Progeria Research Foundation

WWW.PROGERIARESEARCH.ORG • SEPTEMBER 2015

news

Our Third Clinical Treatment Trial Begins!

Supporting study also validates Progeria-aging link

6-year-old Zoey has been enrolled in the lonafarnib trial since July 2013. She and other children may participate in the new trial as it gets underway at Boston Children's Hospital.

In keeping with our intensely focused mission to discover treatments and the cure for Progeria and its aging-related disorders, PRF is initiating another drug trial to treat children from around the world. Everolimus will be added to the current lonafarnib treatment regimen, with the hope that the two drugs together will be even more effective than lonafarnib alone.

Read more about this exciting development on page 4.

CHARITY NAVIGATOR ★★★★ Four Star Charity

We're a 4-Star Charity!

2 Years In a Row!

Mio, 5 years old

PRF's Mission:

To discover treatments and the cure for Progeria and its aging-related disorders, including heart disease.

Progeria is a fatal, "rapid aging" disease that afflicts children, who die of heart disease at an average age of 14 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,

So much activity, so much progress in research, awareness and events! Enjoy the pages that follow, which clearly demonstrate we are making tremendous progress toward a cure – thanks to all of you.

Over half the world's identified population of children with Progeria has traveled to Boston to enroll in the clinical trial involving the effective lonafarnib drug.

In our ongoing effort to discover drugs that may be even more effective than lonafarnib alone, a new clinical trial is starting that will add the drug everolimus.

Our research programs are thriving and growing exponentially, a reflection of the ever-increasing interest and progress in the field. Examples are PRF's $1 million investment in research grants this year, and the nearly doubling of cell lines sent to researchers so they can explore Progeria in the lab.

Our upcoming 8th international workshop in May 2016 will bring together leaders in the field to share and collaborate on the astounding depth and breadth of new research knowledge and advances.

Global awareness of Progeria and PRF's work is being realized in a variety of ways, including our robust social media presence, the Find the Other 150 Campaign, HBO's *Life According to Sam*, and Sam Berns' wildly popular and inspiring TEDx talk.

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 continue to support PRF – 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 16 years. It is only through your continued support that we will treat children with Progeria today and cure them in the future.

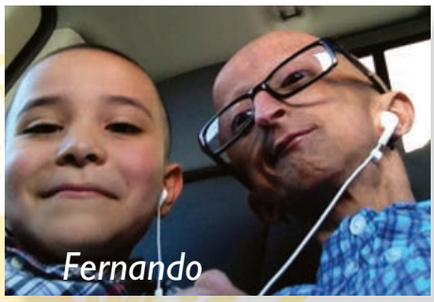
Many thanks to all of you who care so very much.

Together, we *WILL* find the cure!

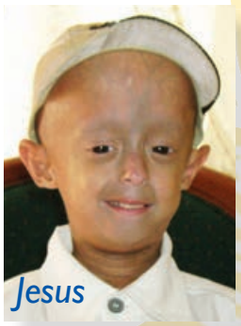
Audrey Gordon, Esq.
President and Executive Director

In Loving Memory..!

17 years old,
from England

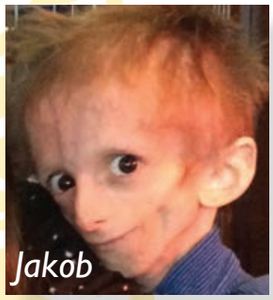


16 years old, from the United States
(pictured here taking a selfie with
brother Emilio)



16 years old,
from Venezuela

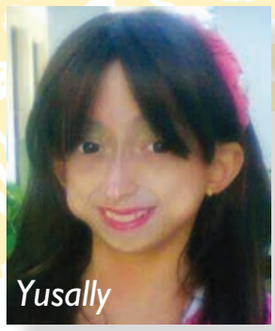
Gone from our sight,
but never our memories ~
Gone from our touch,
but never our hearts.



8 years old,
from Switzerland

3 years old,
from Germany

14 years old,
from Pakistan

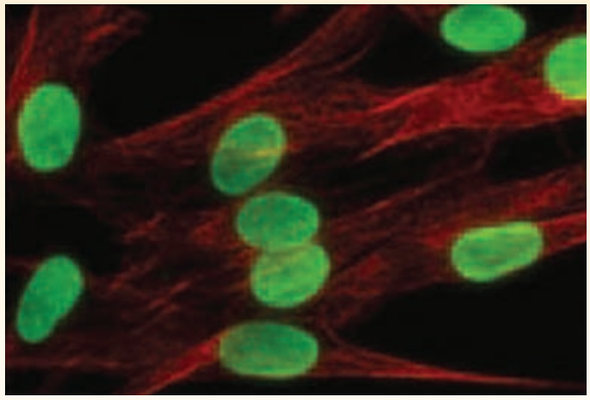


14 years old,
from Dominican Republic

Clinical Trial Update

The Progeria 2-Drug, Phase 1 Clinical Treatment Trial Begins This Fall

PRF is thrilled to announce that we will fund and co-coordinate a third clinical trial, which will assess a two-drug combination of lonafarnib plus **everolimus**. Everolimus is a form of the drug **rapamycin**, but everolimus can be more easily given to children with Progeria because it requires fewer blood draws to measure drug levels. While lonafarnib may block progerin from developing, rapamycin appears to allow cells to more rapidly clear out the toxic progerin. Thus with rapamycin targeting a different pathway than lonafarnib, the combination may prove to be a "one-two punch" to Progeria – hopefully a better treatment than lonafarnib on its own.



Progeria cells treated with rapamycin become normalized.

Rapamycin is known for its anti-aging properties in mice. These findings are part of a growing list of studies that validate the theory that finding the cure for Progeria may also benefit the entire aging population.

Studies by 4 different laboratories have tested rapamycin and/or everolimus in the laboratory on Progeria cells, and all showed benefit:

- Cenni V, et al. Autophagic degradation of farnesylated prelamin A as a therapeutic approach to lamin-linked progeria. *Eur j histochem* 2011
- Cao K, et al. Rapamycin reverses cellular phenotypes and enhances mutant protein clearance in Hutchinson-Gilford progeria syndrome cells. *Sci trans med* 2011
- Graziotto JJ, Cao K, Collins FS, Krainc D. Rapamycin activates autophagy in Hutchinson-Gilford progeria syndrome: implications for normal aging and age-dependent neurodegenerative disorders. *Autophagy* 2012
- Blondel S, et al. Induced pluripotent stem cells reveal functional differences between drugs currently investigated in patients with Hutchinson-Gilford progeria syndrome. *Stem cells trans med* 2014.
- Pellegrini C, et al. All-trans retinoic acid and rapamycin normalize Hutchinson-Gilford progeria fibroblast phenotype. *Oncotarget* 2015.

PRF provided cells and/or funding for some of these projects – more proof that PRF's research-related programs are essential to advancements toward the cure.

This new trial is a collaborative effort that will build upon the knowledge gained from the previous two Progeria trials. The children will be seen by virtually the same team of physicians from Boston Children's Hospital, Dana-Farber Cancer Institute and Brigham and Women's Hospital, all of whom now have world-renowned expertise in Progeria as well as the drugs involved.

What is the difference between Phase 1 and Phase 2 trial segments?

The Boston trial team will begin with Phase 1, where the study team will determine the safest maximum dose of everolimus for children with Progeria. The team begins by giving three children a very low dose of the medication, and carefully observes for side effects. If toxicity is minimal, another three children are enrolled at a higher dose of medicine. This pattern recurs until the safest maximum dose of everolimus is determined and the next study phase can begin. During Phase 2, the study will determine if the effects of the two-drug combination on disease are better than lonafarnib alone. Together, this Phase 1-2 treatment trial may enroll up to 80 children, and take an estimated 3.5-4 years to complete, at a cost of approximately $2.5 million dollars. In addition, Novartis has generously agreed to supply everolimus at no cost, and Merck & Co. has agreed to manufacture and supply the lonafarnib at no cost. ***A big thank you to Novartis and Merck!***

As we begin this new chapter in our trial history, please continue to support PRF – so we can continue to help children with Progeria live longer and healthier lives.

Lonafarnib Trial Extension/Expansion in Full Force!

In our last issue, we reported 37 children had enrolled in the PRF-funded, lonafarnib-only trial. Today, that number has nearly doubled! 70 children from 30 countries and speaking 21 languages have enrolled to date. 25 are first-timers that have not been involved in previous PRF clinical trials – SO EXCITING! That means that 25 children with Progeria are now receiving the cardiovascular benefits of lonafarnib,* while others can continue taking this treatment thanks to PRF and its fantastic partners at Boston Children's Hospital (BCH), Dana-Farber Cancer Institute, and Brigham & Women's Hospital.

*In 2012, the historic finding was made that lonafarnib significantly improved rate of weight gain, bone structure and, most importantly, vascular stiffness for children with Progeria. In 2014, further studies revealed lonafarnib increased estimated lifespan. Given these remarkable findings, the trial team and PRF decided to extend the trial so longer-term effects can be studied and more children with Progeria can have access to this treatment, while we continue the search for more effective treatments and, ultimately, the cure.

PRF's Ambassador Program: An essential part of our trial activities.

As PRF's clinical trial enrollment has grown, so has our need for Ambassadors to assist families when traveling to Boston for their trial visits. Many thanks to ambassadors at *No Thanks Needed*, another Massachusetts-based nonprofit that worked with PRF for two years, and our long-time ambassadors who have been with us since the start of the trials in 2007. Not only have the number of volunteers grown, but we now have bi-lingual ambassadors for multiple languages – AMAZING!

Ambassadors pick up the families when they arrive, take them to BCH's housing at the Devon Nicole House, help them get settled in for the long week of testing, and bring them back to the airport at the end of their visit.

"I am so thankful for this opportunity. I am honored to do my small part for Julia and the other children." – Lola Busta from Peabody, who has been a PRF Ambassador for children including Julia, 15 years old and from Poland, since 2008.

PRF's Ambassador Program is robust, successful and growing, thanks to our amazing volunteers. A world of thanks to them ALL!

Come join our PRF Ambassador team! Contact us at *info@progeriaresearch.org* for more details.

Left to right: Amen and his mother Adoh-Ballo of Togo, PRF Ambassador/Translator Carl Pierre, Omaima from Pakistan, Tom Quigley from No Thanks Needed, and Omaima's parents Yasmeen and Abdul. Both families traveled to Boston in July to enroll in the lonafarnib expansion trial.

Many thanks to everyone who supported our 2015 ONEpossible campaign. We reached our goal of $200,000 – enough to fund 5 children in this 2.8 million dollar clinical trial.

Enzo, 3 years old from Australia and one of the 25 new children to enroll in the lonafarnib trial, says THANK YOU for being ONE to make a cure for Progeria POSSIBLE!

Lola (right), with Julia and mom at the Devon Nicole House.

PRF Awards Nine More Research Grants

Will invest an all-time high of $1 million this year!

Over the past 16 years, PRF has provided over $6.7 million to fund 60 grants for Progeria-related research projects performed in 18 states and 13 countries. The projects have led to important discoveries about Progeria, heart disease, and aging. PRF's commitment to research funding allows innovative projects in Progeria to thrive, and we are thrilled that the number and quality of proposals continue to grow.

SPECIALTY AWARDS:

Francis Collins, MD, PhD
Director, The National Institutes of Health (NIH/NHGRI), Bethesda, MD.

"Post-doctoral Candidate Funding for HGPS Research"

Dr. Collins and his team, together with PRF, co-discovered the genetic cause of HGPS in 2003, and with over a dozen years invested in this work, their aim remains: to understand pathogenesis and seek treatments for Progeria. PRF will fund a full time, highly qualified researcher within Dr. Collins' laboratory, to focus on potential therapeutic approaches for children with Progeria, including RNA-based methods and the use of rapamycin and its analogs, using both cellular and HGPS mouse models.

Jesús Vázquez Cobos, PhD
Professor, Centro Nacional de Investigaciones Cardiovasculares (CNIC), Profesor de Investigación of the CSIC and Director of the Proteomics Platform of the RIC (Spanish Cardiovascular Research Network), Madrid, Spain.

"Quantitation of Farnesylated Progerin in Progeroid Mouse Tissues and Circulating Leukocytes from Hutchinson-Gilford Progeria Patients"

Dr. Cobos and his researchers' focus is improving the technique to quantify farnesylated progerin directly from blood samples from HGPS patients using mass spectrometry. If successful, the technique would provide scientists with an invaluable tool to evaluate the efficacy of experimental treatments in humans and to monitor the progression and severity of this disease.

ESTABLISHED INVESTIGATOR AWARDS:

Vicente Andrés García, PhD
Professor, Laboratory of Molecular and Genetic Cardiovascular Pathophysiology, Vascular Pathophysiology Area, and Dir. of Dept. of Basic Research, Centro Nacional de Investigaciones Cardiovasculares Carlos III, Madrid, Spain.

"Generation of a HGPS Knock-in Pig Model to Expedite the Development of Effective Clinical Applications"

Mouse models have been instrumental in basic and translational Progeria research, but mice do not faithfully recapitulate all aspects of human pathology. Compared with rodents, pigs more closely resemble humans in body and organ size, anatomy, longevity, genetics and physiology. Consequently, pigs are now becoming one of the most important large animal models for preclinical biomedical research. Dr. Garcia's laboratory will generate a pig model of Progeria, and become a core facility for preclinical studies and Progeria treatment testing in pigs.

Bum-Joon Park, PhD
Chairperson and Professor, Department of Molecular Biology, Pusan National University, Republic of Korea.

"Improvement of therapeutic effect of JH4, progerin-lamin A/C binding inhibitor, against progeria syndrome"

Through chemical library screening, Dr. Park's research team recently found a novel chemical named JH4 that blocks the interaction between progerin and Lamin A/C. In a progerin-producing mouse model, JH4 extends life span and ameliorates aging phenotypes. He now aims to investigate strategies for treating disease by using JH4 in Progeria mouse models, leading to potential treatment for children with Progeria.

Welcome, New Medical Research Committee Members!

- **Maria Eriksson, PhD,** Sr. Researcher, Dept. of Biosciences and Nutrition, Karolinska Institute, Stockholm, Sweden
- **Vicente Andrés García, PhD,** from CNIC Madrid, Spain (see photo and bio, above)
- **Marsha Moses, PhD,** from Harvard Medical School and Boston Children's Hospital, United States (see photo and bio, p. 7)

Heartfelt thanks to **Frank Rothman, PhD,** Professor of Biology and Provost Emeritus, Brown Univ., Providence, RI, who served on the MRC for 16 years.

*The MRC is a volunteer committee that reviews all research grant submissions.

Maria Eriksson

Frank Rothman

INNOVATOR AWARDS:

Cláudia Cavadas, PhD

Leader, Neuroendocrinology and Aging Group, Center for Neuroscience and Cell Biology (CNC), University of Coimbra, Portugal.

"Peripheral NPY reverts HGPS phenotype: a study in human fibroblasts and mouse model"

The discovery of new therapeutic compounds is of utmost importance for those with HGPS. The endogenous molecule neuropeptide Y (NPY) activates NPY receptors that are localized in various organs and cells affected by HGPS. Dr. Cavadas leads a team investigating the NPY system as an innovative strategy for the therapeutics, or co-therapeutics, for Progeria.

We are thrilled to announce a first-ever research grant funding partnership with ***Carly Cares, Inc.*** *for Dr. Cavadas' project.* ***www.carlycares.org***

John P. Cooke, MD, PhD

Professor and Chair, Department of Cardiovascular Sciences, Houston Methodist Research Institute, Director of Center for Cardiovascular Regeneration, Houston Methodist DeBakey Heart and Vascular Center, Houston, TX.

"Telomerase Therapy for Progeria"

In children with progeria, the blood vessels age very quickly. This causes vascular disease that leads to heart attack and stroke. Dr. Cooke's team aims to develop a therapy that will extend telomeres (which hold the chromosome together to ensure its normal function), reverse the cellular aging process, and rejuvenate the vascular cells.

Dudley Lamming, PhD

Assistant Professor, Department of Medicine at the University of Wisconsin-Madison, Co-Director of the UW Department of Medicine Mouse Metabolic Phenotyping Platform, Madison, WI.

"Intervention in Progeria by Restriction of Specific Dietary Amino Acids"

Dr. Lamming's laboratory is focused on learning how nutrient-responsive signaling pathways can be harnessed to promote health and delay both normal aging and premature aging in Progeria. His team has determined that a low protein diet significantly reduces mTORC1, but not mTORC2, signaling in mouse tissues. His team will identify a diet that inhibits mTORC1 signaling in vivo, and determine the ability of this diet to rescue HGPS pathology. Since rapamycin and its analog, everolimus, work through these pathways, these studies may add to our understanding of the upcoming clinical treatment trial (see clinical trial announcement on page 4).

Célia Alexandra Ferreira de Oliveira Aveleira, PhD

Researcher, Biomedical Sciences, Center for Neuroscience and Cell Biology (CNC) and Institute for Interdisciplinary Research (IIIUC), University of Coimbra, Portugal.

"Ghrelin: a novel therapeutic intervention to rescue the phenotype of Hutchinson-Gilford Progeria Syndrome"

Ghrelin is a circulating peptide hormone that is the endogenous ligand for the growth hormone secretagogue receptor, with growth hormone-releasing activity. Ghrelin has beneficial roles in different organs and systems, such as cardiovascular protective effect, atherosclerosis regulation, protection from ischemia/reperfusion injury as well as improving the prognosis of myocardial infarction and heart failure. Dr. Aveleira will investigate the potential of ghrelin and ghrelin receptor agonist as treatment for Progeria.

Marsha Moses, PhD

Julia Dyckman Andrus Professor, Harvard Medical School, Director of the Vascular Biology Program at Boston Children's Hospital, Boston, MA.

"Discovering Novel Non-Invasive Biomarkers for Hutchinson-Gilford Progeria Syndrome"

Dr. Moses and her team aim to utilize a state-of-the-art proteomics discovery approach, including mass spectrometry, to discover biomarkers of HGPS and potentially of aging and generalized cardiovascular disease. The insight gained in these studies of Progeria will inform and significantly expand our knowledge of the mechanisms underlying disease, and may ultimately represent therapeutic targets for Progeria, cardiovascular disease and other aging-related disorders.

Joseph Rabinowitz, PhD

Assistant Professor, Pharmacology Center for Translational Medicine, Temple University School of Medicine, Philadelphia, PA.

"Adeno-associated virus mediated co-delivery of wild type lamin A and microRNA against progerin"

As Director of the viral vector core at Temple University School of Medicine, Dr. Rabinowitz's researchers use adeno-associated viruses (AAV) as tools to deliver therapeutic genes to experimental animals, which can be used in clinical trials for humans. MicroRNAs (miRs) are small pieces of RNA that reduce protein expression by interfering with the corresponding messenger RNA of that protein. Research has demonstrated that Lamin A (LMNA) is not expressed at high levels in the brain, and miR-9 expression in the brain is responsible for that suppression. Dr. Rabinowitz' team will deliver miR-9 using an AAV genome, with the aim of rescuing the Progeria phenotype in cells and Progeria mice.

For more details on the application process and the grants PRF has funded, please visit *http://www.progeriaresearch.org/research_funding_opportunities/*

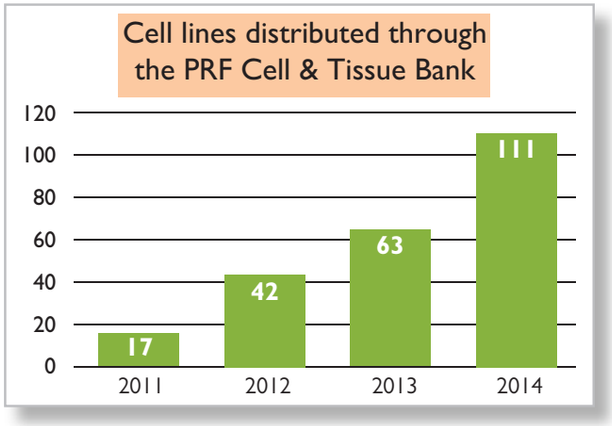
PRF 2014 Annual Report

More growth, more progress, more

PRF's programs are thriving and expand annually with increasing numbers of children identified, researchers involved, and awareness raised. All of PRF's activities work cohesively to provide the core structure essential not only to drive Progeria awareness and research forward, but also to discover what Progeria can tell us about heart disease and aging.

From 2013 to 2014, our program growth continued at a remarkable pace. This includes a 12% increase in the number of known living children and a 76% increase in cell lines distributed to researchers – 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*** and an article published in *Expert Opinion* authored by Executive Director Audrey Gordon and Medical Director Leslie Gordon, wherein the two PRF leaders discuss PRF's history, goals and accomplishments, and how PRF's programs have been pivotal in the journey from obscurity to treatment. ***progeriaresearch.org/whats_new_in_progeria_research***



In recent years, there has been an exponential increase in the number of cell lines distributed to research teams: 553% from 2011 through 2014!

10 Core PRF Activities That Support Our Mission

2014 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY Through 12/31/14
15 children from 12 countries	International Patient Registry	208 children from 51 countries and all continents
4 children diagnosed from Brazil, Honduras, India & South Korea	Diagnostic Testing Program	115 children diagnosed
111 cell lines sent to 27 teams in 12 countries	Cell & Tissue Bank	448 cell lines sent to 80 teams in 17 countries
6 children enrolled; 20 treatment guidelines sent to 8 countries	Medical & Research Database	132 enrolled; 466 treatment guidelines sent to 39 countries
Funded Triple Treatment Trial Expansion (Enrollment ongoing)	Clinical Trial Funding & Co-coordination	3 clinical trials funded since 2007
5 grants awarded	Basic Research Grant Funding	55 grants to 41 labs in 12 countries; funding total >$6.1 million
Initial planning for 2015 & 2016 meetings begins	International Scientific Meetings	11 meetings: 7 general workshops and 4 subspecialties
50 events & over 250 new volunteers	Volunteer Program	Nearly 4,250 volunteers worldwide have helped raise awareness and funds
2 languages added: Indonesian & Swedish	Translations Program	28 languages
15 new children identified and 309 million people reached through media	Public Awareness & Find The Other 150 Campaign	125 living children from 39 countries; over 1.96 billion* people exposed to Progeria and PRF's work

* figure based on media impressions

planned to get us to the cure!

2014 Highlights:

Longer lives for children with Progeria: PRF, Boston Children's Hospital and Brown University teamed up on a major study of lifespan in Progeria. The May 2014 study, published in the prestigious American Heart Association journal *Circulation*, estimated that lonafarnib – the drug tested in the PRF-funded Progeria clinical trials – increased lifespan by at least 1.6 years, over the 6-year testing period. Time will tell if the life extension is longer than 1.6 years; The longest time any of the children were on drug was 6 years, so the researchers will re-evaluate after children are on the drug for a longer period of time. This is the first evidence of treatment influencing survival for this fatal disease – an historic finding in our quest to improve health and extend the lives of children with Progeria.

*Gordon, LB et al., Impact of farnesylation inhibitors on survival in Hutchinson-Gilford progeria syndrome, ***Circulation***, 2014, 130 (27-34)

The extension and expansion of the lonafarnib-only trial continued in 2014, with record numbers of children arriving at Boston Children's Hospital throughout the year: 46 patients from 23 countries speaking 16 languages! As of 12/31/14, a total of 63 children had participated in the trial, 18 of whom are new and taking lonafarnib for the first time. With the historic discovery that lonafarnib improves some disease features, including the cardiovascular system and longer estimated lifespan, PRF is making it possible for children to have access to lonafarnib while we continue to search for even more effective treatments and ultimately the cure.

"Flying out to Boston from Mumbai! Can't thank enough my dearest friends, beloved family, and all from PRF for making this possible!"

– December 2014 Facebook post from Nihal's father, on route to his son's first trial visit. All trial expenses are covered by PRF, including travel, lodging, food, interpreters and testing.

Night of Wonder 2014 sets a new bar With a record, sold-out crowd of nearly 500 people, **Night of Wonder 2014 – Lights, Camera, CURE!** was a special evening as we celebrated the success of HBO's documentary *Life According to Sam*, and the life of Sam Berns, the remarkable boy who captured the hearts of people around the world with his extraordinary personality and inspirational view of life. The event raised $500,000, which is being used to fund the clinical trial expansion and PRF's other research-related programs.

Many thanks to all of you for making such tremendous advancements possible. With your steadfast support, we will work to ensure that 2015 and beyond brings us ever-closer to curing children with Progeria.

Together, we *WILL* find the cure!

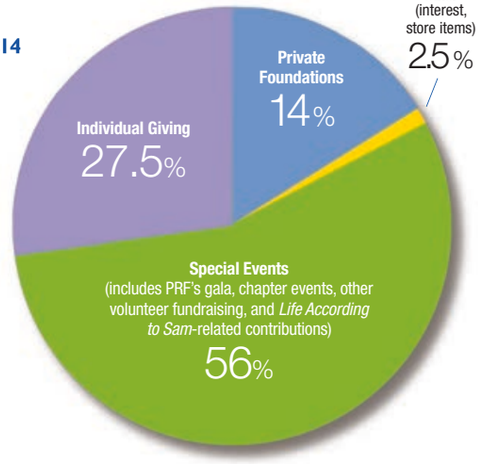
CHARITY NAVIGATOR ★★★★ Four Star Charity

We're a 4-Star Charity!

(Left to right) Megan and Meghan, both 14 years old, were the first to enroll in the lonafarnib trial in June 2007. Their most recent trial visit was November 2014.

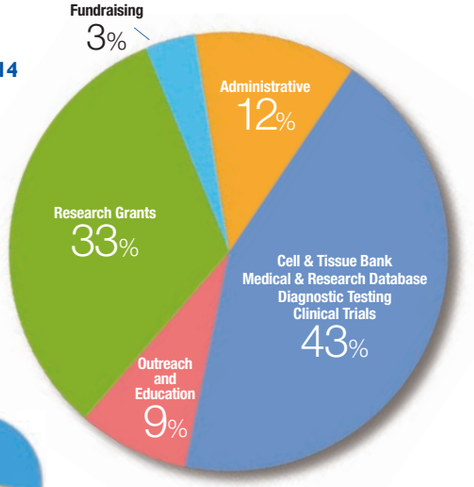
INCOME ANALYSIS 2014

TOTAL REVENUE
$3,004,264

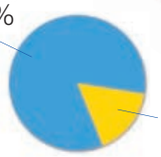


EXPENSE ANALYSIS 2014

TOTAL EXPENSES
$2,553,741



EXPENSE SUMMARY



12/31/14 Net Assets: 4,482,530*
*Much of these funds are designated for upcoming clinical trials and drug development costs

PRF's Support for Researchers Continues to Grow...

Researchers, Register Now!

ACROSS THE TABLE AROUND THE GLOBE

THE PROGERIA RESEARCH FOUNDATION
8TH INTERNATIONAL SCIENTIFIC WORKSHOP
MAY 2-4, 2016 • ROYAL SONESTA HOTEL • CAMBRIDGE, MA • USA

Register at www.prfworkshop.org
Questions? Contact us at workshop@progeriaresearch.org or Tel. 978-535-2594

We look forward to your participation. Together, we *WILL* find the cure!

Lonafarnib (FTI) Available for Preclinical Studies

PRF is excited to announce that the farnesyltransferase inhibitor lonafarnib is now available to the research community. Our goal is to support preclinical studies that investigate the effects of lonafarnib on Progeria.

It is PRF's steadfast mission to find additional treatments and a cure for children with Progeria. Lonafarnib has been shown to benefit some aspects of the disease clinically, as well as increase estimated lifespan, but it is not a cure. As new compounds are identified with the potential to improve Progeria, we anticipate the need to test these new compounds in combination with lonafarnib in vitro and in animal models.

We encourage researchers to be part of this endeavor and to consider similar preclinical studies. Visit ***www.progeriaresearch.org/lonafarnib-pre-clinical-program*** for ordering and other details.

(Left to right) Livia, 2 years old and Rafaely, 10 years old, traveled from Brazil to Boston in August for their lonafarnib trial visit. Livia is new to the trial, and Rafaely has been taking lonafarnib since 2009.

PRF Seeks Proposals for Next Funding Round

In our continuing effort to encourage research on Progeria and its aging-related disorders, we solicit proposals worldwide. All projects are carefully evaluated by our Medical Research Committee and Board of Directors as we strive to fund research targeted to developing treatments and the cure for Progeria.

Awards are given in 3 categories with varying funding levels and lengths of time. Projects must have specific relevance to Hutciinson-Gilford Progeria Syndrome and show promise for contributing to the scientific or clinical advancement in this field of study.

The next deadline is in March 2016, for consideration at the Board of Directors' June 2016 meeting. For more information visit ***www.progeriaresearch.org/research-funding-opportunities***

PRF *ON THE MOVE!*

Zach, 8 years old

PRF's Facebook page reaches One Million Followers!

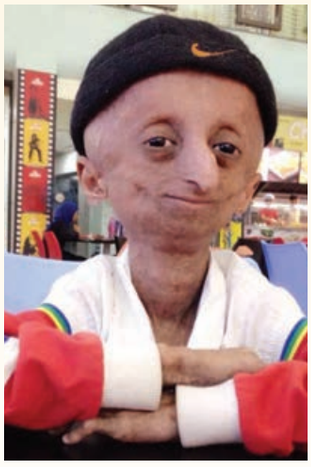
In March, The PRF Facebook page hit 1,000,000 followers, or "Likes". We are thrilled that our messages are being heard by so many around the world. From the US and Brazil to India, the UK and dozens of other countries, more and more people are learning about children participating in the clinical trials, hearing of research advancements, and enjoying stories about our remarkable volunteers.

Have you liked us yet?! Help us get to TWO Million – join us today! ***www.facebook.com/ProgeriaResearch***

And don't forget we are also on Twitter (@progeria), Instagram (progeriaresearch), YouTube (progeria123) and Pinterest (ProgeriaResrch)

Find The Other 150: Focus on China and India

The goal of PRF's global campaign, **Find the Other 150**, is to find, connect with and help the unidentified children living with Progeria so that they can get the unique help they need, including participation in the PRF-funded Progeria clinical drug trials. There are approximately 350 children with Progeria globally, and we currently know of 125. Of the 225 unknown children, an estimated 60 live in India and 76 live in China – that's 2/3 of the children we are searching for!
Go to ***www.findtheother150.org*** to find out how you can help.



Nihal, 17 years old from India, is helping to raise awareness in his country. Learn more at #Finding60inIndia and www.finding60inIndia.in

July 2015: PRF Teams up with *Time Machine* App Creators

Inspired by HBO's "Life According to Sam" (LATS), a group of social media entrepreneurs created an app that motivates people to spend time doing fun, meaningful activities related to PRF and other charities. To support PRF, users were asked to watch LATS and meet the kids from LATS at ***www.progeriaresearch.org/meet_the_kids***. Over 150 million people were reached, raising awareness and support for PRF while inspiring people to spend their time in meaningful ways. Visit **#CureProgeria** and **#itstime**, and download the free app on iOS to learn more.

PRF Welcomes New Staff

As our programs expand, and we continue to be the only organization in the world solely dedicated to finding treatments and the cure for Progeria, we must keep up with this tremendous growth as an organization. To do so, PRF staff now includes a Director of Operations and a Director of Development. We are thrilled that these accomplished individuals have joined our team.

Meryl N. Fink, Esq., Director of Operations

Meryl oversees the day to day management, provides financial oversight and manages human resources for PRF.

Meryl earned a B.S. in Industrial and Labor Relations from Cornell University and a J.D. from the University of Pennsylvania School of Law. She brings to PRF extensive experience as a corporate and securities attorney and 10-plus years of experience in senior management of global law firms. She had also served as a consultant to the non-profit sector with ESC of New England. Ms. Fink is an elected member of the Wellesley, MA Town Meeting, and serves as a member of the Wellesley Human Resources Board and Board Member of the Cornell Club of Boston

Lori S. Morton, Director of Development

Lori works in partnership with the Executive Director to provide leadership and strategic direction for the fundraising efforts of the foundation. Her extensive experience in the field includes Director of Philanthropy and Operations for the LuMind Foundation and Major Gifts Officer for the Accelerated Cure Project for Multiple Sclerosis.

Lori is a graduate of the University of Massachusetts, Amherst, MA. She is a member of the Association of Fundraising Professionals, Boston Philanthropic Advisors Roundtable, The Friday Forum and Women in Development. She is also a volunteer tutor with Literacy Unlimited.

Chapter News and Annual Events

We cannot get to the cure without our dedicated volunteers!

PRF chapters and other volunteers who organize annual events are vital to our mission. As PRF ambassadors, they build tremendous support within their communities, and raise awareness globally. With their help, we can meet our commitment to fund clinical trials, award research grants, and support all of our other research-related programs.

Team "Fighting for Their Future" becomes the newest PRF Chapter!

(l-r) Nathan, Mark, Libby, Phyllis and Bennett Falcone

For the past 5 years, the Falcone family and their supporters have held events and raised awareness for PRF. Now, they are teaming up with PRF as an official chapter. Their community in Flourtown, PA (just 15 miles north of Philadelphia) looks forward to their annual Make A Splash event, and we are excited to work with the chapter board to plan other activities. WELCOME ABOARD!

"We are so excited to be an official chapter of PRF!" says Phyllis, "We can continue to appeal to friends and family who support Nathan and Bennett, while benefitting from PRF's resources. All this means more funding for research so we can find a cure for all the children with Progeria."

Make A Splash: much more than a dip in the pool!

Everyone had a blast at the 4th annual event at the Flourtown Country Club. Swimming, dancing, silent auction, bake sale, balloon artist and ring toss kept everyone busy on a perfectly warm and sunny day.

The dance party is always a big hit!

A Race to Remember…

The entire Foxboro High School track team ran the 5k in honor of Sam Berns

PRF's 13 annual International Race for Research last September was especially meaningful. Held in PRF's hometown of Peabody, MA, the race was dedicated to Sam Berns, the inspiration for PRF's creation who ran each year since its inception. A record crowd of nearly 500 friends, family and other supporters – including his fellow classmates from Foxboro High School – came together to honor Sam. "Sam loved coming to this event, thanking everyone and crossing the finish line", said his parents, Drs. Leslie Gordon and Scott Berns, in their welcoming remarks to the participants. "We are overwhelmed by everyone's support for Sam and PRF."

A runner wearing the special edition t-shirt

(l to r) Kate Carolonza, Hailey McLaughlin & Kimmie Klinger give it all they've got!

Spinning for a CURE!

The 5th Annual Spin for Life spin-a-thon was held at the Lakeland Hills YMCA in Mountain Lakes, NJ in February. This annual NJ Chapter event has raised $160,000 since it began – Keep on peddlin', TEAM ZOEY!

We hope YOU will be inspired to attend one or more of these events next time, or start your own - email *volunteers@progeriaresearch.org* and we'll get you started!

The Michigan Chapter does it AGAIN!

This May 2015, the 10th Annual Miles for Miracles was held in Flat Rock –10 YEARS, AMAZING! Everyone enjoyed the auction, raffles, bake sale, DJ, and kids entertainment, but the biggest hit of the day was the bright orange shirts designed by Lindsay! Thank you to the army of volunteers that help make this event so successful – #yourock!

(l to r) Kristy, Joe and Lindsay leading the way for hundreds of walkers

TEAM PRF runs for a cure at the Falmouth Road Race for the 2nd year in a row!

It was a hot and humid day in August, but TEAM PRF finished strong! Eleven enthusiastic runners wearing bright blue & orange shirts helped raise awareness along the spectator-packed (over 75,000!) 7-mile race route, and over $15,000 for Progeria research. WAY TO GO, TEAM!

It's a Zach Attack!

Zach and his biker buddies are ready to roll at the KY chapter's 5th annual Zach Attack Ride for Progeria. Mom Tina said it best on her Facebook post: "Our hearts are so full every year witnessing the love and support for Zach. It's awesome each year to watch it grow and see more people fall in love and be touched by Progeria."

You Scream, I Scream – for PRF!

For the 2nd year in a row, Freeze Daddy's Ice Cream partnered with the Ohio chapter in honor of Kaylee and Carly-Q with raffles, an auction, a car show and, of course, ice cream – a delicious way to raise $2,800! Thanks to the Loeffler's and all the ice cream lovers in Monclova, OH for making this event so successful.

All FORE a Cure for Progeria!

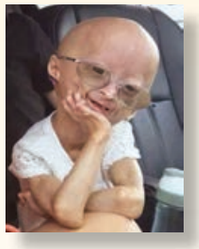
Cam took charge, getting the Butler, PA golfers ready at the 2015 PA chapter Cam's Course Tournament – now in its 7th year!

The First of Many Hikes for Hope…

Nathan and Bennett inspired Amy Rufh to hike over 250 miles on the Appalachian Trail in Pennsylvania during her inaugural Hike4Hope last summer. With the support of her congregation and extended community, she raised over $45,000 for PRF! This year, Amy is tackling portions of the trail in NY, NJ and MD. REMARKABLE!

Nathan and Bennett join Amy during the hike

Everything's Coming up Roses…



Over 840 "Rosebuds" (named for 8-year-old Adalia Rose) donated to the Texas chapter's Team Adalia ONEpossible page – WOW! Thank you all for helping make our campaign a huge success!

Hats ON for Progeria 2015 – Hats OFF to YOU!

Our 3rd annual Hats ON for Progeria Day was a phenomenal success – check out these stats!

- Raised $20,000 – twice as much as last year.
- Participants hailed from 14 countries – 3 times as many as in 2014.
- In the US, schools, companies and individuals from 19 states were involved – a 4-fold increase.

(l to r) Sisters Chelsea and Bentley Werner, Georgia, during their April visit to the PRF offices

Save the Date: Join us on Friday, April 15, 2016 and help us make it the biggest Hats ON day ever!

Students at Medicine College of the Mogi das Cruzes University, Brazil

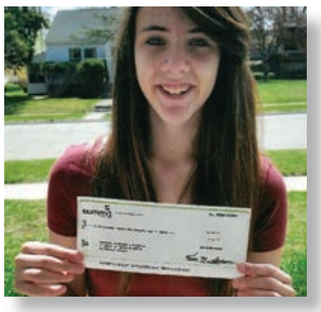
Employees at Berbés Asociados, Madrid

Miracle Makers make things happen for PRF!

They raise awareness by sharing our mission, as well as critical funds with bake sales, lemonade stands, penny collections, birthday gift money and in lots of other generous and creative ways. We are so grateful for all the support we receive from this very special group of people!

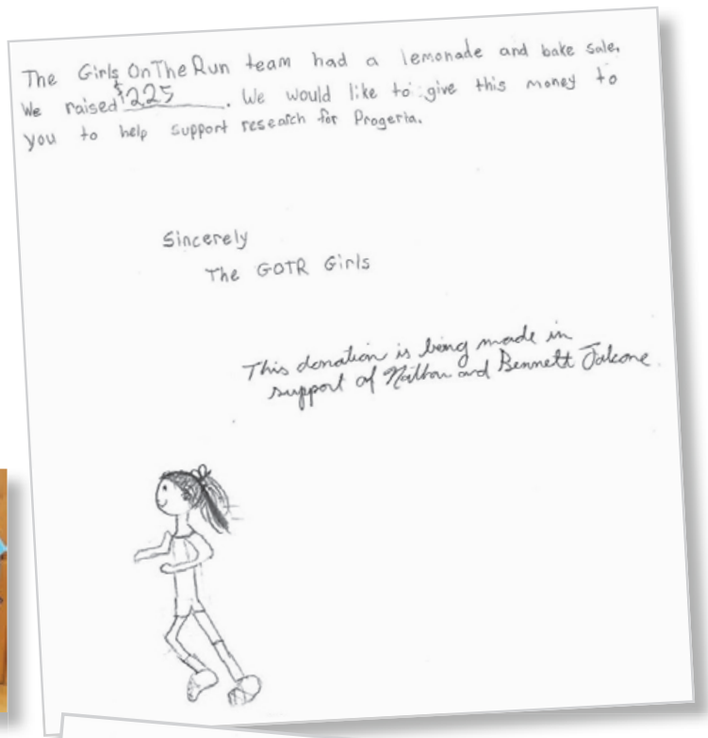
Lafayette, KY Christian Child Care School students holding their annual bake sale for PRF. This year they raised $190 – GREAT JOB!

Hope Childcare in Whitehouse, Ohio held PENNIES for PROGERIA on Rare Disease Day and raised $926 for PRF, in honor of Carly Q. Now that's a LOT of pennies!



"My name is Dezarae. I put together a fundraiser for Hats On for Progeria in honor of Megan Nighbor. I did this because everyone does fundraisers for other things and I wanted people to know about this. I did this for my school in Portage, WI at Wayne Bartles Middle School. I sold suckers and had a penny war."

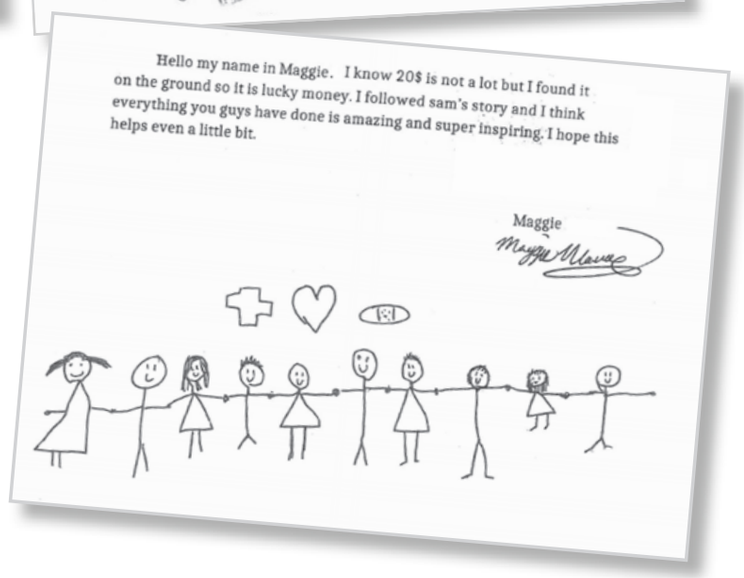
MAIL CALL! We love these letters from our young Miracle Makers, and know you will too!



The Girls On The Run team had a lemonade and bake sale. We raised $225. We would like to give this money to you to help support research for Progeria.

Sincerely
The GOTR Girls

This donation is being made in support of Nathan and Bennett Falcone



Hello my name in Maggie. I know 20$ is not a lot but I found it on the ground so it is lucky money. I followed sam's story and I think everything you guys have done is amazing and super inspiring. I hope this helps even a little bit.

Maggie

We wish we could include stories and photos for all the fundraisers our chapters and Miracle Makers hold throughout the year, but check out our Facebook page and progeriaresearch.org/get_involved for more information and ideas on how YOU can help find the cure!

Sam Berns' legacy of inspiration continues…

And the winner is...

LIFE ACCORDING TO SAM (LATS) won an Emmy for "Exceptional Merit in Documentary Filmmaking". Congratulations to HBO Documentary Films, Sean Fine and Andrea Nix Fine, and the entire talented, passionate team who have helped raise awareness of Progeria and PRF's work through this exceptional film. ***Most of all, we thank Sam – our eternal inspiration.***

The Emmy is the latest in a long line of awards and accolades the film has received since premiering at the **Sundance Film Festival** in 2013:

Christopher Award for 'work that affirms the highest values of the human spirit'

Peabody Award which recognizes 'stories that matter'

Norman Vaughan Indomitable Spirit Award: Mountain Film Festval, Colorado

Audience Award: Nantucket, Woods Hole, Newburyport, Martha's Vineyard and Boston Jewish Film Festivals

Best Documentary: Rhode Island International, New Hampshire and Woods Hole, MA Film Festivals

Best Storytelling: Nantucket, MA Film Festival

"Best of Fest": AFI Docs, MD

With its story of love, determination and hope, LATS and Sam continue to positively and profoundly impact people throughout the world.

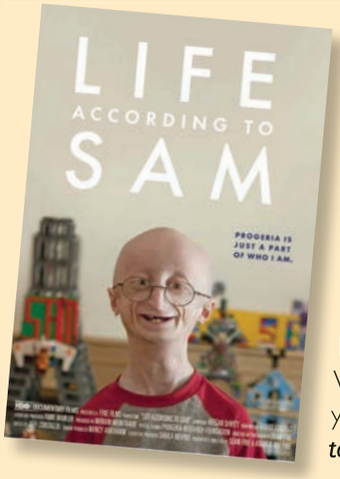
Breaking News: Sam Berns' TEDx talk reaches 10 Million views!

In October 2013, Sam Berns gave a TEDx Mid-Atlantic lecture entitled, "My Philosophy for a Happy Life", raising awareness of Progeria in a unique way. Since then, his video has been shared on YouTube, Twitter, Upworthy, and through websites featuring inspiring quotes and people. On Sept. 2, 2015, it hit 10 million views, making it the 2nd most-watched TEDx video of all time – out of over 30,000 talks! Check out the reaction to the talk at **#SamBerns** and **#LiveLikeSam**

"There's so much we can learn from this young man. He changed my outlook on life! Thanks Sam!"

"Sam Berns is an incredibly wise young man who proves that from life's greatest challenges come the greatest people"

"Sam's legacy of wisdom and optimism lives on, inspiring people to live a full and happy life."



Attention Jr. and Sr. High School Teachers and Students: Bring LATS to your classroom!

Inspiring and heartwarming, LIFE ACCORDING TO SAM is also a powerful tool for engaging students in learning. The film sheds light on: • The biology of disease • The painstaking process of testing treatments • How determination can fuel creative problem solving in the face of adversity • Lessons in inclusion and interpersonal relationships

PRF has created a Discussion Guide for teachers, with dialogue-generating questions on the following topics: • Science and technology • Problem solving • Empathy and relationships • Opportunities for service learning

Download the materials at ***progeriaresearch.org/LATS***.
We know it will be an important and memorable part of this year's learning experience at school. ***We also encourage students to watch Sam's 12-minute TEDx talk.***

"I am a 7th Grade Science instructor, teaching Genetic Disorders to my young students. Through discussion, lectures, and diagrams, we are learning about mutations, chromosomes and genetics. I culminate the unit in showing Life According to Sam. The students have outstanding questions and are completed fascinated as they watch Sam's story unfold. Sam's message is being heard, and will continue to be heard for years to come in my classes."

High School Band Members & Directors: *Check out* ***keepmovingforward-music.com!*** *Arrangements written in honor of Sam and his love of music, Mark Miller's "Keep Moving Forward" and Don Albro's "Keep Burning Bright" are powerful and heartwarming, offering unique learning opportunities for music students.*

Progeria Research Foundation
P.O. Box 3453
Peabody, MA 01961-3453

RETURN SERVICE REQUESTED

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

Zach hopes everyone has a great school year!

Night of Wonder 2016 is the hottest game in town!

Tickets, ads and sponsorships now available for PRF's signature gala and auction

Please join us
Saturday, April 9, 2016 in Boston, MA
for fabulous food, delectable drink,
and an astounding auction...
this is your opportunity
to get in the game and help us
SCORE a CURE for Progeria!

*Visit **www.prfnow.org** today*
to reserve your ringside seat!
For more information,
email prfnow@gmail.com
or call 978-535-2594.