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ONEpossible 2013!	BACK COVER

Triple Trial Enrollment Reopens!

Expansion Gives More Children the Opportunity to Participate

Beginning this summer, up to 40 children will come to Boston Children's Hospital for their first Triple Trial visit. PRF is thrilled to enable them, along with the children currently enrolled, to participate.

Read more about this exciting development on page 4.



Brennen, 4 years old from the United States, all dressed up for a party! His parents plan to enroll him in the Progeria Triple Trial.

International Scientific Workshop 2013

Record-breaking in Every Aspect



With an astonishing 180 participants from 18 countries, and a three-day agenda packed with treatment-focused topics, this year's workshop reflected the exponential growth in the field of Progeria research that will ultimately lead us to the cure.

See pages 6-7 for more details.

Dr. Mark Kieran discusses "Challenges and Strategies for Clinical Trial Design in Progeria." Dr. Kieran is the Principal Investigator for the Boston-based drug trials.





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

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**Progeria is a fatal, "rapid 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.**

**Because of Progeria's
connection to general heart
disease and aging, what we learn
from Progeria research has the
potential to benefit all of us.**

**Message from the President
and Executive Director**

Hello everyone,

From the new faces we hope to see soon in Boston, to the researchers diligently working to find a cure, to the volunteers raising awareness and funds to make that cure possible - this is what PRF is all about. **We are in a race against time for the children!** This is what drives us, inspires us, and convinces us that we will reach our ultimate goal of a cure.

So many wonderful things are happening!

-  Soon HBO's *Life According to Sam* will appear in millions of homes around the world, raising awareness of Progeria and PRF's work to save the children, in an uplifting and enlightening way.
-  Children discovered over the past 3 years now have the opportunity to enter a Progeria clinical trial that includes the effective lonafarnib drug.
-  Our new clinical trial campaign must raise \$4 million to give every child the opportunity for treatments.
-  Our 7th international workshop illustrated the astounding depth and breadth of new research knowledge and advances.
-  103 children living with Progeria have been identified, from 37 countries.
-  Our donors and volunteers – new and returning – devote their time, talent and treasure to raising funds and awareness through positive energy, hard work, and love for the children.

With program growth comes the need to sustain it. Please contribute generously to PRF's **ONEpossible campaign** – to give every child a chance at potentially life-saving drug trials, and to keep our progress going at the phenomenal rate it has enjoyed for the past 13 years. It is only through your continued support that we will treat children with Progeria today, and cure them in the future.

Many thanks to all of you who care so very much.



Audrey Gordon, Esq.
President and Executive Director

In Loving Memory...

of the children who passed away over the past 18 months...

*Gone from our sight, but never our memories ~
Gone from our touch, but never our hearts.*



Priya
20 years old from India



Christian
13 years old from Germany
(pictured here at age 5)



Milagros
11 years old from Argentina



Azeddine
20 years old from Morocco



Valentina
1 year old from Argentina



Eric
18 years old from Canada



Sarah
9 years old from the
United States



Pedro
11 years old from Brazil



BREAKING NEWS!

Progeria Triple Drug Clinical Treatment Trial Expanded to Include 40 More Children

As this newsletter arrives in your mailbox, the first of up to 40 children have already arrived at Boston Children's Hospital for their initial Triple Trial visit. These children come from 24 countries and speak 15 languages! They will travel to Boston four times over a 2-year period, for testing and to receive new drug supply.

The three treatments these children will receive are lonafarnib, pravastatin, and zoledronate. We had fantastic success with our first clinical trial using the experimental drug lonafarnib, where every child improved in one or more ways. But we have a long road ahead in the fight to save children with Progeria. While that first trial was underway, researchers identified two additional drugs that, when used in combination with lonafarnib, may be even more effective than the single drug in improving some of the effects of Progeria. Our "Triple Drug Trial", launched in August 2009, tests this three-drug combination: the farnesyltransferase inhibitor (FTI) lonafarnib, the cholesterol-lowering drug pravastatin, and the bisphosphonate zoledronic acid which is normally used to prevent and treat osteoporosis.

Why are we expanding the Triple Trial? Initial enrollment for the Triple Trial ended in early 2010, and since then over 40 new children with Progeria have been identified. The expansion will allow children found after initial enrollment ended to receive the treatment for Progeria, plus the two other drugs. With the recent breakthrough discoveries from the first trial, we now know that some Progeria disease features can be affected in a positive way, including the cardiovascular system! The Triple Drug Trial expansion allows us to continue exploring additional treatment options while giving all known children living with Progeria today the opportunity to participate in a clinical treatment trial.

Now more than ever, your support is needed!

PRF is funding all aspects of the trial, including travel, lodging, testing and staff.

PRF must raise \$4 million for all children – that's up to 80 in total – to complete the Triple Trial. Please help us kick off this campaign with your ONEpossible donation! (See back cover and return envelope.)

Meet some of the children who may soon enter the Triple Trial:



Yusally
Dominican Republic



Nihal
India



Griffin
United States



Lucy
Ireland



Elias
Mexico

New Trial Results for Lonafarnib Just Published

Shows Promising Results for Strokes, Headaches, Seizures

Publication in September 2012 of the first-ever treatment trial for children with Progeria brought the fantastic news that, through the PRF-funded, first-ever Progeria clinical drug trial, the first treatment for Progeria has been discovered. The news was covered worldwide, as the historic finding was touted as “a scientific sprint” due to the rapid progress, and a potential “unexpected payoff for the rest of humanity” given that Progeria research may help aging-associated heart disease, such as atherosclerosis, in all of us.*

Now there is even more reason to be hopeful about the effects of lonafarnib. A new study† by the Boston Children’s Hospital clinical trial team shows evidence that lonafarnib therapy may improve neurologic status of children with HGPS. Frequency of clinical strokes, headaches, and seizures was reduced from pretrial rates. The study was published in the journal *Neurology* on June 29, 2013. These researchers will continue to ask whether strokes and headaches are decreased during the triple drug trial as well.



Study author Dr. Nicole Ullrich, with new patients Carly & Zoey at Boston Children’s Hospital.

† Neurologic Characteristics in Children with Hutchinson-Gilford Progeria Syndrome Before and After Treatment with the Farnesyltransferase Inhibitor Lonafarnib

Authors: Nicole J. Ullrich, MD, PhD, Mark W. Kieran, MD, PhD, David T. Miller, MD, PhD, Leslie B. Gordon, MD, PhD, Yoon-Jae Cho, MD, PhD, V. Michelle Silvera, MD, Anita Giobbie-Hurder, MS, Donna Neuberg, ScD and Monica E. Kleinman, MD

* Check out our special newsletter on the first trial results at progeriaresearch.org/trialresultsnewsletter.pdf



Zach downs around with the “doctors” in December.



Lindsay and Megan received “Super Star” ribbons during their January visit.

2013 Progeria Research Foundation's 7th Internatio



Hand in Hand Basic & Clinical Science Working Together Toward the Cure

April 24-26, 2013 • Bethesda, MD

A record **180 Progeria clinicians and researchers** gathered from **18 countries** to set the stage for the next round of progress in translating bench research into treatment. **39 talks** and **56 poster presentations** inspired future collaborations and disseminated important scientific findings. Many of the hot topics discussed during the workshop had both a basic and translatable clinical component. *This exchange between basic and clinical investigators will be crucial on the road to success, when we not only cure children with this fatal disease, but also translate discoveries in Progeria to our understanding of heart disease and aging.*

The Meeting Agenda at a Glance

- ✓ Living with Progeria – Patient Perspectives
- ✓ Presentation by Francis S. Collins, NIH Director
- ✓ Current Treatment Trial Discoveries: US & French Teams
- ✓ What Progeria Can Teach Us About Cardiovascular Disease and Aging
- ✓ Lightening Young Investigator Panel Presentations
- ✓ Boston Children's Hospital Clinical Presentations
- ✓ The Biochemistry and Pathophysiology of Lamins
- ✓ The Next Phase: Drug Development, Genetic Therapy

The stage was set during the first evening with a family panel entitled **Living with Progeria – Patient Perspectives**, moderated by Academy and Emmy Award-winning documentary filmmakers **Andrea Nix Fine** and **Sean Fine**, directors of

Life According to Sam (see page 10). Researchers had a unique chance to meet some of the people their work could help: **Meghan Waldron** and her parents,



Tina and **Bill**; **Devin Scullion**, along with his mom **Jamie**; and **Megan Nighbor**, with her parents **Sandy** and **Steve**. They talked to a captivated audience about what it's like to live with Progeria, and thanked the researchers for the work they're doing to help find a cure.

Day two kicked off with a session entitled **Current Treatment Trial Discoveries**. Clinical treatments with the farnesyltransferase inhibitor lonafarnib, pravastatin and bisphosphonate were discussed with regard to their potential impact on Progeria and aging. **Nicolas Lévy** (University of Marseilles) presented new findings from a Progeria clinical trial of pravastatin and zoledronate. Discoveries on the clinical implications of calcium dysfunction in Progeria and aging were presented by **Catherine Gordon** (Brown Univ., Harvard) and **Catherine Shanahan** (King's College London), while cardiologist **Marie Gerhard-Herman** (Brigham & Women's Hospital, Harvard) and neurologists **Michelle Silvera** and **Nicole Ullrich** (Boston Children's Hospital, Harvard) gave new insights into cardiovascular disease and stroke in Progeria and aging. Finally, **Mark Kieran** (Dana Farber Cancer Institute, Harvard) guided a discussion on Challenges and Strategies for Clinical Trial Design in Progeria.



Dr. Lévy

PRF added a new twist to this year's meeting, incorporating two **lightening panel sessions** intended to maximize cross-talk between basic and clinical researchers.

Panel I was packed with PRF-funded scientists who provided cutting edge findings and their implications for a better understanding of the biology of disease in Progeria, CVD and aging. **Kris Dahl** (Carnegie Mellon Univ.), **Jason Lieb** (Univ. of North Carolina), **Kan Cao** (Univ. of Maryland), **Yue Zou** (E.Tennessee State Univ), **Tom Glover** (Univ. Michigan), **Jan Lammerding** (Cornell Univ.), **Karima Djabali** (Technical Univ. Munich), **Thomas Dechat** (Medical Univ. of Vienna), **Maria Eriksson** (Karolinska Inst.) and **Samuel Benchimol** (York Univ.) displayed new work ranging from stem cell studies, to cell treatment with rapamycin, to genome instability and cellular aging in Progeria.



Dr. Cao



“Every year the number and quality of publications in the field grows exponentially. It is the people sitting in this room that are pushing this field forward, making all the difference in finding treatments and cure.”

– Opening remarks by PRF Medical Director Dr. Leslie Gordon

nal Scientific Workshop

In **Panel 2**, physicians from the Boston Children's Hospital clinical trial team representing every area of clinical disease in Progeria shared their latest insights. **Monica Kleinman** (Critical Care), **Leslie Smoot** (Cardiology), **Ashwin Prakash** (Cardiac MRI), **Marilyn Liang** (Dermatology), **Guangwei Zhou** (Audiology), **Brian Snyder** and **Ara Nazarian** (Orthopedics), and **Jessica Spratt** and **Annette Corriea** (Physical and Occupational Therapy) are finding brand new ways to measure treatment effect through a deep understanding of how Progeria affects different body systems in the children.



Dr. Prakash

The final day of talks left no stone unturned. National Institutes of Health Director **Francis Collins** started the day with insights into Progeria's overlaps with generalized aging, followed by perspectives on the overlaps with atherosclerosis of aging and Progeria from the basic perspective, **Vicente Andrés** (CNIC, Madrid) and a pathologist's perspective, **Richard Mitchell**, (Brigham & Women's Hospital).



Dr. Collins presents "Progeria as a Model of What's Right with Medical Research"

In a session on the **Biochemistry and Pathophysiology of Lamins**, **Robert Goldman** (Northwestern Univ.) detailed studies on the importance of B-type lamins on disease, **Colin Stewart** (Inst. of Medical Biol., Singapore) presented studies on induced pluripotent stem cells, and **Kan Cao** (Univ. Maryland) showed cellular effects of the drugs everolimus and rapamycin.

The afternoon session delved into **The Next Phase for Treating Progeria**. **Tom Misteli** (National Cancer Inst.), **Jeff Chamberlain** (U. Washington), and **Carlos López-Otín** (Univ. of Oviedo, Spain) discussed genetic treatment strategies and their potential for Progeria. In a fitting end to the meeting, **Brian Kennedy**, (Buck Institute), **Rafael de Cabo**, (National Inst. On Aging) and **Monica Kleinman** spearheaded a **Future Treatments Panel**, where they debated approaches to treatments with resveratrol and everolimus for children with Progeria.



Drs. Kennedy and de Cabo



Congratulations to this year's Poster Winners! **Ricardo Villa-Bellosta** from Centro Nacional de Investigaciones Cardiovasculares in Madrid won best basic science poster for studies on vascular calcification in HGPS; clinical poster winners were Boston Children's **Isabella Chase** for Dental and Craniofacial studies, and **Nicole Quinn** for her study on Energy Intake, Energy Expenditure and Body Composition in HGPS.

Overwhelmingly Positive Feedback!

With a 100% approval rating overall, the most common remarks from attendees reflected excitement about data sharing and collaboration leading to new ideas for future work. Here are a few attendees' comments:

"These workshops aid in moving the field of Progeria research along at a faster pace as a result of the collaboration, networking, sharing of new ideas and personal interaction."

"I gained a greater understanding of the disease process and what/how we are trying to treat/cure the condition, and how these impact research into normal aging."

"This meeting has given me new ideas and access to new resources by building contacts. I plan to start a new research project on Progeria."

"The greatest strengths of this workshop were the cross discipline and from bench to bedside review of what we've learned, what is going on and what the future brings - all very exciting!"

Many thanks to our Workshop Organizers...

Leslie B. Gordon, MD, PhD, Medical Director, The Progeria Research Foundation

Frank G. Rothman, PhD, Professor and Provost Emeritus at Brown University.

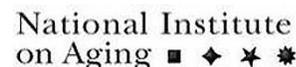
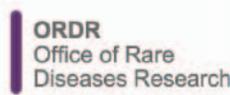
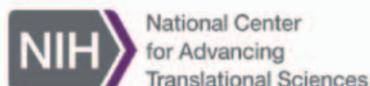
Carlos Lopez-Otin, PhD, Professor of Biochemistry and Molecular Biology, Medical School of Oviedo University, Oviedo, Spain.

Tom Misteli, PhD, Director of the Cell Biology of Genomes Group at the National Cancer Institute, NIH.

...and our Supporters



Thank you to Brown University for providing Continuing Medical Education credits.



The Max and Victoria Dreyfus Foundation

PRF Awards Ten More Research Grants

As of June 2013, PRF has provided over \$4 million to fund 45 grants for Progeria-related research projects performed in 15 states and 10 other countries. We solicit proposals worldwide, in our continuing effort to advance the development of treatments and cure as rapidly as possible.

The following have been funded since our last regular newsletter:



Several of PRF's latest research grantees participated in a panel discussion at the April workshop. Pictured here are, back: Jason Lieb, Yue Zou, Tom Glover, Jan Lammerding, Samuel Benchimol and Maria Eriksson. Front: Kan Cao, Judith Campisi (moderator), Karima Djabali and Thomas Dechat.

Jan Lammerding, PhD, Assistant Professor in the Dept. of Biomedical Engineering, Cornell University's Weill Institute for Cell and Molecular Biology, Ithaca, NY

"Vascular smooth muscle cell dysfunction in Hutchinson-Gilford Progeria Syndrome"

This study will test whether an increased sensitivity to mechanical stress is responsible for the progressive loss of vascular smooth muscle cells in HGPS. Insights gained will yield new information on the molecular mechanisms underlying the cardiovascular disease in HGPS and may offer new clues into the development of therapeutic approaches.



Karima Djabali, PhD, Professor of Epigenetics of Aging, Technical University of Munich, Germany

"Progerin dynamics during cell cycle progression"

Using anti-progerin antibodies and HGPS cellular models, this study will identify progerin direct effectors within the cell's nucleus to determine the initial molecular interactions that are disturbed by progerin expression. Insight gained from this study will permit the identification of new therapeutic targets for HGPS treatment and new cellular endpoints for testing the efficacy of potential interventions.



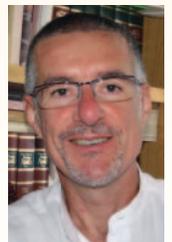
Vicente Andrés García, PhD, Senior Investigator, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology, Dept. of Epidemiology, Atherothrombosis and Imaging, Centro Nacional de Investigaciones Cardiovasculares, Madrid, Spain

"Quantification of farnesylated progerin and identification of genes that activate aberrant LMNA splicing in Hutchinson-Gilford Progeria Syndrome"

This project will develop a method to routinely and accurately quantify progerin expression and its level of farnesylation, and the ratio of progerin to mature lamin A, in HGPS cells.

Measurement of these parameters will help assess the effectiveness of drugs targeting progerin farnesylation, as well as that of future strategies devised to inhibit abnormal processing (splicing) of the LMNA mRNA, the cause of HGPS

in most patients. A secondary objective is to perform pilot studies for the development of a high-throughput strategy to identify mechanisms that activate aberrant LMNA splicing.



Samuel Benchimol, PhD, Canada Research Chair in Biomedical Health; Chair, Department of Biology, York University, Toronto, Canada
“Involvement of p53 in the premature senescence of HGPS”

This research will test novel hypotheses regarding the role of p53 in mediating the premature senescence shown by HGPS cells. The first objective is to test the hypothesis that progerin causes replication stress, which in turn elicits a senescence growth arrest, and that p53 acts downstream of the progerin-induced replication stress. This objective is followed by a more mechanistic aim designed to determine how progerin and p53 collaborate to elicit a senescence response.



Thomas Dechat, PhD, Assistant Professor, Max F. Perutz Laboratories, Medical University of Vienna, Austria
“Stable membrane association of progerin and implications for pRb signaling”

This study will identify the mechanisms responsible for anchoring progerin to the nuclear membrane, and to find ways to inhibit this anchorage by rescuing the dynamic lamin pool and thereby reverting cellular phenotypes associated with HGPS. In addition this research will study the effects of progerin on the regulation, dynamics and activities of the mobile, nucleoplasmic lamin A pool and its associated proteins, and its impact on pRb signaling at molecular detail. The results are expected to shed light on the disease-causing molecular mechanisms behind HGPS, and may help to identify novel drug targets and drugs for more efficient and targeted therapies.



Maria Eriksson, PhD, Assistant Professor at the Department of Biosciences and Nutrition; Associate Professor in Medical Genetics, Karolinska Institute, Solna, Sweden
“Analyzing the possibility for Progeria disease reversal”

Progeria disease progression will be monitored in the bone tissue at different time points following inhibition of the gene mutation in the bone tissue, to analyze possible disease reversal. The desired outcome is improved clinical symptoms and identifying a possible treatment and cure for this disease.



Colin L. Stewart, D.Phil, Senior Principal Investigator and Assistant Director, Institute of Medical Biology, Singapore
“Defining the molecular basis to vascular smooth muscle deterioration in Progeria”

Dr. Stewart and Senior Research Fellow Oliver Dreesen will study how progerin affects the growth and survival of the smooth muscle cells in blood vessels. The goal is to discover what type of DNA is damaged and what biochemical processes necessary for the survival of the smooth muscle cells are affected by progerin.



Dylan Taatjes, PhD, Associate Professor, Molecular Biophysics Program, University of Colorado, Boulder, CO
“Comparative metabolic profiling of HGPS cells and evaluation of phenotypic changes upon modulation of key metabolites”

This study will perform a comprehensive, comparative screen of the metabolites present in cells derived from healthy donors and HGPS patients. Follow-up biochemical and cell-based assays will establish whether key metabolites identified in the screen can induce HGPS phenotypes in healthy cells, or reverse HGPS phenotypes in diseased cells. The research will also begin to evaluate whether targeting these pathways represents an effective approach for therapeutic intervention.



Thomas Misteli, PhD, Senior Investigator and Associate Director, National Cancer Institute at NIH, Bethesda, MD
“Small molecular discovery in Hutchinson-Gilford Progeria Syndrome”

This research focuses on interfering with the production of the disease-causing progerin using molecular tools. This work will attempt to find novel molecules to counteract the detrimental effects of Progeria in patient cells, leading to a detailed biological understanding of Progeria cells and a molecular therapy. Dr. Misteli was also awarded funds to purchase robotic laboratory equipment to identify lead compounds for HGPS drug development.



Tom Glover, PhD, Professor, Department of Human Genetics and Pediatrics, University of Michigan, Ann Arbor, MI
“Identifying Genes for Progeria and Premature Aging by Exome Sequencing”

This study hypothesizes that mutations responsible for atypical progeria can be identified by whole exome sequencing of patient samples. Identifying these mutations is essential to understanding the disease etiology, developing effective treatments, and developing knowledge of intersecting and interacting molecular and cellular pathways in progeria and normal aging. Because of overlapping features, the findings could impact HGPS, other forms of progeria and normal aging.



See “Life According to Sam” on HBO October 21st!

Life According to Sam is the story of an extraordinary child with Progeria, his parents' (PRF co-founders Drs. Leslie Gordon and Scott Berns) search for a cure, and the family's inspirational perspectives on living life to its fullest. Directed by Oscar®-winning filmmakers Sean Fine and Andrea Nix Fine, the 90-minute documentary will air on HBO starting October 21st. We are thrilled that this film will bring awareness of Progeria and the need to find a cure to millions around the world.

The film made its US debut in January at the 2013 Sundance Film Festival, and has been showing at film festivals in Canada and throughout the United States. Visit progeriaresearch.org/lifeaccordingtosam to see rave reviews, fun photos, moving audience reaction, and more.

Host a Broadcast Premiere Party!

This is your chance to bring the Red Carpet to your home! Host a *Life According to Sam* Premiere Party at your home, invite friends and family to watch the film with you and help us make the premiere PRF's biggest one-night event! Contact agordon@progeriaresearch.org to learn more about booking your party.



Join in the conversation
[#lifeaccordingtosam](https://twitter.com/lifeaccordingtosam)

What people are saying about the film:

“Many thanks for phenomenal #LifeAccordingToSam film about @Progeria tonight! So inspiring and amazing!”

“Powerful film on life, love, hope, family, medicine.”

“...the sweetest, life-affirming film... Stunning and thought-provoking.”



The filmmakers at the Sundance Film Festival premiere: Sound/Cameraman Pablo Durana, Associate Producer Anne Marler, Editor Jeff Consiglio, Producers/Directors Andrea Nix Fine & Sean Fine, and Composer Keegan Dewitt.



(Left to Right) Nancy Abraham, Senior Vice President of HBO Documentary Programming; Sheila Nevins, President of HBO Documentary and Family Programming; Scott, Sam, Leslie, and filmmakers Andrea Nix Fine & Sean Fine celebrate the film's completion.



At the Boston premiere reception, Sam talks about the film with Kathy Behrens, NBA Executive Vice President of Social Responsibility and Player Programs.



Sam greets Boston Bruins players Andrew Ference and Milan Lucic, and Brittany Lucic at the Boston HBO Premiere in March.



Roger Berkowitz, President and CEO of Legal Sea Foods, with Scott Berns, Sam Berns and PRF Executive Director Audrey Gordon.

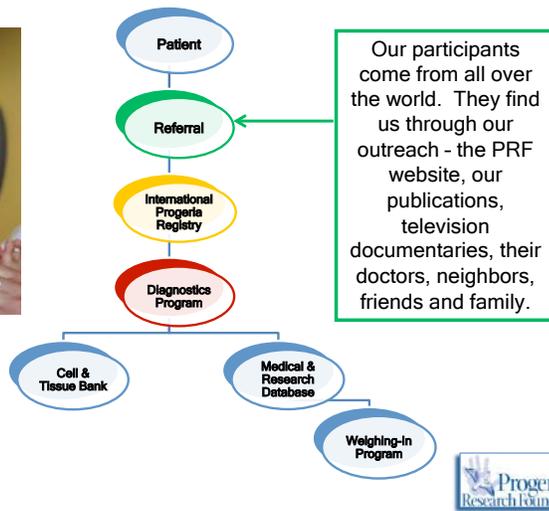
Introducing PRF By The Numbers

Launched in May 2013, PRF By The Numbers is an easy-to-read data sharing tool* originating from The Progeria Research Foundation's programs and services.

Data collected from within our programs is presented in charts and graphs to track our progress year to year, so you can see where we've been and the fantastic advancements we've made for children with Progeria. It is provided as a slide presentation that you can download at www.progeriaresearch.org/prf-by-the-numbers

*The National Institutes of Health states, "data sharing is essential for expedited translation of research results into knowledge, products and procedures to improve human health."

PRF Programs: It All Starts With The Children



Slide #7 provides an introduction to PRF's programs.

Everyone benefits from knowing and learning as much as possible about Progeria and PRF's programs:

- Families and children with Progeria
- The general public and nonscientists of all ages, including students creating reports on Progeria
- Scientists
- Physicians
- The media

We hope you find this new resource useful for your research, school project or story, or just to learn more about children with Progeria and what we're doing to find a cure for them.

Number of Living PRF-Identified Cases

As of April 2013:

Total Number of Children with Progeria Worldwide: **103**

HGPS* worldwide: **90**

HGPS* in the United States: **18**

Progeroid Laminopathies** worldwide: **13**

Progeroid Laminopathies** in the United States: **5**

*Children in the HGPS category have a progerin-producing mutation in the LMNA gene
 ** Those in the Progeroid Laminopathy category have a mutation in the lamin pathway but don't produce progerin



Slide #12, followed by slides showing the countries the children reside in and the languages they speak.

PRF Diagnostics Program

Program Goal:

- Genetic Sequence Testing for Progeria-causing mutations

Pre-requisites for Testing:

- Registration with PRF International Registry
- Possible indications for genetic testing

- Proband, prenatal - family history
- Proband, postnatal - clinical presentation
- Relative of positive proband



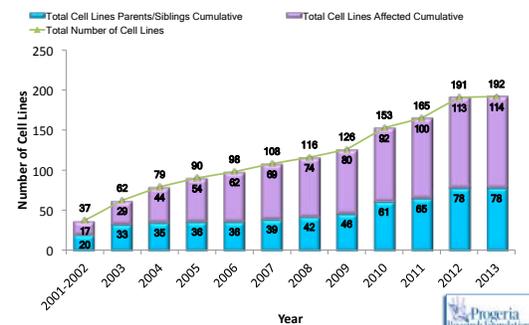
Testing information available at

www.progeriaresearch.org/diagnostic_testing



Introductory program slide, followed by slides showing the number and types of tests performed, mutations identified and a graph incorporating all figures.

Number Of Cell Lines By Year



One of seven slides describing data from the PRF Cell & Tissue Bank.

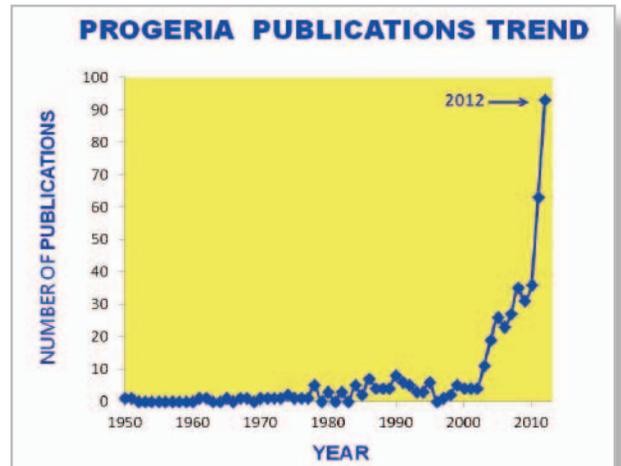
PRF 2012 Annual Report

So much accomplished, lots more planned to get us to the cure!

Given the mid-year timing of this newsletter, we are pleased to include our 2012 annual report.

PRF's programs are thriving and expand annually with increasing numbers of children identified and researchers involved.

From 2011 to 2012, our program growth continued at an impressive pace. This includes a 16% increase in the number of known children, 48% increase in peer-reviewed scientific publications, and 119% increase in public awareness – **wow!**



10 Core PRF Activities That Support Our Mission - All Grew in 2012

2012 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY
21 children from 6 countries	International Patient Registry	181 children from 45 countries
6 children diagnosed from Argentina, India, Philippines, U.S.	Diagnostic Testing Program	106 children diagnosed
26 cell lines from donors; cells sent to 12 teams in 6 countries	Cell & Tissue Bank	191 samples from 173 donors; cells sent to 54 teams in 12 countries
6 children enrolled; 58 treatment guidelines sent to 10 countries	Medical & Research Database	127 enrolled; 440 treatment guidelines sent to 36 countries
Funded Triple Treatment Trial	Clinical Trial Funding & Co-coordination	3 clinical trials funded since 2007
7 grants awarded	Basic Research Grant Funding	45 grants to 33 labs 11 countries; funding total >\$4 million
"New Frontiers in Progeria Research" – Subspecialty Meeting	International Scientific Meetings	10 meetings: 6 general workshops and 4 sub-specialty
1 new chapter and over 300 new volunteers	Volunteer Program	8 chapters and >2500 volunteers worldwide
6 languages added	Translations Program	26 languages
14 new children identified and 444 million people reached through media	Public Awareness & Find The Other 150 Campaign	100 children from 37 countries; over 1 billion people exposed to Progeria and PRF's work

2012 Highlights:

The discovery of the first treatment for Progeria made 2012 a defining year for PRF. The study proved the effectiveness of the FTI drug lonafarnib, one of the most significant steps toward our mission to find treatments and a cure. We now know that Progeria can be influenced in a positive way by medication. Like the historic 2003 gene discovery, the doors of scientific possibilities have been flung wide open. PRF will continue its work with drug discovery and clinical trials in the search for even more effective treatments and, ultimately, the cure.

“The breakthrough here is that we have findings that show Progeria can be altered, that drugs can have an impact on the disease... we have broken open the first of many possibilities for the children”.

- PRF Medical Director Dr. Leslie Gordon, quoted by National Public Radio and CNN Health on the September 2012 Progeria treatment discovery.

The number of children identified reached a milestone with a record 100 children living with Progeria. The number of children has more than tripled, and the number of countries more than doubled, since PRF's inception.

Two more translations of the Progeria Clinical Care Handbook are now available. This portable book provides caregivers vital treatment guidelines for children with Progeria. In 2012, we created Portuguese and Russian translations to add to our English and Spanish versions.

Seven new research grants were awarded in 2012 – an all-time high - with 12 active projects. This was the first full year of implementing our 3-tiered, increased funding structure, designed to foster both new and established investigators who will lead the charge for Progeria's future scientific discovery. It's working! Our grantees are changing the landscape of Progeria research and giving the world ever-increasing hope for treatments and cure.

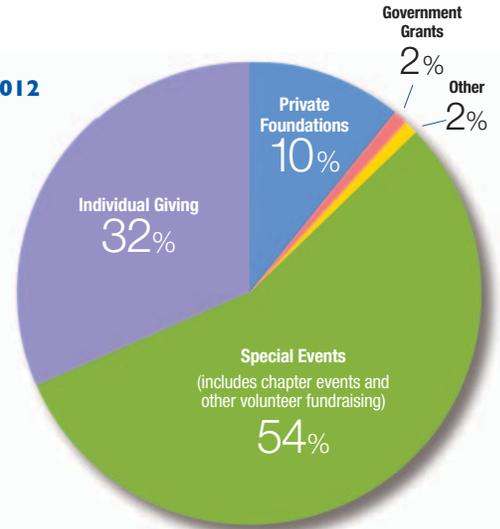
The exponential increase in published Progeria studies that both advance the field and demonstrate Progeria's connection to aging continues. The number of peer-reviewed scientific publications increased from 63 in 2011 to 93 in 2012. PRF's critical impact on Progeria-related research is evident. More than half of new articles were published by scientists who have been funded by PRF.

Public awareness reaches new heights as print, internet and broadcast media coverage of Progeria and PRF reached nearly 444 million* people through hundreds of pieces that aired worldwide. Moreover, our web site had 424,000 visits – an 80% increase from 2011 - and 74% of them were new visitors.

*Number reflects total reach of media publications; number of actual readers of each story is unknown.

INCOME ANALYSIS 2012

TOTAL REVENUE
\$1,806,700



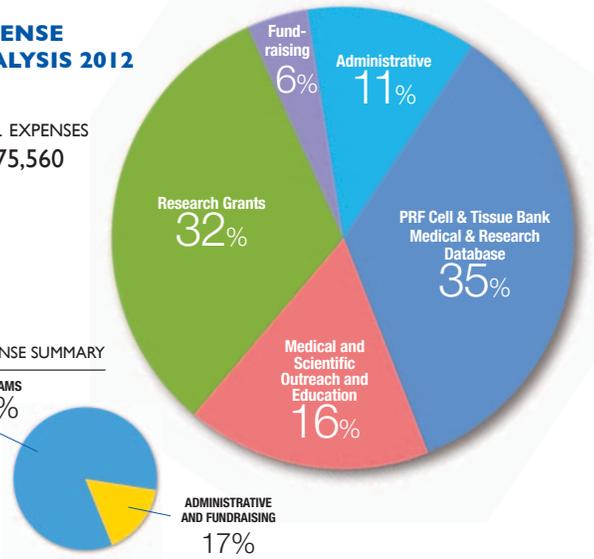
EXPENSE ANALYSIS 2012

TOTAL EXPENSES
\$1,575,560

EXPENSE SUMMARY

PROGRAMS

83%



ADMINISTRATIVE AND FUNDRAISING
17%

The Wall Street Journal article about the treatment discovered through the first clinical drug trial – one of dozens of top tier media outlets that reported on this historic finding.



PRF ON THE MOVE!

Our outreach efforts continue to increase awareness of Progeria and PRF's work, as we strive to find all children with Progeria in the world and engage as many partners as possible in our quest for the cure.

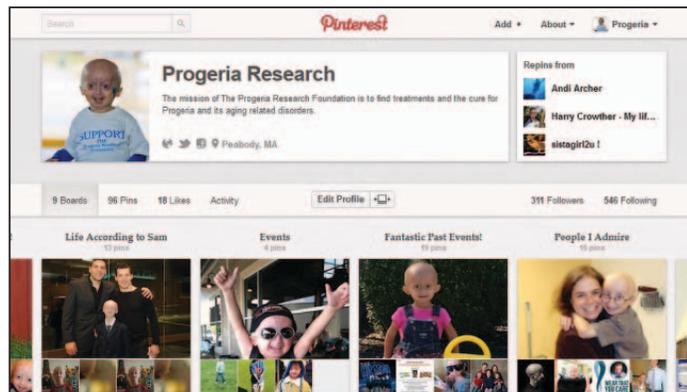
“Find The Other 150” Reaches a Milestone

In November 2012, we reached an historic number in our effort to identify all children with Progeria throughout the world: **100!** As of June 2013, that number has climbed to 103 children from 37 countries speaking 25 languages. This represents a 91% increase over the past 3 ½ years – astonishing! Experts say there are 200-250, and so our outreach efforts continue. “Like” our new Facebook page, “[Find The Other 150 Campaign](#),” where you can learn about the children and brainstorm ways to reach our goal.



Social Media Update: Growing by Leaps and Bounds!

PRF's social media presence is increasing at a phenomenal rate: over 30,000 **Facebook** followers, 1,000 people **tweeting** about Progeria and 3 million viewers of 35 videos on **YouTube!** This year we added 3 new ways to connect with the world through **Instagram** (@progeriaresearch), **Google+** (Progeria Research Foundation) and **Pinterest** (progeriaesrch). Go to progeriaresearch.org where 30,000 people visit monthly, and click on the social media icons to enjoy the latest PRF news and join the conversations!



Snapshot of PRF's Pinterest page.



Instagram

Start Your Engines!

Thanks to two talented supporters, awareness of Progeria and PRF is penetrating the drag racing and motorcycle racing worlds. **Erica Enders**, who made history last summer as the first female to win an NHRA Pro Stock race, filmed a public service announcement* with Zach Pickard that aired on dozens of stations in the US. And champion AMA Pro XRI 200 Series rider **Kyle Wyman**



Kyle with Zach and Cam at the Indianapolis Speedway last summer.

did the same with Zach and his buddy Cam. They also mention Progeria during TV and radio interviews, and post and tweet about PRF to their fans, urging them to support PRF's work. Thanks Erica, Kyle, GK Motorsports, KLR Group and Gaston Kearby for helping us get ever-closer to our own finish line – **the cure!**

*View these PSA's and lots of other great videos on our YouTube channel: Progeria | 23.



Erica and Zach share a special hug during the PSA filming at the Charlotte Motor Speedway.

International Sub-specialty Meeting – “New Frontiers In Progeria Research”

In January 2012, PRF convened a specialized meeting of high level researchers not currently funded by PRF, and either new to the field of Progeria or not yet working in it. Designed by the PRF Medical Research Committee, the goal of this 2-day, highly interactive meeting was to stimulate and broaden research by involving researchers qualified to fill essential “holes” in the current field. We thank the participants for accepting this opportunity to brainstorm how to address important unanswered questions towards saving children with Progeria.



*Left to Right: Brian Schreiber, MD, V.P. Medical Affairs, Sigma-Tau Pharmaceuticals, Maryland; Ronald Kahn, MD, Senior Investigator, Harvard and Joslin Diabetes Center, Boston, MA; Andrei Gudkov, PhD, DSci, Director & Chief Scientific Officer, Cleveland BioLab, Buffalo, New York; Valerie M. Weaver, PhD, Prof. Surgery, Dir. Therapeutics, University of California San Francisco, CA; *Tom Glover, PhD, Professor of Human Genetics, University of Michigan; Adrian R. Krainer, PhD, Chair, Cancer and Molec. Biology, Cold Spring Harbor Lab., New York; *Judy Campisi, PhD, Senior Scientist, Lawrence Berkeley National Laboratory, CA; *Ted Brown, MD, PhD, Director, NY State Institute for Basic Research in Developmental Disabilities; *Leslie Gordon, MD, PhD, Medical Director, PRF, Brown U. Harvard U.; *Bryan Toole, PhD, Prof. Cell Biology and Anatomy, Medical University of South Carolina; *Christine Harling-Berg, PhD, Brown U.; Walter Cabri, DCh, R&D Chemistry, Director of Analytical Development, Sigma Tau, Rome, Italy Vicente Andres Garcia, PhD, Centro Nacional de Investigaciones Cardiovasculares, Madrid, Spain; *Frank Rothman, PhD, Professor Emeritus, Brown U.; *Tom Misteli, PhD, Senior Investigator, National Cancer Institute; Jamal Tazi, PhD, Professor, Centre Nationale de Recherche Scientifique, Montpellier, France; *Monica Kleinman, MD, Critical Care and Anesthesia, Boston Children’s Hospital, Harvard; (not shown - Jeffrey Chamberlain, PhD, PI Dept of Neurology, University of Washington, Seattle, WA and Dir., Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Center).
PRF Medical Research Committee member

PRF’s Medical Director Educates and Broadens Awareness at Conferences and Hospitals



Dr. Leslie Gordon travels the world to educate families and the medical community on advances in Progeria research. Most recently, Dr. Gordon spoke at the following:



Pfizer’s Orphan and Genetic Disease Summit, Cambridge, MA: “Saving Children with Progeria: the Journey from Obscurity to Treatment and the Quest for a Cure.”



Hasbro Children’s Hospital Grand Rounds in Providence, RI: “The Progeria Journey – Finding Treatments and Cure for A Rare and Fatal Disease.” (This 1-hour presentation can be viewed at progeriaresearch.org/grand_rounds)



American College of Medical Genetics, March of Dimes Plenary Session Charlotte, NC, “Hutchinson-Gilford Progeria Syndrome: Translational Medicine From Gene Discovery to Treatment Trials.”



Centro Nacional de Investigaciones Cardiovasculares Conference in Madrid, Spain: “The Progeria Story – A Model for Translational Research.”



Italy Progeria Reunion, Montegrotto Terme, Italy: “Clinical Trial of a Farnesyltransferase Inhibitor in Children with HGPS.”

Chapter Events

All We Can Say is WOW! Our hardworking, dedicated chapters, whose races and other events continue to grow each year, have been vital to our fundraising and awareness efforts. Based in California, Kentucky, Michigan, New Jersey, Ohio, Pennsylvania and Texas, many of you have supported these events each year – THANK YOU!

Interested in starting a chapter? Let's talk!
Email Michelle at mfino@progeriaresearch.org

A Texas-sized Welcome to Our 8th Chapter!

Team Adalia, the TX Chapter of PRF, will be led by Adalia's parents, Natalia Amozurrui and Ryan Pallente. They're



planning lots of fun events – check our website for more details.

The TX Chapter Board: Raven, Ryan, Rebecca, Rosa, Gloria, Natalia and Adalia (missing: Events Coordinator Edie).

Zach Attack Golf Scramble – Nicholasville, KY

The KY Chapter held their 2nd Annual golf scramble at the beautiful Golf Club of the Bluegrass, where 22 teams teed off to support children with Progeria. Join Zach and friends at this year's Scramble in the fall, and watch for pancake breakfasts, Harley rides and more throughout the year!

Looks like Zach could teach Tiger a few things about golf!



Carly's Party...

FUN for a Cure in Maumee, OH

Held at The Pinnacle and with the help of 50 volunteers led by chair Jennifer Smith, 400 guests raised over \$50,000 to support Progeria research. The Ohio chapter event featured a silent auction, raffle, door prizes and music by Alter Ego.

Save the Date
for Carly's Party
2013!



YEARONE - FOOSE Bash – Braselton, GA

The GA chapter put its pedal to the metal last September when YearOne and automotive guru Chip Foose hosted this popular weekend car show. Over the past 7 years, YearOne and its Hot Rodders Children's Charity have raised over half a million dollars to help find a cure for children with Progeria. Come join them at this year's show September 21.



Chip Foose captivates the crowd with his extraordinary drawings – art in motion!

7th Annual Kaylee's Course – Monclova, OH

It was a perfect day for this Ohio chapter 5K Run/2 Mile Walk. Participants enjoyed refreshments, silent auction, quilt raffles and more – and they're all looking forward to the next one October 12th.



Zach, Cam, Kaylee and Carly had a great time!

Kilometers for Cam – St. Joseph, MI

The Whirlpool Corp. Healthworks Program joined the Southwest MI Chapter for the 5th Annual Kilometers for Cam last September, and what a day it was! With a 100% increase in participants – from 500 in 2011 to 1,000 in 2012! – they raised over \$65,000, nearly double from the year before as well. Cam and friends hope to see you this year on September 14th!



Runners are ready to start the race!

Annual & Other Events

Spin-for-Life Expands in Every Way

The NJ chapter added a 2nd location to their annual spin-a-thon, resulting in 200 cyclists pedaling their way to raise a



record \$42,000! The 30-minute sessions were full to capacity, with cyclists of all experience levels participating, and once again Karen Carolonza spun for the entire 4 hours – you have until January to train and join her!

NJ Chapter leader Barbara Batesko (on the right) and Karen pedal for Progeria!

Team Zoey Golf Tournament & Birthday Celebration – Wayne, NJ

For the 3rd year in a row, the Preakness Hills Country Club hosted 100 golfers on the course and at an auction-dinner reception, raising \$165,000! Zoey had a great time too, celebrating her 3rd birthday – come wish Zoey a happy 4th birthday at this year's tournament on October 3rd.

8th Annual Miles for Miracles – Flat Rock, MI

The unseasonably cool weather didn't stop hundreds of Lindsay's family and friends from having a great time at the MI Chapter's signature event. Lindsay designs the t-shirt every year, and we love this year's "Keep Calm and Walk On" motto!



Lindsay leads the crowd.

This is just a sampling of the many Chapter events that take place throughout the year. We hope you can join us soon for one or more!

Here are just a few Special Events that took place since our last regular newsletter. Visit our website for details on others, and information on upcoming activities.

Let us know if you want to organize an event – we can show you how!

We Want the Airwaves Benefit Concert Rocks Boston!

It was a full house at The Paradise Rock Club for this WFNX Tribute. Lots of music and dancing, with the \$15,000 in proceeds donated to PRF. Thank You to the station, the bands and the music fans – **ROCK ON**

2nd Annual Make a Splash – Flourtown, PA



The Falcone family had a great day at the pool.

It was "everyone in the pool" as 325 swimmers joined Nathan and Bennett at the town's country club to swim, play, bid on silent auction and raffle items, delight in the balloon artist's creations and enjoy tasty cookies. Lots of summer fun – and \$18,000 for PRF! Save the Date and a dry towel for this year's event on September 7 – **MAKE SOME WAVES!**

6th Annual Pelican Run – Brownstown, IN

It was another fabulous year for the Zach Attack Pelican Run, with perfect weather and the Brownstown Electric Supply Company team out in full force. Thank you to owner Carl Shake and the entire BESCO family – **YOU'RE FLYING!**



Zach, his parents and the Pelican statue the event is named after.

Our progress toward a cure is made possible by funds raised at these volunteer-driven events. **THANK YOU!!!**

11th Annual International Race for Research - Peabody, MA

Last year, an astonishing 420 walkers/runners came out to support PRF – 30% more than 2011. With perfect weather and a sea of athletes wearing bright race t-shirts visible from a mile away, we also broke the \$25,000 mark! Our 2013 race is September 7th – **ON YOUR MARK, GET SET, GO!**



Team Champions, winner of the 2012 Team Award.

1st National Hats ON for Progeria Day EVERYWHERE!

Our inaugural Hats ON day was a huge success, raising nearly \$12,000 with over 1,000 people wearing PRF or other hats to work and school in 14 states and the UK. If you missed it, you can still hold your own event any time – visit progeriaresearch.org/hats-on-for-progeria for details. Next year, we're hoping for Hats ON participants in every state and 5 other countries – **LET'S DO THIS!**



Hats off to the Texas Office of National Vendor!



NEW! PRF hats, in khaki and blue. Buy yours today at progeriaresearch.org/shop_in_our_store

Night of Wonder!

Our 6th Night of Wonder theme was **Color me a Cure!**

It was an unforgettable evening that raised nearly \$300,000, thanks to hundreds of volunteers, auction donors, sponsors, advertisers and guests. From the blown-up drawings made by children with Progeria, to the presentation of the 1st SAM (Science And Medicine) Award to NIH Director Francis Collins, to the appearance of honorary co-chairs (Boston Bruins' Milan Lucic and Andrew Ference), to the heartwarming appeal by Carly's mom, it was truly a WONDERful evening.



Gala chair Vickie Robbin with Milan Lucic.



Debbie Ponn, who will chair the 2014 gala, with Andrew Ference.



Sam presents Dr. Collins with the Science And Medicine (SAM) Award.



Part of the banner thanking our major sponsors: The Berns Family, Gretchen & Bob Morrison, Debbie Ponn, and Elaine & Skip Wohlner.

SAVE THE DATE for Night of Wonder 2014
Lights, Camera, CURE!
April 12, 2014 at the Royal Sonesta, Cambridge, MA.

Join us as we celebrate PRF's accomplishments and raise money to support the programs that will get us to the cure. **Night of Wonder 2014** will cap an exciting year of HBO premieres, broadcasts, and the DVD release of *"Life According to Sam"* (see page 10). Our NOW 2014 committee is hard at work planning PRF's signature, major fundraiser: **Watch for details... and...ACTION!**

PRF Miracle Makers

Through their love of sports, fashion, birthday celebrations and more, our young volunteers continue to inspire and amaze us with their kindness and generosity. Thank you all, for making a *miraculous* difference in the lives of children with Progeria.

Cherry Hill Schools Volley for PRF

Thank you to Steve Redfeam and everyone involved in the Annual "Volley for Support" Tournament between staff of the PreK-12 Cherry Hill Township New Jersey Schools. The event featured raffles, food and lots of friendly competition. **We're glad they're on our team!**



Kentucky Student Entrepreneur Gets Creative With Raffle

Raffles are a common way to raise money, but this one's a first for us: 5th grader Macy created a booth called "CHILLIN' WITH ZACH" where she sold \$1 raffle tickets for a chance to eat

lunch with Zach, raising over \$50 for PRF. **Now that's using business sense for good!**

3 on 3 Basketball a Slam Dunk for PRF!

Thank you to Jessica and everyone involved in this year's annual basketball tournament at Seton Hill University in Pennsylvania. PRF was honored to be their chosen charity this year.

Flat Rock Michigan Rams Play for Progeria

The Rams held a softball tournament to raise awareness for Progeria and support Lindsay. Wearing "Rams Play for Progeria" t-shirts, every player was "Lindsay" for the day – **now that's a Home Run!**



Many schools and businesses hold periodic fundraisers and choose a different charity each time. Please suggest PRF if you can!



Gray Wolves Spike it for Progeria

The Lourdes women's volleyball program of Sylvania, Ohio set up collection cans and

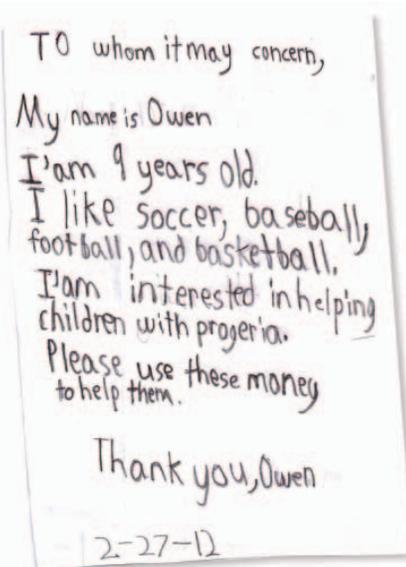
sold t-shirts at a recent match. Kaylee and her family had a ball watching the exciting games. **A great rally for PRF!**

Sweet 16 is a Treat for PRF

For Alexis's 16th birthday, instead of gifts for herself she selflessly asked friends and family to donate to PRF, which raised over \$275 – **Now that's sweet!**



The birthday girl (center, holding balloon).



Owen sent PRF a \$10 bill with this heartwarming letter.

Lots of Miracle Makers have had friends and family donate to PRF in lieu of gifts for weddings, Bar Mitzvahs, birthdays and Christmas. How thoughtful!

Please visit our Facebook page or www.progeriaresearch.org/miracle_makers to read about many more Miraculous people. We hope their stories inspire you to become a Miracle Maker, too!



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

RETURN SERVICE REQUESTED

To learn more about how you can help, contact us:
info@progeriaresearch.org
978-535-2594

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Alessandro hopes you're having a great summer!

It's time for ONEpossible 2013!

Where people, **ONE** at a time, make a **CURE** for Progeria **POSSIBLE**
Campaign runs through August 30th

The day has finally come! On July 14th, Zoey and Carly were the first of up to 40 children to enter the Triple drug Trial. This year's **ONEpossible** campaign kicks off raising the additional funds needed, paving the way for this exciting expansion of services.

Zoey and Carly's moms are co-chairing ONEpossible 2013. Please help them reach their goal of \$150,000 using the enclosed return envelope, or on-line at onepossible.org



THANK YOU for your support!

