children.

The message board is also a place where parents can ask questions and receive answers to medical issues from Dr. Leslie Gordon, PRF's Medical Director. Parents can also carry on a real-time conversation with one or many people. Lots of families have signed up since the June launch, and are chatting away!

Thank you to **CVS/pharmacy**® for sponsoring our 2005 Newsletters!

PRF's Mission: **To discover the cure and effective treatments for Progeria and its aging related disorders.**

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Message from the President & Executive Director

Hello everyone,

It's so wonderfully busy here at the office that sometimes I literally stop in amazement – at the pace, the number and variety of things that we do here each day, and the joy at knowing how much we accomplish daily, moving closer and closer to our goal of treatments and a cure – with all of you leading the charge.

And how could Susan, Lynne and I not be busy?! Families of children with Progeria, volunteers and donors contact us every day, wanting to know how they can help, or needing help. We are so glad to be here, to respond to such varied inquiries as:

- Does my child have Progeria?
- I'd like to sell wristbands for you.
- Can you give me some information for a school report? I want my classmates to know about this disease.
- I need to order cells from you for my research.
- I'm writing an article on Progeria, can you answer a few questions for me?
- Can you put me in touch with other families of children with Progeria for support?

In addition to all the great things that occurred before this year and are ongoing, 2005 is turning into yet another amazing year in many new ways: Relatives of children with Progeria are mobilizing in record numbers to participate in our programs and raise money for Progeria research; Every day Kim, Debbie and other members of the Night of Wonder committee are working to make this the biggest gala ever; People are emailing us every few days to proudly tell us the most recent tally of sponsors they've gotten for the Race for Research; the number of participants in the November workshop is rising; the list goes on and on!

Keep those emails and that support coming! You provide the momentum, and are a constant reminder that the work we are doing is vital to the families and to you, because each and every one of you, for many different reasons, want to save the lives of children with Progeria. You have all taken ownership of our mission and we gladly share it, because it is only **Together** that we **WILL** find the cure!

Sincerely,

Audrey Gordon, Esq.
President, Executive Director





Upcoming Events

Join us for our biggest and best Race for Research EVER!

NORTH SHORE BANK is teaming up with PRF for the Fourth Annual International Race for Research in PRF's home town of Peabody, Massachusetts on **September 10th**. Start training for this 5K run, or take it a bit easier with the 2-mile fun run/walk. This is a terrific event that everyone enjoys - there are costumed characters for the kids (and the young at heart!), food, drinks and t-shirts for all, and awards for top finishers and all kids.

Here's how you can help:

- Grab a friend, neighbor, relative, co-worker – anyone and everyone you know to run or walk with you
- Ask people to sponsor you for \$10, \$20 or more. Tell them it's to save kids' lives and they might give you an extra \$10!
- Raise \$100 or more and get free race registration
- There's a prize for the person who raises the most in sponsorships – it could be YOU!

What are you waiting for?! Download an application at <http://www.progeriaresearch.org/events.shtml>

You don't live nearby? That's OK - run in your neighborhood on the same day, or organize a race where you live.



Celebrate the Science! with Dr. Sanjay Gupta, Chip Foose and hundreds of other PRF supporters at the 2005 Night of Wonder Gala on November 5th in Boston, Massachusetts



Our planning committee is hard at work making this the biggest and best Night of Wonder yet. World-famous CNN senior medical correspondent Dr. Sanjay Gupta is our honorary chairperson. Dr. Gupta was one of the first reporters to cover the historic Progeria gene finding in April 2003, and he plays an integral role in the network's medical coverage – a perfect fit for PRF's research goals and our gala science theme!

We will also be honoring TLC's Overhaulin' star Chip Foose with PRF's new Amy Award, named after Chip's sister who had Progeria and inspired all those around her with her upbeat personality and zest for life.

Here's a sampling of the items that will be on the auction block:

- 1 week stay at a luxurious, oceanfront condo at Myrtle Beach Surf Club
- A week for 2 at Canyon Ranch health spa in Arizona
- Lots of Red Sox tickets!
- A visit to the set of Spiderman III
- A VIP weekend at the Baseball Hall of Fame...and hundreds of other items!



The Night of Wonder has raised over \$300,000 since 2001 and every dollar has been spent directly on research to find the cure. Help us make *Night of Wonder 2005 - Celebrate the Science!* our biggest fundraiser ever - Here's how:

- **Become a corporate or individual sponsor:** levels range from \$2,500 to \$20,000. Join Harvey Industries, Schering-Plough, Eastern Bank, PriceWaterhouseCoopers, the Red Sox Foundation and others, or maybe you know someone interested in becoming a sponsor...

- **Place an ad** in our colorful program book. Ads range from \$250 to \$1,000.
- **Donate an auction item:** we need airline vouchers, hotel stays, New England sports tickets, golf foursomes and restaurant gift certificates in the MA area, sports memorabilia, and items for themed baskets (kid's items, sports, jewelry, health & beauty).
- **Suggest new people to add to our invitation list.** Email PRFNOW05@yahoo.com for more information.



PRF WRISTBANDS ARE HERE!

Join the craze with our high quality, fashionable bands in the PRF colors of blue and white, with **"Progeria Research Foundation - Together we WILL find the cure!"** on the outside, and the PRF web address inside. They're \$5 each, or three for \$10, in youth or adult sizes. They also make great gifts for friends, family, and others who want to help find a cure for children with Progeria. By wearing them, you are showing your support and spreading the word about Progeria. We've already sold 500, raising over \$2,000. Please contact susanr@progeriaresearch.org if you'd like one – or two, or ten...

The PRF International Workshop November 3-5, 2005 Boston, Massachusetts

PRF is assembling a group of internationally recognized scientists in Boston for its fourth workshop. Our goal is to provide a highly interactive, collegial environment for scientists to present recent experimental findings in the field of Progeria that will promote new ideas for future research and foster new scientific collaborations. PRF workshops in the past have generated alliances that have led to scientific breakthroughs, such as the discovery of the gene that causes Progeria – the sky's the limit on what we can accomplish through this meeting!

Elements new to this workshop will include a poster session and the opportunity for researchers – many for the first and only time - to meet with children and families living with Progeria.

PRF workshops have helped to take Progeria from a position of almost no recognition and scientific effort, to a vibrant field of research, and a new avenue for investigating aging and cardiovascular disease. We have no doubt this workshop, with the added participation of the European Progeria research community, will further advance the work towards treatment and the cure.

Experts in all aspects of Progeria will participate, including:

Paris, France: Gisele Bonne, MD, leader of a research group at the Institute of Myology that studies the genetics and pathophysiology of the laminopathies (diseases genetically related to Progeria).

Staten Island, New York: W. Ted Brown, MD, PhD, Director of the New York State Institute for Basic Research in Developmental Disabilities and a leading expert on Progeria.

Bethesda, Maryland: Francis S. Collins, MD, PhD, Director of NIH's National Human Genome Research Institute responsible for the Human Genome Project and senior author of the article identifying LMNA as the gene responsible for Progeria.

Chicago, Illinois: Robert D. Goldman, PhD, Stephen Walter Ranson Professor and Chairman of Cell and Molecular Biology at Northwestern University Medical School whose research examines the relationship between the structure and function of lamins.

Providence, Rhode Island: Leslie B. Gordon, MD, PhD, Assistant Professor of Pediatrics at Brown University, Associate Investigator on the clinical studies of Progeria at NIH, and founder and medical director of PRF. Dr. Gordon directed the previous PRF workshops and serves as the Principal Investigator on the PRF Cell & Tissue Bank, Medical & Research Database, and Diagnostics Testing projects.

London, England: Raoul Hennekam, MD, PhD, Professor of Clinical Genetics and Dysmorphology at the Institute of Child Health and Great Ormond St. Hospital for Children, he has led research studies in Progeria.

Cambridge, Massachusetts: Richard T. Lee, MD, Cardiologist at Brigham and Women's Hospital, Dr. Lee's research focuses on how cells in the heart and blood vessels respond to mechanical forces, which may aid in the prevention of heart attacks.

Marseilles, France: Nicolas Levy, MD, PhD, Professor of Genetics at the Faculte de Medecine de la Timone and leader of a research team at the Hospital of Timone that is developing Progeria mouse models to help understand how to treat Progeria and possibly correct the genetic defect.

Seattle, Washington: George M. Martin, MD, Professor of Pathology and Adjunct Professor of Genetics Emeritus at University of Washington School of Medicine who has made significant research contributions in the biology of aging and aging-related diseases.

Bethesda, MD: Elizabeth G. Nabel, MD, PhD, Director of the National Heart, Lung and Blood Institute at NIH studying pathogenesis and genetic therapies for vascular diseases.

Los Angeles, California: Stephen G. Young, MD, Professor of Medicine at the University of California, Dr. Young's research focus is in lipoproteins and atherosclerosis.

Clinical Studies in Full Swing at the National Institutes of Health Numbers have already surpassed 2005 goals

PRF and NIH have teamed up to conduct clinical studies on Progeria - studies that will prepare us for treatment protocols in the future. Progeria families have been flying in from all over the world to NIH in Bethesda Maryland, where an elite team of experts is gathering a large amount of critical medical information on Progeria. We need these studies to assess whether, when it becomes available, a particular treatment is right for each child, and to understand whether a treatment is working to slow down or reverse the disease process. By starting now, we will be years ahead of schedule when a new treatment is ready to be implemented for the children. To participate in this study, contact Dr. Leslie Gordon at lgordon@progeriaresearch.org.



The Contreras family took part in the clinical studies in July.

Your support helps us supplement the costs that NIH does not cover, so that no family involved in this important project has to pay any portion of the expenses. Thank you!

More Scientists Associated with PRF Publish Important Data on Progeria Research

The field of Progeria research is growing by leaps and bounds! In June, another important study was published, bringing us ever closer to solving the puzzle of how to repair the genetic defect that causes Progeria.

Blocking protein farnesyltransferase improves nuclear blebbing in mouse fibroblasts containing a targeted Hutchinson-Gilford Progeria syndrome mutation.*
Proceedings National Academy of Sciences, July 2005

***By Shao H. Yang, Julia I. Toth, Yan Hu, Salemez Sandoval, Stephen G. Young, and Loren G. Fong, David Geffen School of Medicine, UCLA; Margarita Meta, University of California, San Francisco; Pravin Bendale and Michael H. Gelb, University of Washington, Seattle; Martin O. Bergo, Sahlgrenska University Hospital, Sweden**

After creating a gene-targeted mouse model of Hutchinson-Gilford Progeria Syndrome (HGPS), the authors set out to prove that the inhibition of a process called protein farnesylation with farnesyltransferase inhibitors (FTIs) can block the damage to the nuclear envelope caused by the mutant protein progerin. The studies suggest the cells can be repaired using this approach.

The mutant prelamin A in HGPS, commonly called progerin, is caused by a mutation in LMNA that results in the deletion of 50 amino acids within prelamin A and prevents normal processing to mature lamin A. The presence of progerin in cells adversely affects the integrity of the nuclear lamina, resulting in misshapen nuclei and nuclear blebs.

Fong and his group examined the effects of an FTI on this process and found that it resulted in a striking improvement on nuclear shape (reduced misshapen and damaged nuclei).

“These studies suggest a possible treatment strategy for Progeria” says co-author Dr. Steven Young, “raising the hope that FTIs might ultimately prove useful for treating Progeria.”



Sam and Devin have a mini-reunion at NIH. Both have participated in the clinical studies.

Two More Research Grants Funded by PRF in July Bringing us Closer to Treatments and a Cure

Thanks to the tremendous and ever-increasing support from all of you, PRF is currently funding an astonishing 8 research projects. Here are the latest two, approved in June:



Lucio Comai, PhD, University of Southern California, Los Angeles, CA
Functional Analysis of Hutchinson-Gilford Progeria Syndrome

Dr. Comai hypothesizes that expression of the mutant Lamin A protein progerin (that causes Progeria) results in premature aging and cardiac disease as a consequence of altered composition and function of Lamin A-containing complexes within the nucleus. To test this hypothesis, he will seek to identify cellular factors that differentially interact with lamin A and progerin. These studies will provide critical information on the molecular defects of Progeria, as we work toward developing treatments at the cellular level.

Dr. Comai is Associate Professor of Molecular Microbiology & Immunology at the USC Keck School of Medicine, and a member of the Keck School’s Institute for Genetic Medicine, Norris Comprehensive Cancer Center and Research Center for Liver Diseases.



Loren G. Fong, PhD, University of California, Los Angeles, CA
New Mouse Models to Study the Cause of Hutchinson-Gilford Progeria Syndrome

Since the discovery of the Progeria gene mutation more than 2 years ago, efforts have gone on in several laboratories to create a mouse that produces the “bad” lamin A (progerin) made in Progeria. Dr. Fong and his colleagues have succeeded in doing this, and now will investigate the effects of mouse progerin on the growth and metabolic properties of cells, the development of atherosclerosis, bone abnormalities and lipodystrophy in the whole animal, and finally to test whether any abnormalities can be reversed by farnesyl transferase inhibitors, at present the leading candidates for treatment of Progeria.

Dr. Fong is an Assistant Adjunct Professor at UCLA, and has joined forces with Dr. Stephen Young, a May 2005 PRF grantee, to tackle this important scientific and medical problem.

Family Miracle Makers

Many families of children with Progeria have been holding events to help in the search for a cure for their children, grandchildren, nieces, nephews, and siblings. We are so grateful for the trust and confidence they have in PRF's work.

Kennedy Family Holds Dance, Golf Tournament in Indiana

The family of 19-month-old **Sarah Kennedy** held a benefit dance and silent auction in February, and Sarah's grandfather **Chris Kempf** organized a golf-scrabble at Eagle Valley Golf Course in April. The events raised \$7,000! 124 golfers enjoyed a beautiful day out on the course. "We were truly blessed and many commented it was the best scramble they had ever played in," said Chris. "I am humbled to have been able to do this for the foundation and for Sarah. We have already begun discussing next year's event, and we will continue collecting money in our donut shops as well. Thank you for all you do."



Kaylee and Sarah (with their moms) at the golf event. They became fast friends.

Auntie Power!!! Aunts of two children take the fight for a cure to their communities.

Carol Beane, Queen Mum of the St. Mary's, Ohio Chapter of the Red Hat Society and the great-great aunt of 2-year-old **Kaylee Halko**, and her fellow "Rowdy Rabbits" have sold our cookbooks, and are planning an August bake sale. The Red Hat Society is a "fun after fifty" club for women. Members wear bright red hats and purple outfits and have "a genuine enthusiasm for wherever life takes them next." "We thought it would be even more delightful to be doing some good in this world, while having fun!" said Carol.

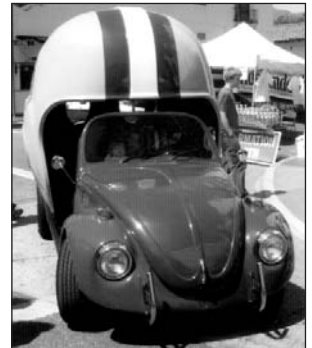


Megan with parents and Aunt Sue

Headed by **Amazing Aunt Sue**, **Megan Nighbor's** family held a 2-day Summer Splash Benefit in Wisconsin in July. This event had it all: pool tournament, raffle, Karaoke contest, kids pedal pull, dunk tank, bake sale (you have never seen a bake sale quite like this!), motorcycle ride, silent and live auction, craft fair and live band that had everyone up on the dance floor. The event raised over \$22,000!

Amy Foose's Celebrity Brother Signs Autographs at Auto Shows

Chip Foose, award winning car designer and star of TLC's popular *Overhaulin'* spent hours at two recent car shows, signing t-shirts for a cause near and dear to his heart: Chip's sister **Amy** died of Progeria in 1985, before PRF existed to provide support and hope to the families. Appearing in April at the "Wheels and Waves" show in Chip's hometown of Santa Barbara, California, and at the second largest car show in the world, "Back to the Fifties" in St. Paul, Minnesota in June, Chip signed autographs and drew pictures on t-shirts and custom posters for the never-ending line of fans, for a donation to PRF. Over \$6,600 was raised from both shows. We appreciate everything Chip, his parents **Terry and Sam**, and friends, family and fans are doing as we race to find the cure for Progeria.



A special attraction at the April show: "The Amy Car", built by Chip and his dad in 1986 as a memorial to Amy.



Zach's 3rd Birthday Celebration Held July 9th

Thanks to **Zach Moore's** tremendous popularity and the generosity of many who donated space, entertainment and food, Zach's family and friends had a BIRTHDAY BASH on the campus of Oklahoma State University. And mom **Molly** generously turned the party into a fundraiser for PRF. "Zach has everything he needs except a cure," said Molly, "so instead of giving Zach gifts, please buy a t-shirt for \$15 or make a donation to PRF in honor of Zach turning 3." They raised over \$2,400 and donations are still coming in.

T-Shirts are still available!!

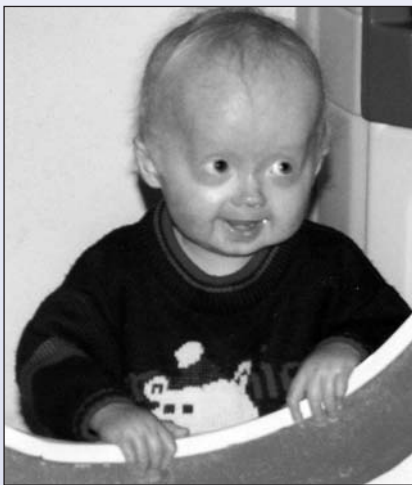
Call PRF
978-535-2594

Check out lots more Miracle Makers on the web site

We've got so much going on that we ran out of room!!
Watch for a big spread on the latest volunteer heroes in the next issue, or visit
http://www.progeriaresearch.org/miracle_makers.shtml to see them today!

*Gone from our sight
But never our memories –
Gone from our touch,
But never our hearts...*

we remember



Kieran
*8 years old,
from England,
who passed away
in March 2005*



Saim
*14 years old,
from Turkey,
who passed away
in July 2005*