

news

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Night of Wonder — SAVE THE DATE!	BACK COVER

Lonafarnib Managed Access Program Launched!

Trial participation and travel to Boston no longer required for most children in order to access this treatment.



It took 16-year-old Mateo and his mother 21 hours to travel to Boston from his village in Argentina, for his final 2-drug trial visit in July. Thanks to the Managed Access Program, he may be able to get lonafarnib through his local physician.

We are thrilled to announce that the lonafarnib Managed Access Program (MAP) is now up and running. PRF and lonafarnib manufacturer, Eiger BioPharmaceuticals, have worked together to create this Program. The MAP enables eligible children and young adults with Progeria to obtain the drug lonafarnib through their local physicians in countries that allow the MAP to be offered.

See page 4 for more details on this exciting program.



★★★★★
CHARITY NAVIGATOR
Four Star Charity

We did it again!
PRF joins just 8% of reporting charities in accomplishing this highest distinction for **6 straight years**.

Our scientific workshops are a driving force in the search for the cure!

Details on our latest international conference on pages 8-9.



A Message from the President and Executive Director

Dear PRF Family,

It's been 20 years since PRF's inception, and I marvel at all we have accomplished together in that time, all the activity going on today, and all that is ahead of us. Thanks to YOU, we will continue our remarkable, fast-paced journey in full force, until we CURE PROGERIA.

One of the most striking changes is that we now refer to the population we serve as 'children *and* young adults with Progeria'. Our discoveries have shown that lonafarnib is helping those with Progeria live longer, healthier lives. And as the lead story in this newsletter demonstrates, PRF has ensured that all those with Progeria have access to it while we continue to search for even more effective treatments and the cure.

Another demonstration of how far we've come can be found in the 2nd edition of our Clinical Handbook (details on page 7). This 130+-page book contains updated and new information gleaned from extensive research over the past decade since the first edition, including new chapters on genetic counseling, lonafarnib treatment, and adolescence, as well as expanded chapters on cardiovascular care.

In the short time since PRF's inception, we have progressed from:



No medical knowledge of this condition, to gene discovery that led to the first ever clinical drug trials for children with Progeria...



Obscurity of one of the rarest diseases on earth, to worldwide awareness and recognition...



Almost no attention by clinicians and researchers, to Progeria seated in the forefront of scientific efforts to discover other treatments and the cure.

PRF's 20 years of existence has been an extraordinary journey of hope and passion, fueled by the courage of the families, and the support of thousands like you around the world. I have no doubt we will continue in the same way, until Progeria is cured.

Our driving force is reflected in the Handbook introductory message from Sam Berns' mom and PRF co-founder/Medical Director Dr. Leslie Gordon:

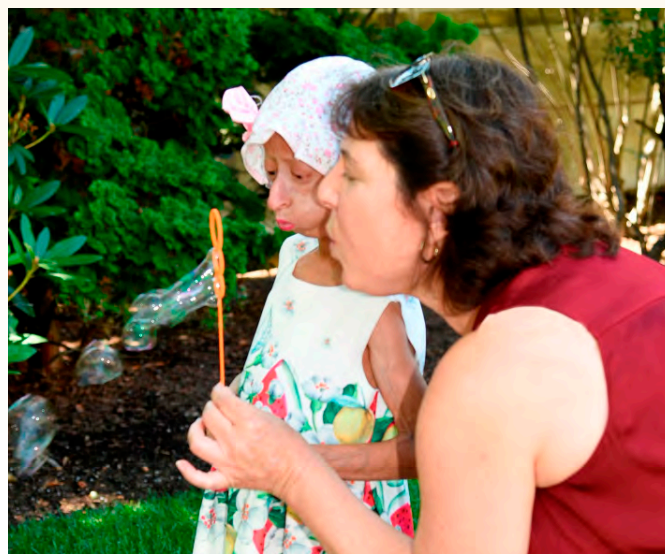
Most importantly, this handbook was created from love – the love that helps us all strive every day to make a difference in the lives of children who deserve every happiness that life can offer.

Many thanks to all of YOU - for the love you bring to PRF, and to the children and young adults with Progeria.

Together, we *WILL* find the cure!

Audrey Gordon, Esq.

President and Executive Director



*In July, Audrey and Leticia enjoyed some
playtime near Boston Children's Hospital.*

PRF's Vision:

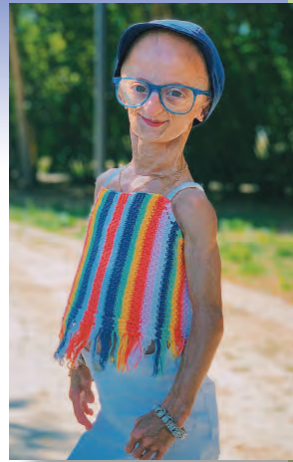
A world in which every child with Progeria is cured.

PRF's Mission:

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

STAFF:

Audrey Gordon, Esq. *President & Executive Director*
Gina Incrovato *Director of Operations*
Kelsey Tuminelli *Clinical Trial Coordinator/Family Liaison*
Michelle Fino *Event Director and Development Officer*
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Christina Sollecito *Executive Administrative Assistant to the Medical Director*
Odette Kent *Data Specialist*



Progeria is a fatal, "rapid aging" condition 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 heart disease and aging, what we learn from Progeria research has the potential to benefit all of us.

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Meghan Waldron

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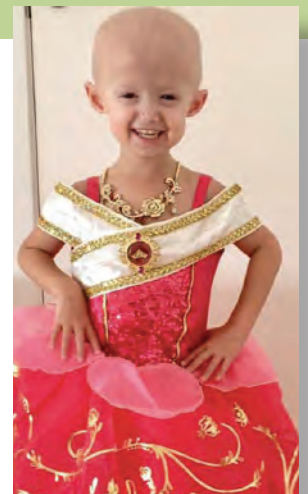
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Lonafarnib Managed Access Program Q&A

Ensuring Access to Progeria and Progeroid Laminopathy Treatment for All

1. What is a Managed Access Program (MAP)?

A managed access program enables an eligible person with a life-threatening condition to gain access to a medicine that has not yet been approved by their country's regulatory authority for commercial sale.

2. Who is eligible to participate in the MAP?

The MAP will provide lonafarnib to eligible children and young adults with Hutchinson-Gilford Progeria Syndrome and Progeroid Laminopathies in three categories:

- *those who have never taken lonafarnib before;*
- *those who have taken lonafarnib before but are not at this time;*
- *those who have been taking lonafarnib as part of a clinical trial and would like to continue upon completion of their participation in the clinical trial.*

3. What about countries where the MAP is not available?

Children who live in countries where the MAP is unavailable will be able to receive lonafarnib through the current PRF-funded clinical treatment trial at Boston Children's Hospital. PRF continues to assure access to lonafarnib for all children, worldwide!

4. What is needed to be considered for the MAP?

All those eligible and wishing to participate must have a local doctor who is willing to assist with enrollment, ordering, dispensing, and product use. A patient may only be registered for the MAP by their treating physician.

5. How long will the MAP last?

In the US, Eiger BioPharmaceuticals will continue to provide access to lonafarnib through the MAP until lonafarnib is approved by the FDA and thus available commercially. PRF and Eiger are currently pursuing FDA approval. In some countries, MAP will be in place even after the drug is approved in the US.

For more information on the MAP, please follow this link: <https://www.progeriaresearch.org/clinical-trials/>

For doctors to enroll their patients in this program, please email Clinigen's Medicine Access team at medicineaccess@clinigengroup.com or call +44 (0) 1932 824123.

For those who have been taking lonafarnib as part of the PRF-funded clinical trial and would like to continue lonafarnib treatment upon completion of their trial participation, MAP bridges the gap between completion of clinical trials and FDA approval.



Amber and her brother Michiel of Belgium walk along Boston's historic waterfront during their clinical trial visit. Read more about them on page 6.

Clinical Trial News

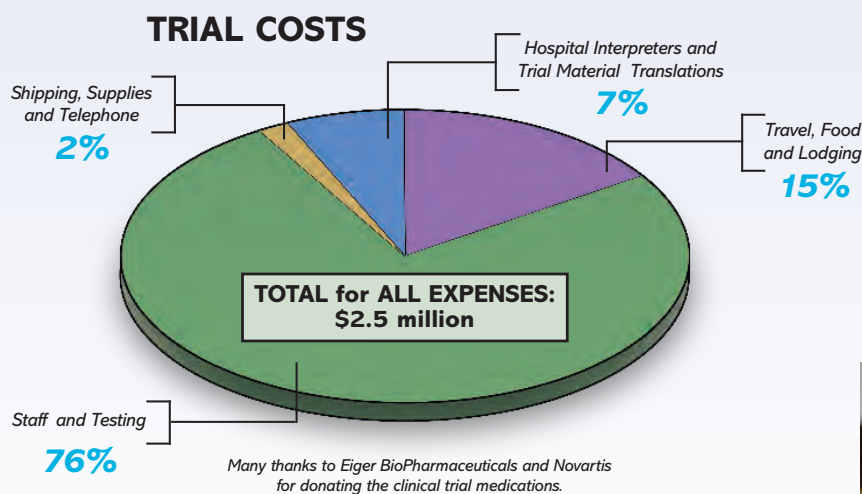
Everolimus plus Lonafarnib 2-Drug Trial Update: Lots of visits, lots of progress!

With 1/3 of the children and young adults having finished their trial visits, PRF and the trial team are achieving all milestones of enrollment, trial visit completion, and budget for the Progeria 2-drug clinical trial! All trial expenses are covered by PRF, and trial medications are donated by Eiger BioPharmaceuticals and Novartis. Families have flown to Boston from 26 countries, speaking 17 languages, to participate. Cost and language are no barrier thanks to PRF's donors, chapters, and volunteers!

Trial Goals: Our hope is that this 2-drug combination of everolimus and lonafarnib is more beneficial than lonafarnib alone. Research has shown that lonafarnib may block the toxic, disease-causing protein, *progerin* from developing in the first place, and everolimus appears to help cells better eliminate the progerin that does develop and attack the body's cells. Thus, the combination may prove to be a "one-two punch" to Progeria.

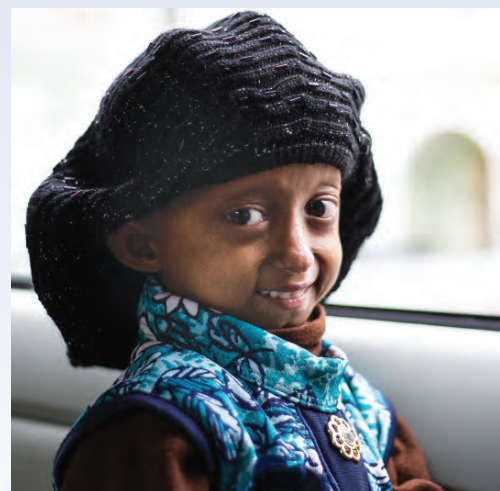


60 children have traveled to Boston from 26 countries for PRF's 2-drug trial.



All 60 enrolled children have completed their first of two visits to Boston, and 20 have completed both. Fourteen more are scheduled for their final visit in 2019, and the remaining children will finish in 2020. In 2021, a rigorous review of the trial data will determine whether the 2-drug combination is an effective treatment for Progeria. We are hopeful that the addition of everolimus will give these children and young adults even longer, healthier lives.

Progeria is a fatal, premature aging disease that causes severe premature atherosclerosis. Without lonafarnib treatment, children die at an average age of 14 years. The drug lonafarnib has been shown to increase the children's lifespan and give them stronger hearts so they can be as active as possible. Such exciting progress!



8-year-old Prachi from India was in Boston for her final trial visit in May. She took a break from her hospital visits to play the piano at a local children's play center, dressing up for the "recital" in a colorful tutu!

THANK YOU for making these trials – and this tremendous progress – possible.

An Interview with Michiel and Amber

21-year-old Michiel from Belgium and his 13-year-old sister Amber have been involved with PRF's clinical trials and other programs for many years. Michiel participated in our very first trial in 2007, and Amber started 2 years later. Enjoy getting to know them!



What are your favorite hobbies?

Amber: Watching Netflix shows on my iPad. Some of my favorites are Friends, The Big Bang Theory, and Riverdale.

Michiel: Playing video games, spending time with friends.

What words best describe you?

Amber: Inquisitive, social, honest, family-oriented

Michiel: Awesome

If you could only eat one food forever, what would it be?

Amber: Chicken wings! When we are in Boston, I get them with BBQ sauce.

Michiel: Steak! Any kind, rare, with creamy mushroom sauce or from a Japanese place with garlic, onions and soy sauce.

What has been the happiest day of your life so far?

Amber: I don't know, there are a lot of beautiful days. And I'm very grateful for the things I can do.

Michiel: I can't choose a particular day, there are so many, but I'm so grateful that I've been able to do lots of fun things.

What are some of your favorite places you've traveled?

Amber: New York, Denmark, Amsterdam, Disneyland, Rhodes, Portugal

Michiel: Universal studios in Florida, New York, Amsterdam, and so many more!

When were you diagnosed with Progeria?

Amber: 6 weeks after I was born

Michiel: When I was 5 years old

What has your experience been so far with getting treated for Progeria? (When were your clinical trial visits, what does your current regimen look like, etc.?)

Amber: Starting in August 2009, I've gone to Boston every 2 years. Every weekday after school time, the physiotherapist comes around. And I take my medication every day.

Michiel: I started in 2007 and at first I had to go back to Boston every 4 months. Now, it's every 2 years, and I get physiotherapy every day. I also take lonafarnib every day.

What do you wish more people understood about you?

Amber: That I'm just a kid. I want to do normal stuff like everyone else. And I like hanging with the teenagers.

Michiel: That just because I look different doesn't mean I am different.

Research Grants

Breakthrough Research Continues as PRF Grants Two More Awards

Our research grant program began almost immediately upon PRF's creation. Given the extreme lack of knowledge of this rare condition in 1999, PRF knew that basic science was the key to finding treatments and the cure for children with Progeria. With the Progeria gene discovery in 2003, and 20 years of PRF research funding, the science of Progeria has gone from obscurity to treatment trials, and we are zooming towards new cutting edge treatments. **How far we have come!**

Since 1999, PRF has awarded 73 grants to researchers working in 14 countries! The work produced by these scientists has led to breakthroughs and new treatments that help children with Progeria live longer, healthier lives, while also driving discovery in heart disease and aging. Our latest grant recipients are breaking new ground for the future of Progeria research:



Abigail Buchwalter, PhD

Assistant Professor

*Cardiovascular Research Institute,
University of California, San Francisco*

“Defining the Feasibility of Progerin Protein Clearance as a Therapy for HGPS”

Projects in the Buchwalter lab center around defining the mechanisms that govern the establishment, specialization, and maintenance of nuclear organization across cell types. Of particular interest is the role of the nuclear lamina in instructing the organization of the genome within the nucleus, and defining how this order is disrupted by disease-linked mutations.

This research may influence how treatment strategies are designed for children with Progeria. If progerin is a long-lived protein in critical disease tissues such as the blood vessels, mechanisms to destabilize progerin at those sites will be important for effective treatment strategies.



Martin Bergö, PhD

Professor of Biosciences

Karolinska Institute, Stockholm, SWE

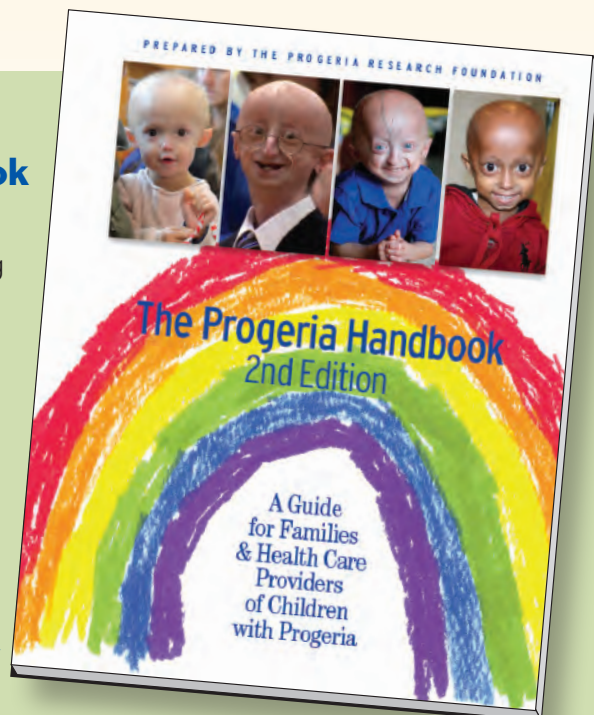
“Development and Preclinical Testing of ICMT inhibitors for HGPS therapy”

Dr. Bergö's research is based on the finding that reduction of ICMT, an enzyme needed for processing progerin, reverses many of the pathological features in Zmpste24-deficient, progeria-like mice. His preliminary studies show that Progeria cells grown in the laboratory grow faster and longer when treated with ICMT inhibitors. Dr. Bergö will test drugs that block this enzyme, and potentially therefore block the production of progerin, looking for whether Progeria mouse models become healthier and live longer when treated with this type of drug.

Announcing the New and Expanded Edition of the Progeria Clinical Handbook

PRF has published its 2nd Edition of **The Progeria Handbook** to help guide families, doctors, schoolteachers and others caring for children and young adults with Progeria (editor, L. Gordon, © 2019). With 33% more material, this 131-page updated version illustrates how far we have come in our clinical understanding of Progeria since the first edition was published in 2010. Additions include sections on genetics and genetic counseling, the lonafarnib treatment, and new cardiovascular recommendations for caregivers. Many thanks to all of our volunteer expert contributors to this major resource.

The handbook is available in English, Japanese and Spanish, and can be downloaded at <https://www.progeriaresearch.org/patient-care-and-handbook/>



2018 International Scientific Workshop

The Progeria Research Foundation's 9th International Scientific Conference in Cambridge, MA, an overwhelming success.

World-renowned physicians, scientists and clinicians from 14 countries were joined by children with Progeria and their families in September, 2018, to share the latest advances in Progeria research and set the stage for new discoveries.

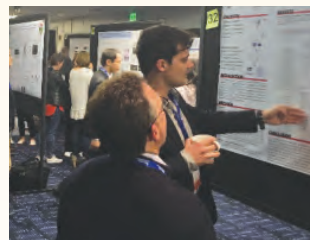


The workshop kicked off with a very special evening program that began with a Family Panel session. Moderated by **Leslie Gordon, MD, PhD** (PRF) and featuring PRF's Ambassador **Meghan Waldron** and her parents, **Tina and Bill**, this session created an in-person connection between children and families dealing with Progeria and the research community.

Eric S. Lander, President and Founder of the Board institute of MIT and Harvard, gave a rousing Plenary Talk on New Genomic Approaches for Understanding Diseases.



The program was packed with fresh new ideas. Scientific presentations highlighted the latest developments in biological and clinical research on Progeria, including new essential research tools such as animal models and biomarkers. New data on small molecules, RNA therapeutics, and 3 talks on gene editing were also presented – all of which will propel us towards our next wave of clinical trials. For more details, please visit progeriaresearch.org/workshop2018/



2-year-old Alptug is captivated by Dr. Collins' guitar playing.



Meghan talks about her many interests and activities, and thanks the researchers for their work. She recently turned 19 and began college in Boston.

Francis Collins, MD, PhD, Director of the National Institutes of Health and central leader in Progeria research then delivered a lively musical kickoff. Dr. Collins delivered a talk entitled *Progeria: from basic science insights to therapeutic opportunities*.

A record 52 Poster Presentations were featured on the evening of meeting day 2. Poster topics included new mechanisms in HGPS, as well as pre-clinical models and the potential of new candidate drugs as a treatment.

One highlight of the workshop was late breaking data presented by **David Lui** (Broad Institute). Dr. Lui spoke about his team's exciting early results on gene editing for Progeria. You can see his TED talk on the subject at https://www.ted.com/talks/david_r_liu_can_we_cure_genetic_diseases_by_rewriting_dna?



For more details, please visit
progeriaresearch.org/workshop2018/

Special thanks to our Workshop Organizers and Supporters:

Scientific Organizing Committee:

Leslie Gordon, MD, PhD, Medical Director,
 PRF, Boston, MA

Vicente Andrés García, PhD, Centro Nacional
 de Investigaciones Cardiovasculares Carlos III (CNIC),
 Madrid, Spain

Tom Misteli, PhD, National Cancer Institute, NIH, MD

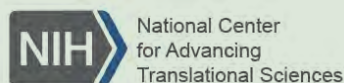
Mark Kieran, MD, PhD, Dana Farber Cancer Institute,
 Boston, MA

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With a 99% approval rating, the feedback was incredibly positive:

- ✓ "A very well-planned and executed workshop. Very motivating!"
- ✓ "Fantastic meeting, with ample discussion of a variety of mechanisms of disease and treatment options in preclinical and clinical settings."
- ✓ "As always, really enjoyed the workshop, and it re-inspired me to continue to work hard for these wonderful children. I love being on this team for the kids."
- ✓ "One of the best conferences I have ever attended."

**The 2020 Scientific Workshop Registration is Now Open
 RESEARCHERS, REGISTER NOW at www.PRFWORKSHOP.org!**

THE PROGERIA RESEARCH FOUNDATION 10TH INTERNATIONAL SCIENTIFIC WORKSHOP

researching possibilities
 exTENDING lives

NOVEMBER 2-4, 2020 AT THE ROYAL SONESTA HOTEL IN CAMBRIDGE, MA, USA

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

Chapter, Family and Annual PRF Events

Special events are key to our quest for the cure. We are delighted to see the thousands of faces who continue to participate in a variety of fundraisers each year – races, bowl-a-thons, car shows, pool parties, and much more - all in support of children and young adults with Progeria. THANK YOU to everyone who came to one or more events this past year – you are ALL helping PRF get closer to the CURE!

6 years running! TEAM PRF Rocked the 2018 Falmouth Road Race!

In a field of over 13,000 runners, the 15 members of TEAM PRF stood out in their golden tank tops bearing PRF's signature logo and tag line. It was a perfect day for running the 7.1-mile route along the Falmouth coastline, and for raising awareness and funds for children with Progeria.



Members of the 2018 TEAM PRF celebrate after the race.

Pins were falling, fun was rising at Team Zoey's Strike Down Progeria bowlathon.

It was a sold-out event at Hanover Lanes in Hanover, NJ, where Team Zoey bowlers enjoyed pizza and arcade games along with their strikes and spares on the lanes. How fun!



BFF's Zoey and Anna are a perfect score!

It was flashback to the '80s at the 14th Miles for Miracles.

Thank you to everyone who came out to support Lindsay and PRF at the **Miles for Miracles** walk. The 1980s costumes added even more fun to this awesome, long-running annual event. GO MICHIGAN CHAPTER!



The Ratcliffes know how to throw a party!



Runner #1021, John Castellet, organized 49 runners – our largest team yet – as part of Team Tufts for Meghan in PRF's 2018 Race for Research!

International Race for Research – 17 years strong!

PRF Ambassador Meghan Waldron (circled above) inspired the 300+ runners and walkers with a rousing speech and by running the 5k course in under 30 minutes – amazing! This year, we officially welcomed our furry friends to the race with dog watering stations along the route.



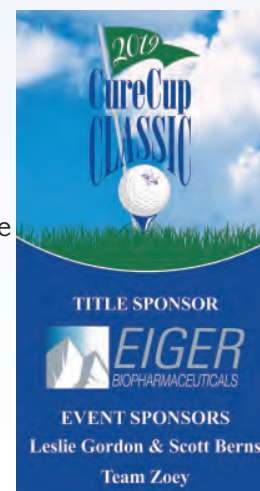
Beasley enjoyed lots of treats and attention on top of a great run!

Interested in running for a great cause?

We welcome runners nationwide to participate in 5ks, marathons and other races on behalf of children with Progeria, and we will work with you to get a bib number if needed. Contact Michelle at mfino@progeriaresearch.org to help win the ultimate race – for the cure!

Over 100 tee off at the 2nd Cure Cup Classic Golf Tournament

Players stepped up their game for the Longest Drive and Closest-to-the-Pin contests, at the Ipswich Country Club. Dinner, raffles, auction and reminiscing over the competition made for a perfect end to the day – thank you to all 'fore' participating!





Our friends at Genpact National Vendor getting silly with HatsON day

Hats OFF to 7 years of HatsON for Progeria

Seven years ago, we invited the PRF community to help raise awareness by taking part in a new tradition – Donning their favorite hats for the very first **HatsON for Progeria** Day. Since then, this event has taken on a global reach, with supporters showing their solidarity from Australia to India, and all across the USA!

Please join us for our Fall HatsON event on **October 4, 2019**, and next year's **HatsON: March 20, 2020!** Don't forget to send us your photos by tagging your social media posts with **#HatsONforProgeria!**

Fishing for the Cure

The third annual *Deallaney Hudson King Mackerel Fishing Tournament* had a huge turnout, with boatloads of sponsors and participants. Contestants headed out on Cape Lookout Shoals along the Southern Outer Banks of North Carolina and then gathered afterwards at the Topsy Turtle to see who had the greatest catch. Many thanks to all involved who continue to make this unique tournament a success!



It was all thumbs up in a sea of support at this year's tournament!



The Falcone family dries off from yet another great year of Make-A-Splash (September, 2018)

More Than A Splash!

Our PA East Chapter's annual signature event, **Make-A-Splash**, is a fun-filled day for the entire family. Featuring a pool party, raffles, silent auctions, 'Mad Science' experiments, yard games, magicians, music and more, this event never disappoints (even in a little rain!)

Team Zach Flies High

PRF's Kentucky Chapter held a 'paint night' party at *Painting with a Twist*. Do you recognize the subject in the paintings? It's the bird in PRF's logo!



Check out Zach's unique color choice in the back row: his favorite color yellow!

Getting Loose with Chip Foose



*Chip and Zach enjoy some private time together, watching *The 3 Stooges* and other vintage comedy!*

2018 marked the 11th **Chip Foose-Year One Braselton Bash** auto show in support of PRF. Car lovers united from across the U.S., bringing their classic auto gems for all to admire. Legendary *Overhulin'* star Chip Foose, whose sister Amy had Progeria, was on hand to sign autographs and judge Best in Show. With door prizes, a silent auction, music, food and a burn-out exhibition, this is the Georgia car show to go to!

Gobbling for Good

Family, friends and classmates of Zoey Penny gathered at the 8th Annual Team Zoey "**Miles of Hope**" Turkey Trot, a 5k road race named one of the top Turkey Trots in the country. Each year, this event gets bigger and better as entire classes of students, walkers, and runners join in the fun!



Zoey and friends are all smiles before taking to the streets of Verona, NJ in November 2018

Our Miracle Makers

PRF's Miracle Makers make a HUGE difference in the lives of children with Progeria.

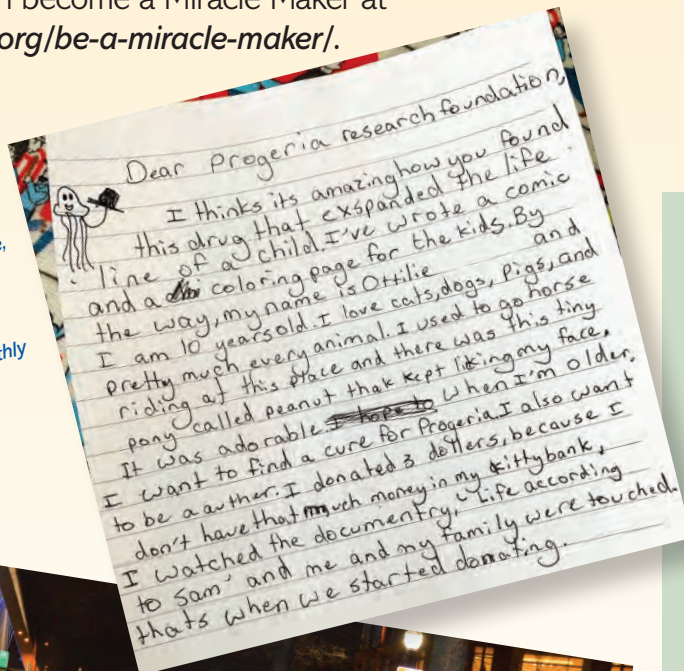
We are so grateful to all of our Miracle Makers – volunteers who help PRF in a variety of ways – for donating their time and energy to help PRF. From our photographers, graphic designers and translators, to our fundraisers, ambassadors and event staff, THANK YOU for sharing in our mission.

Learn how you can become a Miracle Maker at progeriaresearch.org/be-a-miracle-maker/.



Local resident, Penny Papantonakis volunteers to do office work at PRF headquarters in Peabody, MA. Earlier this year, she raised \$700 for PRF, pooled from her 7th grade classmates and donation bins she placed in her family's businesses. Such an industrious and sweet young lady!

A heartwarming letter from Otilie, a young PRF Miracle Maker. Otilie's family are now monthly donors!



"I'm not letting being deployed [in Afghanistan] stop me from donating!"

I've been struck in the heart by the bravery and aura that surrounds these courageous children with Progeria [...] I'm humbled by their attitude and smiles. It helps me through tough times when I'll start complaining about things here, and then these children give me a level of perspective...

Before I was a pilot, I was a music major in college and a junior/senior high school marching band geek. To see Sam [Berns] accepted by that group has been a lasting image and an inspiration to me that, with determination, we all can achieve what others may not think possible.

Thank you for the opportunity to help in a very small way by contributing to the ultimately successful cure for this disease!"

- Glenn B., annual ONEpossible Donor



Last December, 35 awesome volunteers helped sell tickets for a 50/50 raffle held during a Boston Bruins game. This enthusiastic group had a blast cheering on the home team and raising \$15,000 for PRF!



Fluent in Spanish and Portuguese, long-time PRF volunteer Alicia Sheridan was a huge help when Leticia from Brazil and Mateo from Argentina were in Boston for their final trial visits. Alicia was able to assist both with a fun photo shoot!

ONEpossible campaign success!

THANKS to recurring donors like Glenn (above), we've once again had a successful ONEpossible campaign, raising \$150,000 to help fund the research grants, scientific workshops, clinical trials and other work needed to treat and cure Progeria.

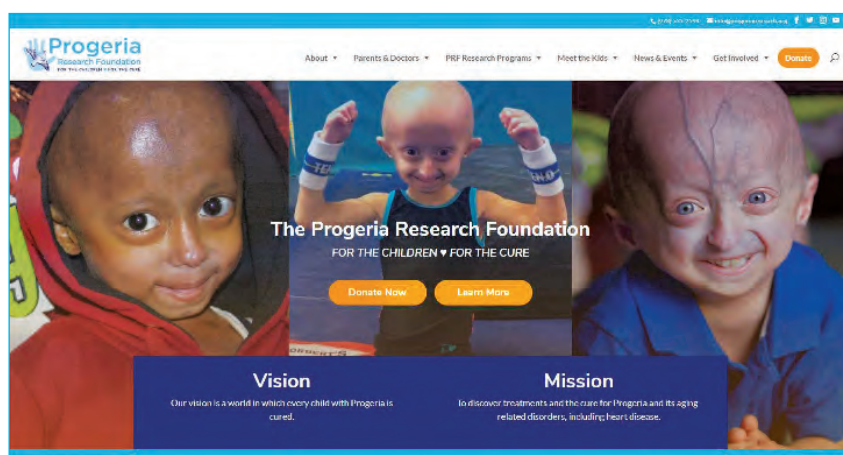
You are all ONE making a cure POSSIBLE!

PRF On The Move!

PRF's New Look

This year has brought a slew of exciting changes to the PRF brand. We officially launched our new logo, carrying over the iconic seagull and Sam Berns' handprint from the previous logo and adding the tagline, "FOR THE CHILDREN ♥ FOR THE CURE," which succinctly captures the 'who' and 'why' of our mission. The heart symbolizes love as a key ingredient – the main motivator in all we do for the children. With over 200 web pages packed with important information, our new website offers easier navigation and better ways to get to know the children we are working to save.

If you haven't already made your way to our new website, check it out at www.progeriaresearch.org.



PRF Medical Director Delivers Lectures at Universities and Symposiums

Dr. Leslie Gordon is highly sought after for speaking engagements, and is often invited to present in the University setting. As a world expert in Progeria research, she recently presented "Hutchinson-Gilford Progeria Syndrome: Progress Towards Treatments and a Cure for an Ultra-Rare Fatal Pediatric Disease" to Brown University students, and "Progeria: The disease, the challenges and the history" at Boston University.

Nationally, Dr. Gordon spoke at a symposium honoring the long-time Progeria researcher, Dr. Robert Goldman, who has served as chair of the Department of Cell and Molecular Biology at Northwestern University since 1981.



Dr. Leslie Gordon (back, right) is pictured with other presenters and organizers at the symposium celebrating the career of Dr. Robert Goldman (front, center)



Find the Child logos for India and China. Each logo incorporates colors from the country's flag, and also brings in the iconic hand from the PRF logo.

'Find the Child' Campaign Launches

Due to the tremendous success of previous years' campaigns in 2009 and 2015, we are excited to launch our **'Find the Child'** initiative to search globally for the undiagnosed children with Progeria so that they, too, can have access to the unique care they need. In partnership with GlobalHealth PR, a world-wide health communications group, as well as its sister agencies abroad - MediaMedic in India, and Madison Communications in China, PRF is building an international awareness campaign to assure the greatest possible reach. With approximately 200 unknown, about 2/3 of whom we believe are in China and India, we hope this effort brings many more children to PRF.

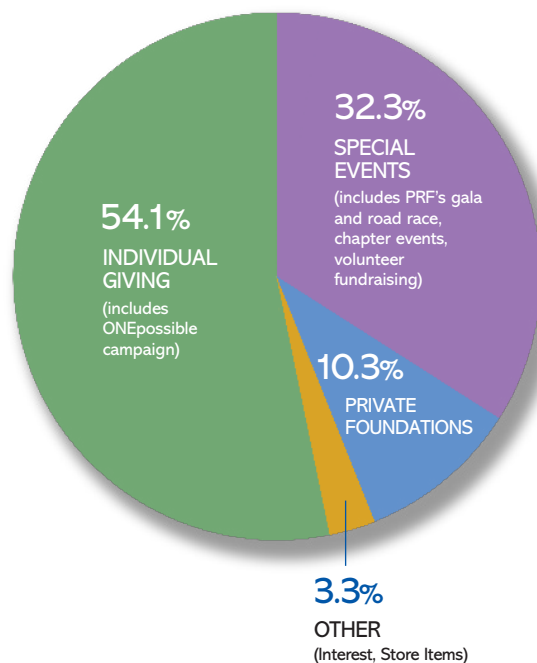


We have a full house! We're thrilled to announce that PRF has grown to a staff of 12 full- and part-time employees at our Peabody, MA headquarters. Among our latest additions is a part-time communications manager, Eleanor Maillie, filling a brand new role at the organization.

You can see all individual staff photos and roles at www.progeriaresearch.org/officers-and-staff/.

PRF 2018 Annual Report

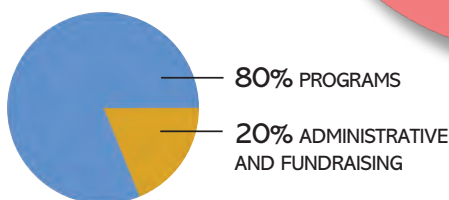
INCOME ANALYSIS - 2018



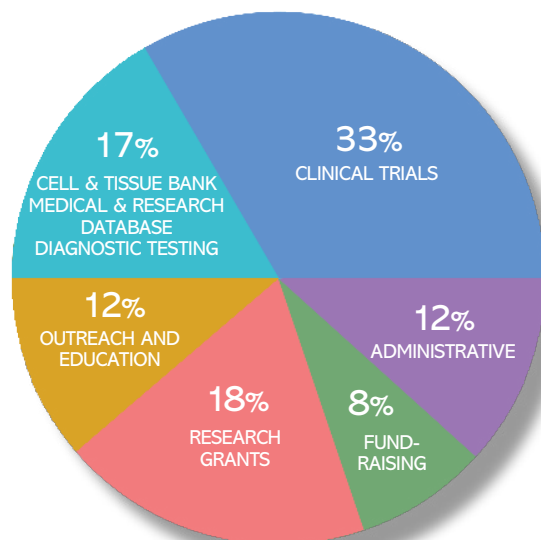
TOTAL REVENUE
\$2,884,715

TOTAL EXPENSES
\$2,507,376

PROGRAM RATIO



EXPENSE ANALYSIS - 2018



Core PRF Activities That Support Our Mission

2018 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY (1999 - 2018)
17 children from 12 countries	International Patient Registry	258 children with Progeria from 62 countries and all continents have registered with PRF
126 cell lines sent to 27 teams in 8 countries	Cell & Tissue Bank Diagnostic Testing Program	1027 cell lines sent to 161 teams in 23 countries; 141 children tested through PRF
13 children enrolled; treatment guidelines provided to all	Medical & Research Database	176 enrolled; treatment guidelines sent to the families in 48 countries and 1 U.S. territory
Everolimus + Lonafarnib Trial, phase II begun	Clinical Trial Funding & Co-coordination	96 children from 37 countries have participated in PRF clinical trials, initiated in 2007.
3 research grants ongoing	Research Grant Funding	71 grants to 41 labs in 15 countries; funding total: \$7.7m
2018 International Workshop held	International Scientific Meetings	13 meetings: 9 general workshops and 4 subspecialties
35 events, 125 new volunteers	Volunteer Program	4,800 volunteers worldwide have helped raise awareness and funds
27 children who visited the U.S. and required an interpreter	Translations Program	PRF's program and medical care materials are translated into 38 languages
20 new children identified	Public Awareness	Presently: 161 living children from 49 countries

In Loving Memory...

*Gone from our sight,
but never our memories ~*

*Gone from our touch,
but never our hearts.*



Ishan

7 years old,
from India



Alexey

7 years old,
from Russia



Ludmila

16 years old
From Argentina



Josiah

14 years old,
from the United States

Bharath

19 years old,
from India

We honor those who have passed away over the last year,
and will forever keep them close to our hearts and minds.



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

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



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NIGHT OF WONDER!

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

Join us on Saturday evening, April 25, 2020
in Boston, MA for festive food, delectable drink,
and an auction that is *out of this world...*

Help us get to the cure with 'flying colors' –
Visit prfnow.org today.

For more information, email prfnow@gmail.com or call 978-535-2594.



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