2-Drug Clinical Trial in Full Swing!
Phase 1 going strong as children begin to enroll

With 9 children enrolled and more children anticipated to join through 2016, the trial team will soon know the optimal clinical trial dose of everolimus when given in combination with the drug lonafarnib. If the drug is tolerated, they and other children will move on to Phase 2, a two-year trial to determine if the two-drug combination is more effective than lonafarnib alone.

Read more about the PRF-funded Progeria clinical trials – our best hope for treatment and cure – on page 4.

International Scientific Workshop 2016
PRF’s 8th international conference an overwhelming success

Presenters from 14 countries showcased their progress in translating laboratory research into treatments, as researchers and clinicians work to find a cure for Progeria and unlock the mysteries of heart disease and aging.

More details on pages 6-7
PRF’s Mission:
To discover treatments and the cure for Progeria and its aging-related disorders, including heart disease.

Staff:
Meryl Fink, Esq., Executive Director
Audrey Gordon, Esq., Director of Development
Gina Incrovato, Clinical Trial Coordinator
Michelle Fino, Director of Volunteer Activities
Lynn MacKinnon, Development Director
Ana Valverde, Executive Assistant to the Med. Dir.
Reggie Cannon, Executive Assistant to the ED & Office Manager
Kim Paratore, Board Secretary

Corporate Officers:
Meryl Fink, Esq., President
Carl Alviti, CPA, Treasurer
Barbara F. Gordon, Esq., Clerk

Board of Directors:
Scott D. Berns, MD, MPH, FAAP, Chair
Carl Alviti, CPA
Karen N. Ballack, Esq.
Sandra Brennick, Esq.
John Marozzi
Larry Milke
Kim Paratore

Board of Advisors:
Roger Berlowitz
Monica Kleinman, MD
Barbara Batesko, MD
Marla & Tim Halko
Joe & Kristy Ratcliffe
Tina Pickard
Terry Foose

Chapters & Their Leaders:
California, Terry Foose
Kentucky, Tina Pickard
Rhode Island, Kristy Radcliffe
New Jersey, Barbara Batesko
Ohio, Mara & Tim Haiko and Heather & Ryan Fudala
Pennsylvania East, Phyllis & Mark Falcone
Pennsylvania West, Jim Schonover and Stephanie Howard

Legal:
Karen N. Ballack, Esq.
Welch, Goral & Nieves, LLP
Silicon Valley, CA
Sandra Brennick, Esq.
Quinn Emanuel Urquhart & Sullivan LLP, NY, NY

Accounting:
Carl Alviti, Marie Financial, LLC, Wellesley, MA

Web Site:
Karen Gordon Betsaumy, Coordinator
Boston Web Marketing, Technical/Design

Graphic Design:
Marie Migliaccio, Topsfield, MA
Julie Fritchard, CR Design, Foxboro, MA
 Regina Printing, Middletown, NJ

Medical Research Committee:
Brian F. Tuck, PhD, Chair
Vivante Andrea Garcia, PhD
Christine Harling-Berg, PhD
W. Ted Brown, MD, PhD
Judith Campisi, PhD
Maria Eriksson, PhD
Thomas W. Oliver, PhD
Leila B. Gordon, MD, PhD
Monica Kleinman, MD
Tom Misulis, PhD
Manfa Moses, MD, PhD

Chapters & Their Leaders:
California, Terry Foose
Kentucky, Tina Pickard
Rhode Island, Kristy Radcliffe
New Jersey, Barbara Batesko
Ohio, Mara & Tim Haiko and Heather & Ryan Fudala
Pennsylvania East, Phyllis & Mark Falcone
Pennsylvania West, Jim Schonover and Stephanie Howard

Legal:
Karen N. Ballack, Esq.
Welch, Goral & Nieves, LLP
Silicon Valley, CA
Sandra Brennick, Esq.
Quinn Emanuel Urquhart & Sullivan LLP, NY, NY

Accounting:
Carl Alviti, Marie Financial, LLC, Wellesley, MA

Web Site:
Karen Gordon Betsaumy, Coordinator
Boston Web Marketing, Technical/Design

Graphic Design:
Marie Migliaccio, Topsfield, MA
Julie Fritchard, CR Design, Foxboro, MA
Regina Printing, Middletown, NJ

Progeria is a fatal, “rapid aging” disease that affects children, who die of heart disease at an average age of 14 years – the same heart disease that afflicts millions of normal aging adults.

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

Message from Audrey Gordon and Meryl Fink
Hello everyone,
In this year’s newsletter, we are thrilled to present a special, 2-page spread of PRF’s timeline - highlights that tell the remarkable story of all that we have been able to accomplish with your support. The pages that follow depict not only PRF’s past history and successes, but also the activities that will lead us to new treatments and a cure, including:

In April the first children travelled to Boston to enroll in our third clinical drug trial one more step toward discovering treatments for Progeria.

The 8th international workshop held in May illustrated the astounding depth and breadth of new research being done around the world.

Our 8th Night of Wonder gala raised more funds for research than ever before, and brought the level of excitement and commitment of our supporters to new heights.

For the first time ever, annual funding for research grants exceeded $1,000,000.

We are also pleased to announce an exciting new set of staffing changes. As detailed on page 12, Meryl will soon become PRF’s Executive Director and Audrey will take on a new role as PRF’s Director of Development.

We are both eager to build on the amazing strides that PRF has made to date. From our very first board meeting in 1999 through today, the same vital ingredients continue to drive PRF’s mission - unwavering hope, determination, passion and commitment to finding a cure for children with Progeria. We and PRF’s Board of Directors are confident that these changes will ensure those ingredients endure, and that drive continues.

All of you have been an essential part of PRF’s history and successes, and we look forward to continuing our journey to cure Progeria with you.

Together, we WILL find the cure!

Audrey Gordon, Esq.
Meryl Fink, Esq.
The Progeria 2-Drug, Phase 1 Clinical Treatment Trial Has Begun

Studying will determine the safest maximum dose of everolimus for children with Progeria.

Our third clinical trial is now well underway at Boston Children’s Hospital! The excitement among the families, trial team and PRF staff is palpable as a new drug is introduced that may treat children with Progeria. The two-drug combination of lonafarnib plus everolimus (a form of the drug rapamycin) may prove to be a “one-two punch” to Progeria and thus a better treatment than lonafarnib on its own. Everolimus targets a different pathway in attacking the toxic protein progerin. While lonafarnib blocks progerin from developing, everolimus causes cells to more rapidly clear out progerin.

The first patients enrolled on April 25, 2016, and a total of 9 children have enrolled to date. Children from the US, Italy, South Africa, Sweden and Denmark are anticipated to join through the remainder of 2016.

Clinical Trials Protocol: Phase 1 vs. Phase 2

The goal of a Phase 1 portion is to determine the safest maximum dose of a drug. Once that occurs, the next study Phase 2 can begin, which determines if there are positive effects from the drug.

Lonafarnib Monotherapy Trial Expansion Continues

The US FDA requires patients to already be taking lonafarnib to be eligible for the 2-drug trial. Thus the lonafarnib-only expansion has continued not only for newly identified children, but also for those that began taking lonafarnib in 2009-2010 because it is improving their cardiovasculature and lifespan.*

Over the past year, 10 more children have enrolled, bringing the total to 78 from 33 countries, speaking 24 languages – REMARKABLE!

*In 2012, the historic finding was made that lonafarnib is the first known treatment for children with Progeria, significantly improving weight gain, bone structure and, most importantly, vascular stiffness. Further studies revealed lonafarnib increases estimated lifespan. PRF trials have made sure that children with Progeria have access to this treatment, while we continue the search for more effective treatments (like perhaps everolimus) and, ultimately, the cure.

Through the 2016 ONEPossible campaign, you helped raise $200,000 to fund the start of the 2-drug trial. Phase 1 will cost between $500,000 - $1 million, depending on when the right dosage is determined. THANK YOU for being ONE to make a cure for Progeria POSSIBLE!

Results of Triple Drug Trial for Progeria Published in high-impact journal Circulation

This clinical trial added two drugs, pravastatin and zoledronic acid, to the already successful drug lonafarnib. While lonafarnib continued to provide benefit, adding the two drugs did not improve health over and above the lonafarnib single therapy. PRF continues its aggressive strategy to identify promising drug candidates that may offer children with Progeria longer, healthier lives - like our new two-drug trial.

In an accompanying editorial, National Institutes of Health Director and study author Francis Collins, MD PhD, wrote: “...additional therapeutic options are emerging, and there is more momentum than ever in the basic and clinical research communities.”

Since 1999, PRF has granted over $7 million to fund 64 Progeria-related projects in 18 states and 13 countries. The projects have led to crucial discoveries about Progeria, heart disease, and aging.

SPECIALTY AWARD:

Jed William Fahey, Sc.D.
Assistant Professor and Director, Culman Chemothrombosis Center, Johns Hopkins School of Medicine, and Bloomberg School of Public Health, Center for Human Nutrition, Baltimore, MD.

“The capacity of plant-derived isothiocyanates to surpass the efficacy of sulforaphane, with reduced toxicity to Progeria cell lines.”

Recent studies have shown the chemical sulforaphane, which is found in broccoli, improves the health of cultured Progeria cells. However, the doses likely needed to treat people are much higher, and may cause unacceptable toxicities. Dr. Fahey will test over 100 compounds closely related to sulforaphane, to examine whether any are better at improving Progeria without causing cellular toxicity, at doses that may be reasonable for humans.

INNOVATOR AWARDS

Silvia Oroga-Gutiérrez, PhD
Associate Professor, Universidad Complutense de Madrid, Spain; Ramón y Cajal Scholar; Fubright Scholar; The Scripps Research Institute, La Jolla, CA.

“New isoprenylcysteine carboxylmethyltransferase (ICMT) inhibitors for the treatment of progeria.”

A chemical called an ICMT inhibitor has been shown to improve Progeria in the laboratory, by blocking the pathway used by cells to produce progerin. Dr. Ortega and her team aim to develop new ICMT inhibitors for the treatment of Progeria by performing what drug companies call “medicinal chemistry,” where a chemical is developed for use as a medicine in humans.

Roland Foisner, PhD
Professor of Biochemistry, Medical University of Vienna and Deputy Director, Max F. Perutz Laboratories, Vienna, Austria; Editor-in-Chief, Journal Nucleus.

“Contribution of endothelial cell dysfunction to cardiovascular disease in progeria and implications for diagnostic and therapeutic targets.”

Artery walls contain several layers containing different types of cells that can play different roles in cardiovascular disease development. Dr. Foisner has created a mouse model expressing progerin in the vascular endothelium, the innermost cell layer of blood vessels. He will investigate how progerin impairs endothelial cell function and how this affects heart function.

Juan Carlos Belmonte Izpisua, PhD
Professor, Gene Expression Laboratories at The Salk Institute for Biological Studies, La Jolla, CA.

“The use of novel technologies to identify and validate potential therapeutic compounds for the treatment of HMGP.”

Dr. Belmonte and his team will use a well-established technique pioneered in their lab to model the vascular smooth muscle characteristics associated with the most acute symptoms of heart disease in children with Progeria. Using these cells, they will perform a high-throughput screening to develop compounds that prevent progerin production.

PRF Awards Four More Research Grants

Since 1999, PRF has granted over $7 million to fund 64 Progeria-related projects in 18 states and 13 countries. The projects have led to crucial discoveries about Progeria, heart disease, and aging.

Zany, 6 years old, gets her first dose of everolimus from her mother, while Carly, 5 years old, watches and waits for her turn.

Brothers Bennett, 7 years old, and Nathan, 11 years old, are all smiles as they receive their award ribbons during their trial visit.

Morena, 8 years old from Argentina, began taking lonafarnib in December 2015. She is now eligible to participate in the 2-drug trial.

Meghan, 15 years old, Brenten, 7 years old, and Lindsey, 12 years old, participated in the triple trial. All three children have been taking lonafarnib for several years, and are now adding everolimus in the hopes that the 2 drugs will be more effective together.

PRF Newsletter
173 world-renowned researchers and clinicians in the field of Progeria converged from 14 countries to meet in Cambridge, MA in May for PRF’s 8th International Scientific Workshop entitled, Across the Table, Around the Globe. Twenty-five speakers and 46 poster presentations showcased important scientific findings, presented the progress in translating bench research to potential therapeutic treatments, and inspired future collaborations between the research and medical communities. The breadth and scope of work is expanding every year, as experts work tirelessly to find a cure for progeria and unlock the mysteries of heart disease and aging.

Scientists presented new data in support of over 20 possible therapeutic avenues for HGPS, a significant expansion over the prior meeting in 2013. There were wide-ranging discovery strategies that included RNA therapeutics, lamin A post-translational processing pathways, small molecules discovered as a result of high throughput screening, agents adapted from the general aging field such as autophagy and antioxidants; treatments rooted in LMNA structure and function, and a strategy for improving genomic stability. If all of these new discoveries lead to just one effective treatment for children with Progeria, then these global research efforts will be a tremendous success.

Overwhelmingly Positive Feedback!
With a 98% 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:

“The meeting was so much better than I had hoped for – I learned a tremendous amount, and felt very welcomed into your research community.”

“What a spectacular meeting!”

“I wanted to thank you for giving us the opportunity of being part of such a wonderful experience. Everybody was very committed to their work and to the families of these children, and that is admirable.”

“My head is still buzzing with a new appreciation for all the amazing science your foundation is sponsoring.”

The Meeting Agenda at a Glance

Evening 1: Children & Parents Living with Progeria: Toddlers & Teens
   Plenary Talk on landmark pre-clinical research findings: Vicente Andrés García, PhD; Centro Nacional de Investigaciones Cardiovasculares, Spain

Day 2: Clinical Outcomes and Biomarker Discovery in HGPS and Modeling
   Moderator: Monica Kleinman, MD; Boston Children’s Hospital, MA
   Pharmacological Intervention in HGPS and Aging Models
   Moderator: Brian Kennedy, PhD; Buck Institute, CA

Evenings 1 and 2 capped off with Poster Presentations of topics ranging from new potential therapeutic targets for treatment, to potential biomarkers and technology for non-invasive measures used to determine efficacy of treatment, to new discoveries in atypical progeria patients, to recent findings from clinical trials.

Day 3: Molecular and Cellular Mechanisms in HGPS and Aging
   Moderator: Maria Eriksson, PhD; Karolinska Institute, Sweden
   Emerging Therapeutics
   Moderator: Tom Mistelli, PhD; National Cancer Institute, MD

Summation: The Next Phase - Strategies for the Future; Science and Medicine Coming Together.

In a riveting Summation Session, Judy Campisi, PhD (Buck Institute), Mark Koran, MD, PhD (Boston Children’s Hospital) and NIH Director Francis Collins, MD, PhD spearheaded a lively discussion about how to work as a community to guide the future for progeria research.

As a fitting closure to the conference, Dr. Collins performed the song Dare to Dream, which he previously wrote and performed during his TEDMED talk that featured Sam Berns.

Many thanks to our Workshop Organizers…
Leslie B. Gordon, MD, PhD, Medical Director; PRF
Audrey Gordon, Esq, President & Executive Director; PRF
Frank G. Rothman, PhD, Professor & Provost Emeritus, Brown University
Tom Mistelli, PhD, Senior Deputy Director for Research, Center for Cancer Research, NCRI, NIH
Vicente Andrés García, PhD, Professor, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology, CNIC

...and our Workshop Supporters

NATIONAL INSTITUTE ON AGING
NATIONAL HEART, LUNG, AND BLOOD INSTITUTE

DSF Charitable Foundation
The Max and Victoria Dreyfus Foundation, Inc.

Hologic cares

Carly Cares

Cross the Tables Across the Globe

Across the Table, Around the Globe

For more details please visit progeriaresearch.org/workshop2016
We’ve come so far in such a short time, and with your continued support, Together, we WILL find the cure!

1999
March 1999
The Progeria Research Foundation (PRF) is incorporated as a Massachusetts non-profit organization. On June 9, 1999, the Board of Directors holds its first meeting.

August 1999
PRF awards its first research grant. To date, PRF has awarded 62 grants totaling over $7 million. The programs finding that the field reached towards treatment and cure, and have helped Progeria scientific publications increase by 2,200% since PRF was founded.

2000
January 2000
PRF launches its International Registry, a comprehensive resource of information on Progeria for researchers, families of children with Progeria and their physicians, and the general public. Together with social media sites (over one million Facebook followers), millions around the world follow PRF’s progress regularly. www.progeriaresearch.org

2001
November 2001
PRF holds a historic, first-ever workshop in Bethesda, Maryland. PRF has organized 11 successful scientific conferences that have brought together scientists and clinicians from all over the world to share their expertise and cutting-edge scientific data, and foster collaboration in the fight against Progeria.

2002
January 2002
The PRF Genetics Consortium is formed. The goal of this group is to find the gene for Progeria, which was accomplished ten months later.

March 2002
PRF launches the Cell & Tissue Bank at Rhode Island Hospital and Rutgers University Cell & DNA Repository, so researchers have the biological tools needed to advance Progeria research. The Bank now holds an impressive 21 lines, including cutting-edge induced pluripotent stem cells (iPSCs) at the Ottawa Hospital Research Institute.

PRF launches the Progeria Medical & Research Database at Brown University to help learn more about the disease and provide treatment recommendations. Four years later the Database information helped define the primary clinical parameter for Progeria clinical drug trials.

2005
November 2005
A record 16 scientists from 9 countries meet for PRF’s fourth scientific workshop in Boston (MA, by Dr. John D. Cryer), the number of participants and countries nearly doubled. With leading scientists in the fields of heart disease, aging genetics, and lipids, the depth and breadth of research into Progeria grow stronger with every meeting.

2006
June 2006
PRF launches a public service announcement (PSA) campaign featuring the recognizable voices of long-time PRF supporters, Ted Danson and Mary Steenburgen. The PSA received endorsement by the Ad Council and aired in Times Square. PRF has since created many PSAs with other celebrities, including Boston Bruins hockey player and Dave Matthews.

February/July 2006
PRF-funded studies find that farnesyltransferase inhibitors (FTIs) are a potential drug treatment for Progeria as they prevent some signs of disease in Progeria mice. These and other studies along with data analyzed from PRF’s Medical & Research Database, pave the way for a clinical drug trial using the FTI lonafarnib.

2007
April 2007
Boston Children’s Hospital approves the first-ever Progeria Clinical Drug Trial funded and co-coordinated by PRF. For the first time, we have a possible treatment for children with Progeria. 28 children with Progeria from 16 countries come to Boston to enroll in the 2-year clinical trial. Enrollment took less than 6 months, thanks to PRF’s International Registry and the remarkable teamwork with families, their doctors, and trial partners.

2008
August 2008
PRF’s 7th chapter opens in Kentucky. Chapters, as well as thousands of other volunteers worldwide, help raise awareness and conduct local fundraising events so that PRF’s goal of developing treatments and the cure will be achieved more rapidly to help those who need it most.

2009
April 2009
In a spectacular show of support for children with Progeria, PRF’s first annual campaign, called the ONe possible campaign reaches its $100,000 goal. To date, ONe possible supporters have raised $1.2 million (each ONE will make a cure POSSIBLE). August 2009
Clinical Trial #2: Triple Drug Therapy begins. PRF and Boston Children’s Hospital begin a three-drug combination trial. Researchers have identified two additional drugs that, when used in combination with the FTI drug being tested, may provide an even more effective treatment for children with Progeria than FTIs alone. This much larger trial involves 45 children from 24 different countries.

2010
October 2010
A landmark study led by PRF’s medical director in partnership with the National Institutes of Health, demonstrates that the Progeria-causing protein works and increases in everyone as we age. The study suggests a new risk factor for heart disease and confirms that by examining one of the rarest diseases, we can gain insight into the heart disease that affects millions, and the aging affecting us all.

2011
September 2011
PRF establishes Investigator Innovator, and Specialty Awards for basic research. This new grant program structure increases funding towards finding new treatments and cures for Progeria and aging-related diseases, including heart disease.

2012
September 24, 2012
FIRST EVER TREATMENT FOR PROGERIA DISCOVERED: PRF makes history with the discovery that lonafarnib is the first-ever treatment for Progeria. Every child in the clinical trial showed improvement in one or more areas, including the vital children’s quality of life.

2013
January 2013
HBO Film’s Life According to Sam (LATS) debuts at the Sundance Film Festival featuring Sam Berns and his parents (PRF founders) Drs. Leslie Gordon and Scott Berns, this riveting documentary about love, life, and hope for children with Progeria received Emmy Peabody and Christopher Awards, and ten Kudos Awards.

2014
April 2014
In a stunning development, a study shows that the drugs tested in the Progeria clinical trial increases estimated lifespan of children with Progeria. Lonafarnib appears to be giving them improved cardiovascular and longer lives. Remarkable!

2015
July 2015
PRF launches its Discovery Series to continue for more effective drug candidates, the PRF-funded expansion of the clinical trial gives every child with Progeria access to lonafarnib the drug that could give them longer and healthier lives. 78 children have enrolled to date, including 21 new children from 11 countries speaking 21 languages.

2016
April 2016
Children begin to enroll in a new 2-drug trial that include the treatment lonafarnib plus everolimus, a form of rapamycin, with the hope that the two drugs together will be even more effective than lonafarnib alone. Nine have enrolled through August 2016, and more are scheduled to arrive at Boston Children’s Hospital to participate.

NEW DRUG, AND NEW HOPE FOR PROGERIA.

lonafarnib alone. Nine have enrolled through August 2016, and more are scheduled to arrive at Boston Children’s Hospital to participate.

Lonafarnib expansion trial.

LONGER LIVES, STRONGER HEARTS, AND HOPE FOR THE FUTURE OF CHILDREN WITH PROGERIA.

lonafarnib expansion trial.

lonafarnib on Progeria. As new compounds are being developed, the PRF-funded expansion of the lonafarnib expansion trial, planning for a new trial and a new international scientific workshop, and two high $1 million investment in research grants.

Lonafarnib expansion trial.

Lonafarnib expansion trial.

Lonafarnib expansion trial.

Lonafarnib expansion trial.

Lonafarnib expansion trial.
PRF 2015 Annual Report

PRF's programs are the cornerstone of our successes that will culminate in new treatments and a cure for children with Progeria, as well as increase our understanding of heart disease and aging. 2015 saw expansion of all programs, with increased numbers of children identified, more researchers involved, and greater awareness worldwide. This includes a 28% increase in research grant funding – a testament to PRF's global awareness efforts and the ever-growing interest in Progeria research.

For more details on PRF’s programs and services, check out PRF By The Numbers at progeriaresearch.org/prf-by-the-numbers.

In the past 5 years, there has been an exponential increase in our research grant funding: 365%!

10 Core PRF Activities That Support Our Mission

<table>
<thead>
<tr>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
<th></th>
</tr>
</thead>
<tbody>
<tr>
<td>24 children from 14 countries</td>
<td>232 children from 54 countries and all continents</td>
<td>123 children diagnosed</td>
<td>580 cell lines sent to 98 teams in 18 countries</td>
<td>147 enrolled; 493 treatment guidelines sent to 42 countries</td>
<td>3 clinical trials funded since 2007</td>
</tr>
<tr>
<td>8 children diagnosed from Argentina, Brazil, India, Indonesia, Philippines &amp; US</td>
<td>8 children diagnosed</td>
<td>132 children diagnosed</td>
<td>15 children enrolled; 27 treatment guidelines sent to 11 countries; lonafarnib trial expansion continues - 13 children came to Boston for trial visit; new 2-drug trial approved</td>
<td>62 grants to 41 labs in 13 countries; funding total &gt;$6.7 million</td>
<td>10 meetings; 7 general workshops and 3 subspecialties</td>
</tr>
<tr>
<td>132 cell lines sent to 21 teams in 8 countries</td>
<td>132 cell lines sent to 21 teams in 8 countries</td>
<td>123 children diagnosed</td>
<td>580 cell lines sent to 98 teams in 18 countries</td>
<td>147 enrolled; 493 treatment guidelines sent to 42 countries</td>
<td>3 clinical trials funded since 2007</td>
</tr>
<tr>
<td>15 children enrolled; 27 treatment guidelines sent to 11 countries; lonafarnib trial expansion continues - 13 children came to Boston for trial visit; new 2-drug trial approved</td>
<td>15 children enrolled; 27 treatment guidelines sent to 11 countries; lonafarnib trial expansion continues - 13 children came to Boston for trial visit; new 2-drug trial approved</td>
<td>62 grants to 41 labs in 13 countries; funding total &gt;$6.7 million</td>
<td>10 meetings; 7 general workshops and 3 subspecialties</td>
<td>62 grants to 41 labs in 13 countries; funding total &gt;$6.7 million</td>
<td>10 meetings; 7 general workshops and 3 subspecialties</td>
</tr>
<tr>
<td>7 grants awarded</td>
<td>7 grants awarded</td>
<td>62 grants to 41 labs in 13 countries; funding total &gt;$6.7 million</td>
<td>10 meetings; 7 general workshops and 3 subspecialties</td>
<td>62 grants to 41 labs in 13 countries; funding total &gt;$6.7 million</td>
<td>10 meetings; 7 general workshops and 3 subspecialties</td>
</tr>
<tr>
<td>45 events &amp; over 220 new volunteers</td>
<td>45 events &amp; over 220 new volunteers</td>
<td>45 events &amp; over 220 new volunteers</td>
<td>45 events &amp; over 220 new volunteers</td>
<td>45 events &amp; over 220 new volunteers</td>
<td>45 events &amp; over 220 new volunteers</td>
</tr>
<tr>
<td>3 languages added: Serbian, Tamil &amp; Vietnamese</td>
<td>3 languages added: Serbian, Tamil &amp; Vietnamese</td>
<td>3 languages added: Serbian, Tamil &amp; Vietnamese</td>
<td>3 languages added: Serbian, Tamil &amp; Vietnamese</td>
<td>3 languages added: Serbian, Tamil &amp; Vietnamese</td>
<td>3 languages added: Serbian, Tamil &amp; Vietnamese</td>
</tr>
<tr>
<td>18 new children identified and over 300 million people reached through media</td>
<td>18 new children identified and over 300 million people reached through media</td>
<td>18 new children identified and over 300 million people reached through media</td>
<td>18 new children identified and over 300 million people reached through media</td>
<td>18 new children identified and over 300 million people reached through media</td>
<td>18 new children identified and over 300 million people reached through media</td>
</tr>
</tbody>
</table>

Annual PRF Research Grant Funding

- 2010: $224,916
- 2011: $329,851
- 2012: $426,494
- 2013: $506,879
- 2014: $854,452
- 2015: $1,050,876

2015 Highlights:

Annual funding for research grants reached a milestone, exceeding $1,000,000 for the first time. PRF awarded 7 new research grants in 2015, contributing to a total of 18 active projects and a funding rate of 35%. From inception through 2015, 174 grant applications were received and 62 were approved for funding. Researchers from 44 institutions in 13 countries are leading the charge for Progeria’s future scientific discovery, changing the landscape of Progeria research and giving the world ever-increasing hope for treatments and a cure.

Approval of our new two-drug clinical trial: PRF and the PRF-funded Boston Children’s Hospital clinical team obtained all necessary approvals for our third clinical trial. This trial will test whether lonafarnib in combination with a drug called everolimus can provide better treatment for Progeria than lonafarnib alone. Read more about this trial on page 4.

Lonafarnib-only trial: Children continued to enroll in the extension and expansion of the lonafarnib-only trial. In 2015, 15 children came to Boston Children’s Hospital from 13 countries speaking 12 languages – amazing! With the historic discovery that lonafarnib improves some disease features, including the cardiovascular system and longer estimated lifespan, PRF is expanding access to the only current treatment for Progeria. Concurrently, we are initiating our new two-drug trial with everolimus and continuing the search for even more effective treatments and ultimately, the cure.

PRF’s Medical Research Committee Holds 2-Day Summit in Boston: This brilliant group met for grant reviews, discussion of the nature and design of future workshops, and expert guest presentations by Dr. Gary Nabel, Chief Scientific Officer at Los Alamos, and Dr. Mark Kieran, Director of Pediatric Medical Neuro-Oncology at Dana-Farber Cancer Institute and Boston Children’s Hospital, who addressed strategies for genetic therapy, high-throughput progena mouse testing facilities, CRISPR technology, and future clinical drug trials. The meeting served to assure that PRF is on the cutting edge of science, with dynamic programs that propel us towards treatments and cure.

You make all of this progress possible – Thank You! With your steadfast support, we will work to ensure that 2016 and beyond brings us even closer to curing children with Progeria. Together, we will find the cure!
PRF Welcomes New Executive Director; Founding ED Takes on New Role at PRF
A new chapter in PRF leadership will begin this month, as Meryl N. Fink, Esq., becomes the Executive Director. Meryl brings to PRF 10+ years of experience in senior management of global law firms. For the past year, Meryl has served as PRF’s Director of Operations, providing financial oversight while also managing the field office and human resources. She will expand on these tasks in her new role, working with the Board of Directors, Board of Advisors, officers, staff, professional support, donors, volunteers, and the families we serve to ensure mission-driven success for PRF through program administration and development, internal management, and financial strength and growth. Meryl quickly became an integral part of PRF’s operations,” said PRF Board Chair, Dr. Scott Berns, “and the Board is confident she will be a great asset to the organization and the children in this new role.”

PRF’s Social Media Platforms Continue to Drive Awareness and Support
Our ever-growing social media activity keeps millions informed and engaged in PRF’s mission and accomplishments. Over one million Facebook followers from 45 countries share our news items, events, photos, and stories posted at facebook.com/ProgeriaResearch, and our YouTube page (Progeria) is 2nd most popular of all time, with over 17 million views! TEDx talks have been given in every corner of the globe, and in February, a new milestone was reached: one billion views of TEDx Talks — AMAZING! How fitting that Sam’s was featured in TEDx worldwide announcement in which they encourage everyone to “explore 15 amazing talks…which have sparked both global and local conversations and connections.” Sam’s dad, Scott Berns gave a moving tribute to his son and this milestone at TEDx Mid-Atlantic 2015. See Progeriaresearch.org/TEDx for more.

PRF Medical Director Presents at Esteemed Nobel Mini-Symposium
The Karolinska Institute in Stockholm, Sweden held a Nobel Mini-Symposium titled “Premature aging: From basic research to clinical treatment in a decade” in May. The world’s leaders in Progeria research, including 4 PRF research grantees and 4 members of PRF’s Medical Research Committee presented their Progeria work.

PRF ON THE MOVE!

Incoming Executive Director Meryl Fink (left) and former ED/new Director of Development Audrey Gordon.

Sam Berns’ Talk Featured in TEDx Billion Views Tribute
Out of over 40,000 TEDx talks, Sam’s “My Philosophy for a Happy Life” is the 2nd most popular of all time, with over 17 million views! TEDx talks have been given in every corner of the globe, and in February, a new milestone was reached: one billion views of TEDx Talks — AMAZING! How fitting that Sam’s was featured in TEDx worldwide announcement in which they encourage everyone to “explore 15 amazing talks…which have sparked both global and local conversations and connections.” Sam’s dad, Scott Berns gave a moving tribute to his son and this milestone at TEDx Mid-Atlantic 2015. See Progeriaresearch.org/TEDx for more.

Incoming Executive Director Meryl Fink (left) and former ED/new Director of Development Audrey Gordon.

PRF Medical Director Presents at Esteemed Nobel Mini-Symposium
The Karolinska Institute in Stockholm, Sweden held a Nobel Mini-Symposium titled “Premature aging: From basic research to clinical treatment in a decade” in May. The world’s leaders in Progeria research, including 4 PRF research grantees and 4 members of PRF’s Medical Research Committee presented their Progeria work.

We continue to be inspired by those who learn about PRF’s mission to cure children with Progeria and take action to help in a variety of ways. We wish we could feature all of them — go to progeriaresearch.org/miracle_makers to read more stories and learn how you can join this phenomenal group of supporters!

Fallon, Abigail and Girls on the Run of Enderingham, PA, hold a very successful Bake Sale - a tasty way to raise over $450 for PRF. What an amazing group of generous and enthusiastic girls!

All in the Family...Jula Fleming has been a Miracle Maker for years, and now her mom Tricia has joined in. Her PA yoga studio, Twister’s Wellness Centers, holds “donation only” classes, with all proceeds going to PRF. Tricia says, “I could tell from the moment I met Sam that his spirit and affectionate nature was the reason for his own generosity. As you get to know him, you can’t help but think of your own generosity and how you can pass that on. Thank you, Michelle, for sharing your story.

Thank you, Michelle, for sharing your story.
Your kind words and actions inspire all of us at PRF!

Join the Leadership by Legacy Society with a planned gift to PRF
Reduce estate taxes and leave a lasting legacy that meets your philanthropic and financial goals:
• Make a bequest in your will of cash, securities, or real estate to PRF;
• Name PRF as a beneficiary of your life insurance policy or retirement plan;
• Have an immediate impact today by donating shares of stock (this avoids capital gains tax on the gain).

“What kind of legacy do we want to leave? One that guarantees that all children with Progeria have every resource necessary. And if Progeria research improves the lives of millions with heart disease, what a gift to us all.”
- Leadership by Legacy members John and Marlene Marozzi

To learn more or for suggested will language, please contact plannedgiving@progeriaresearch.org or visit progeriaresearch.org/planned_giving

Join PRF’s monthly giving program and be part of our Champions for the Cure
For as little as $10 a month, you can help provide a steady flow of support that can rely on for our work to bring treatments and the cure to children with Progeria. It’s easy and fast – just click on the “Donate Now” button on our website homepage and choose “Recurring Donation”.

There are many ways to help children with Progeria – go to progeriaresearch.org/get_involved to see them all!
Chapter, Family & Other Annual Events

We are so grateful to all who organize, attend and support these fun community events that help raise awareness and funds needed to support our mission. THANK YOU ALL!

The sun shined brightly last September at our 14th Annual International Race for Research. Signs with inspiring messages from Sam Berns’ TEDx talk lined the race route in Peabody, MA, adding a special feel to the day. Congratulations to Meghan Waldron, who finished 1st in her age group!

Meghan had a great run.

Make a Splash 2015: fun for the whole family!

Swimming, dancing, silent auction, balloon artist and ring toss made the 5th annual Make A Splash party at PA’s Flourtown Country Club loads of fun for everyone!

Nathan, Bennett and kids of all ages join in the games.

I 11th Annual Miles for Miracles – Michigan’s Event of the Year!

Every May, hundreds of fans of Lindsay Ratcliffe come together to support the Michigan Chapter’s signature event. Lindsay designed another awesome t-shirt, the weather was perfect, and the food, auction and games tents were full of happy supporters!

A sea of blue covers Fountain Park as the crowd starts the walk.

14th Annual Make a Splash party. A sea of blue covers Fountain Park as the crowd starts the walk.

Team Enzo Sausage Sales a Sizzling Success!

We love inaugural events – they not only add new community energy and supporters, but also turn into annual ones! “Our First Australian Sausage Sizzle was an absolute success,” says Catherine, Enzo’s mother. “Everyone is already looking forward to next year’s.” Thank you to our friends down under!

Everyone helps out at the Sausage Sizzle.

Enzo dons his hat in Australia.

Hats ON for Progeria April 15, 2016: a global celebration of solidarity for children with Progeria

Our 8th Night of Wonder was an unforgettable evening that raised a record-breaking $570,000! The sports-related theme “SCORE for the CURE” was a huge hit, as guests celebrated PRFs championship successes that will lead to a cure for Progeria.

There were many special highlights, from the thematic, never-before-seen outtake of Sam umpiring in Life According to Sam, to the heartfelt acceptance speeches of Amy Award winner Bob Morrison and S.A.M. (Science and Medicine) Award recipient Dr. Monica Kleinman. Thanks to the volunteers, auction donors, sponsors, and guests for being such hardcore fans for children with Progeria.

Our major sponsors helped make the evening a phenomenal success – Thank you all!

The Morrison Family
Leslie Gordon & Scott Berns
Debbie Mendelson Ponn

I 8th Annual Cam’s Course flies like an Eagle!

Organizer Brooke Howard, AKA “Auntie Brooke” was overwhelmed by the success of this year’s August tournament in Butler, PA. “I can’t wait to see what we can accomplish together next year!” A special thank you to Brooke and her husband Jeff for all their hard work, and to Title Sponsor and Cam’s good friend Bob McCarthy for his steadfast and generous support.

(left to right) Jeff, Bob, and Brooke are all smiles after a record-breaking event.

8th Annual Cam’s Course flies like an Eagle!

3rd Time’s a Charm for Ohio’s Freeze Daddy’s

This is a delicious community event.

Glass City Corvettes and Oak Park Gang Car Club display cars, Kaylee serves ice cream with a smile, and Carly has fun with the crowds. Thanks to owner Ron Loefller – Kaylee and Carly appreciate your support!

Kaylee and brother TJ work the counter.

NY’s Mountain Lakes High Fashion Show Shines for Team Zoey

Zoey and students from the Mountain Lakes school system strut their stuff during the 2016 Team Zoey Fashion Show. SO BEAUTIFUL!

3rd Time’s a Charm for Ohio’s Freeze Daddy’s

A sea of blue covers Fountain Park as the crowd starts the walk.

2016 Team Zoey Fashion Show. SO BEAUTIFUL!

泓

Night of Wonder 2016: the biggest SCORE yet!

Our 8th Night of Wonder was an unforgettable evening that raised a record-breaking $570,000! The sports-related theme “SCORE for the CURE” was a huge hit, as guests celebrated PRFs championship successes that will lead to a cure for Progeria.

There were many special highlights, from the thematic, never-before-seen outtake of Sam umpiring in Life According to Sam, to the heartfelt acceptance speeches of Amy Award winner Bob Morrison and S.A.M. (Science and Medicine) Award recipient Dr. Monica Kleinman. Thanks to the volunteers, auction donors, sponsors, and guests for being such hardcore fans for children with Progeria.

Our major sponsors helped make the evening a phenomenal success – Thank you all!

The Morrison Family
Leslie Gordon & Scott Berns
Debbie Mendelson Ponn
Introducing Our New Youth Ambassador, Meghan Waldron!

Accomplished cellist and violinist, high school cross country and track team athlete, published poet – Meghan Waldron is a talented and busy 15-year-old who will now add PRF Youth Ambassador to her impressive list of activities. Meghan will serve as spokesperson for The Progeria Research Foundation, from the perspective of a youth with Progeria.

We look forward to Meghan’s participation in engaging youth around such events as Hats ON for Progeria, sharing her ideas on how to impact people through social media, and participating in PRF media outreach.

“I’m honored and excited to help raise awareness of Progeria and PRF among my peers and others around the world”, she says. Welcome, Meghan, we are too!