Progeria Research Foundation
FOR THE CHILDREN ♥ FOR THE CURE

WWW.PROGERIARESEARCH.ORG • SEPTEMBER 2023

news

Brand-New Progeria Test Measures Treatment Benefit Faster and Reveals Even Longer Lifespan Benefit with Lonafarnib!

PRF's remarkable progress toward new treatments and the cure continues: our Medical Director, Dr. Leslie Gordon, and her team have discovered for the first time that progerin, the toxic protein that causes Progeria, is present and detectable in the blood. In light of this, the team developed a blood test for progerin, giving us a brand-new **Progeria biomarker.** This biomarker revealed that progerin levels dropped by over 40% after just four months of lonafarnib use. Researchers may be able to find even better treatments if future clinical trial drugs can decrease progerin blood levels even more. The progerin blood test is sure to help speed up future treatment and cure discoveries!

With this novel biomarker tool, the scientists also found that, with long-term lonafarnib treatment, **life extension for children and young adults with Progeria is greater than we thought** – MUCH greater!

Three friends and long-term lonafarnib users: siblings Amber (left, age 17) and Michiel (right, age 25) from Belgium, met up in Italy with Sammy (center, age 27) over the summer.

See page 6 for more details on these exciting findings.

A Message from the President and Executive Director

Dear Friends,

There are so many incredibly rewarding aspects of my job:

- Witnessing friends and family of a little boy named Sam Berns, my nephew, come together 24 years ago – and every day since – to help PRF grow and succeed;
- Meeting new people from all walks of life – from famous celebrities, to talented pro athletes, to brilliant scientists, to thousands of others who donate what they can – all of whom have joined the PRF family for no other reason than they just want to help;
- Being part of the extraordinary progress we have accomplished – and continue to achieve together – towards treatments and cure.

And by far, the #1 highlight of my days is **spending time with children and young adults with Progeria** – the people at the core of our mission, for whom all of the activity in the pages that follow is done.

Their laughter at blowing and chasing bubbles; their intense concentration while we play Jenga or Connect Four; their personalities shining through as they show me their favorite "pose" for the camera; their endurance during full days of testing at the hospital; and the loving bond with their parents who guide them through their journey to Boston for life-saving treatments that PRF simply could not fund without your support – how lucky I am to experience such extraordinary character and love!

The many faces of Sammy and Audrey - goofing around at the Laminopathies Conference hotel. (Madrid – June 2023)

Having fun taking selfies with 5-year-old Zein from Egypt and 13-year-old Aaditya from India. (Boston - August 2023)

Enjoy the next 14 pages, full of the latest news on exciting research breakthroughs and other activities (SO many!), organizational growth, fundraisers that fuel the work, and, of course, photos and stories about children and young adults with Progeria.

Because it's all...
For the Children ♥ For the Cure.

Together, we *WILL* find the cure!

Audrey Gordon, Esq.
President and Executive Director

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.

Progeria is a rare, fatal, "rapid-aging" condition. Without treatment, children with Progeria die of heart disease at an average age of 14.5 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.

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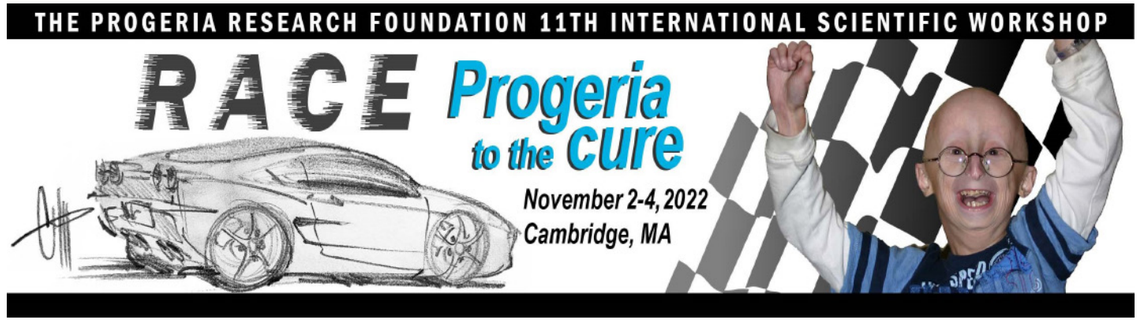
PRF's 2022 International Scientific Workshop



PRF's 11th International Scientific Workshop – *Race Progeria to the Cure*, **was a huge success,** bringing together 124 registrants from 14 countries. Leading physicians, researchers, and families with Progeria met in Boston, MA, to share the latest findings in Progeria research and set the stage for future collaborative efforts to discover new treatments and the cure. Many new partnerships were forged, and cross-continental collaborations between the research and medical communities have resulted!

We kicked off opening night with our **first-ever panel moderated by an adult with Progeria,** 27-year-old Sammy Basso from Italy, which included personal updates about school, travel, and work from Sammy and others with Progeria, both in-person and virtually from around the world. Attendees also enjoyed **a surprise musical performance by Dr. Francis Collins,** White House Science Advisor and whose lab led the discovery of the Progeria gene. Dr. Collins serenaded the audience with several popular songs with modified lyrics about PRF's progress toward the cure. ***How special!***

Sammy (left), Merlin (right) and Carlos (center) captivated the audience by sharing the new and exciting activities they've accomplished in the past year.

Over the next 2 days, the spotlight turned to **28 oral presentations and 26 posters,** which showcased recent scientific discoveries and progress in the development of new drugs nearing clinical trials, such as the small molecule drug **'Progerinin'** (see page 7 for more details). Researchers also presented findings on the transformational potential of **RNA therapeutics and genetic editing** to correct the root cause of Progeria, as well as new **cardiovascular surgeries for aortic stenosis** that are available for young adults when needed.

For the first time, we hosted a **Sunrise Session,** aimed at connecting junior investigators with field leaders at small table rounds for an intimate hour of conversation. The tables were full, and the excitement about the mentorship was palpable and carried over into the rest of the sessions.

A workshop favorite was the **Lightning Round** - a 1-minute, single slide presentation by 26 junior scientists who presented dynamic teasers of the posters they would present that evening, hoping to attract workshop attendees to their posters for comprehensive discussions.

Poster presenters line up for their 1-minute lightning round presentations.

Attendee feedback was once again exceptional! Here are a few examples:

- *" Excellent conference with phenomenal speakers."*
- *" As always, outstanding workshop; can't be missed!"*
- *" It was a great pleasure to take part in the meeting. Felt great energy and passion toward the research."*

For more details, visit https://bit.ly/2022ScientificWorkshop.

Dr. David Liu , Professor at Harvard/ Broad-MIT, delivers the plenary talk on Base Editing and Prime Editing

Special thanks to our Workshop Organizing Committee...

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

Audrey Gordon, Esq.
President and Executive Director, PRF, Boston, MA

Vicente Andrés, PhD
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And our Workshop Supporters:

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PRG Science & Technology

NIH National Center for Advancing Translational Sciences

NIH National Institute on Aging

Asociación Progeria Alexandra Peraza

We look forward to convening the Progeria scientific community for the next phase of progress toward a cure at PRF's 2024 workshop!

Biomarker Discovery in Progeria *continued from front cover*

Previous studies showed that lonafarnib, the only FDA-approved treatment for Progeria, increased lifespan on average by 2.5 years. Now, time and the biomarker test have demonstrated that life expectancy for those on the drug for 10 years or more increases by ***almost double*** that figure, to an average of 4.5 years. This represents **more than a 30% increase in lifespan** - AMAZING!

The study was published in the world's top cardiovascular journal *Circulation*, vol. 147:

Leslie B. Gordon , MD, PhD; Wendy Norris, MS; Sarah Hamren, BS; Robert Goodson, BS; Jessica LeClair, PhD; Joseph Massaro, PhD; Asya Lyass, PhD; Ralph B. D'Agostino Sr, PhD; Kelsey Tuminelli, MS; Mark W. Kieran , MD, PhD; Monica E. Kleinman, MD. Plasma Progerin in Patients with Hutchinson-Gilford Progeria Syndrome: Immunoassay Development and Clinical Evaluation. Article commentary: Eriksson, M., Haugaa, K. & Revêchon, G. Readily Available Tools to Detect Progerin and Cardiac Disease Progression in Hutchinson-Gilford Progeria Syndrome.

A much faster pathway to treatments and cure...

Biomarkers offer the ability to objectively measure whether a treatment has beneficial effects. Because progerin is the toxic protein that causes Progeria, it is the optimal biomarker candidate.

Until recently, Progeria researchers have been unable to readily gauge disease state, because they lacked the technical capacity to measure Progeria's toxic progerin protein in blood samples. It could take several years to determine if a drug was effective, by evaluating clinical tests such as heart function and bone health. Dr. Leslie Gordon's team not only developed a test to quantify progerin levels in blood samples, but went on to show that lower progerin can indicate longer lifespan. Researchers may now be able to **detect a drug's benefits as early as four months after starting treatment** and at multiple points along each clinical trial. In future trials, the biomarker test could be pivotal in determining whether to continue with drugs that appear to successfully combat Progeria, adjust a treatment dose to a child's needs, or stop a treatment that may not be beneficial to avoid unnecessary side effects. It will still be very important to measure longer-term clinical benefits to things like heart and bone health, but the progerin biomarker is a valuable partner to these tests, and a huge step forward in detecting early drug effects.

12-year-old Alessandro from Italy shows some love for PRF's exciting research updates!

The future of Progeria research is brighter with this discovery.

"Measuring this new progerin biomarker from a blood sample means we can move faster towards finding better treatments and the cure for children with Progeria. Future treatment strategies that we are developing for Progeria, including genetic therapies such as RNA therapeutics and DNA base editing, will all benefit greatly from this discovery." - Dr. Leslie Gordon

"One of the most remarkable stories ever shared on this podcast"
– Dr. Carolyn Lam, world renowned heart specialist and host of the podcast *Circulation on the Run*, on the journey that led to these exciting findings. **Hear the full interview** about the profound impact of this study directly from Dr. Gordon here: https://bit.ly/3PpunA5 (starting at 6:41).

Because the heart disease (atherosclerosis) that children with Progeria experience is the same as that of the general aging population, the biomarker test can also be used to investigate the role of progerin in heart disease and aging. These studies could determine whether progerin should be a target for aging and heart disease therapies, which has the potential to help millions of aging adults.

WHAT'S NEXT for treatments and cure?

Check out our two latest and greatest initiatives...

Thanks to YOU, PRF continues to make a *dramatic* impact on the length and quality of life for those with Progeria. The finding that those who've taken lonafarnib for 10+ years have an average 4.5-year, or 30%, increase in life expectancy is truly phenomenal, **but our work must continue - to the cure!**

PRF co-founder and Executive Director Audrey Gordon seals the deal in June with Dr. Bum-Joon Park of PRG S&T to move forward with pre-trial work.

Kickstarting a New Clinical Drug Trial: Preparations for Progerinin Trial Have Begun!

PRF, in collaboration with Korean-based study sponsor PRG Science & Technology (PRG S&T), is hoping to kick off a **brand-new clinical trial** soon with a drug called **Progerinin**. Laboratory evidence shows that this drug, when taken in combination with lonafarnib, **may be more effective than lonafarnib alone**.

PRF funded the laboratory work that led to the formation of PRG S&T and its development of Progerinin. Pre-trial work has begun at Boston Children's Hospital, in anticipation of bringing children from all over the world to enroll for this trial in the coming months. We're super excited about starting a new trial with such a promising drug, and look forward to sharing more details with you as they become available.

RNA Therapy: Drug Administration Feasibility Study Has Begun!

PRF has taken the first patient-involved steps toward a clinical trial in RNA therapy – SO exciting!

Background: In January 2021, we reported **breakthrough findings in RNA therapeutics**, wherein this therapy inhibited production of RNA coding for the Progeria disease-causing protein, progerin. The study,* led by Dr. Francis Collins, White House Science Advisor and former Director of the National Institutes of Health (NIH), revealed that Progeria mice treated with a drug named SRP-2001 reduced the harmful progerin mRNA and protein expression in blood vessels, as well as in other tissues. The blood vessels were stronger, and the mice showed an increased survival of over 60% compared to untreated mice. Thus the work with this promising therapy continued, and we are **taking the next step with a Feasibility Study** as follows:

Typically, RNA therapeutics are liquids that are injected intravenously (directly into the vein). However, those with Progeria would not be able to tolerate intravenous delivery of the required weekly dosage. Thus, PRF developed a **subcutaneous delivery system** whereby the liquid can be injected with a small needle under the skin. **A 6-month study is now underway at BCH to determine the feasibility of this delivery approach for those with Progeria.** The team is testing whether administration of a saline solution can comfortably be injected subcutaneously. If it is successful, we will be **one step closer to a clinical trial in RNA therapy!**

*Our work with genetic therapies is advancing full-speed ahead! **RNA therapy and DNA Gene Editing** studies have shown a vast improvement in Progeria mice lifespan. PRF continues to invest substantial funds into their development, with the hope that these research efforts will lead to clinical trials, and, ultimately, the cure.*

These cutting-edge therapies have huge potential!

Four-year-old Bastian from Mexico is a superhero who gives a thumbs-up to YOU, for making all of this progress possible!

*Erdos, M.R., Cabral, W.A., Tavarez, U.L. et al. A targeted antisense therapeutic approach for Hutchinson–Gilford progeria syndrome. *Nat Med* (2021).

PRF's Research Grants Program: Funding the Science for 24 Years!

Since our inception in 1999, PRF has provided $9.3 million to fund 85 grants to Progeria-related research projects performed at **55 institutions in 15 countries** – our reach is truly worldwide! The work produced by these scientists has led to breakthroughs that are translating into longer, healthier lives for children with Progeria, while also driving discovery in heart disease and aging.

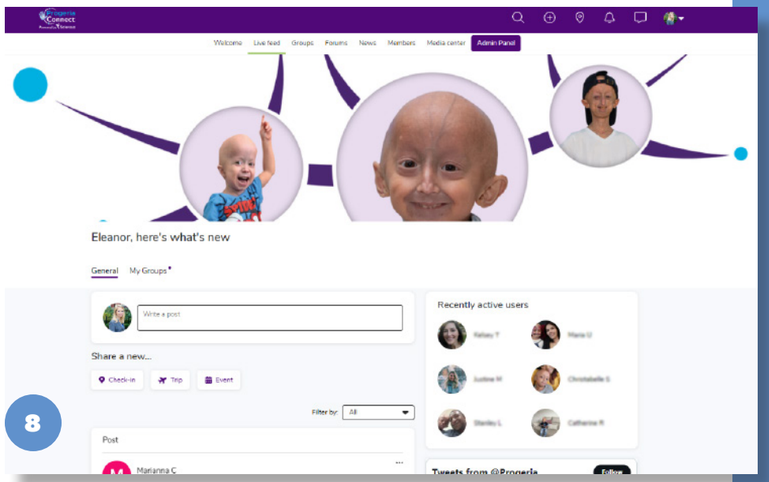
Our latest grant recipient is breaking new ground for the future of Progeria research:

Ricardo Villa-Bellosta, PhD

Center for Research in Molecular Medicine and Chronic Diseases (CiMUS) – Spain

"Progeria and Vascular Calcification: Diet and Treatments."

A key area of research in Dr. Villa-Bellosta's lab is the excessive calcification of the cardiovascular system, including the aorta, coronary artery, and aortic valves, which largely determines early mortality in children with Progeria. The molecular mechanism of the vascular calcification in Progeria has been previously analyzed in Progeria mice, which show a profound deficiency of extracellular pyrophosphate, a key inhibitor of calcification. Dr. Villa-Bellosta will aim to determine the molecular mechanisms that promote or reduce vascular calcification and lifespan in Progeria, focusing on the importance of specific nutrients that are consumed daily. He will also analyze the efficacy of two new potential therapeutic approaches which restore pyrophosphate to normal, and could improve the quality of life and longevity. He will use Progeria mice and and aorta vascular smooth muscle cells to analyze the effect of these nutrients/treatments on the vascular calcification and lifespan both alone and combined with lonafarnib.



Progeria Research Foundation

Progeria Connect

Powered by Sciensus

Announcing the launch of

Progeria Connect

This year, in partnership with British pharmaceutical company Sciensus, PRF launched ***Progeria Connect,*** **a private, global online platform** for those directly impacted by Progeria to easily and readily communicate with PRF and each other without language barriers. This new PRF program provides an opportunity for the children and young adults, as well as their parents and other close relatives, to **learn from each other and from PRF, share resources and experiences, and thrive as a community,** no matter their location or language spoken.

Progeria Connect benefits include:

- **Private groups to share ideas and experiences,** including Teens with Progeria, Young Adults 18+, and Parents of Children with Progeria;
- **Instant access to credible information** on patient care, clinical trials and the latest research findings;
- **Live Zoom video chat, with real-time text translations for multi-language calls;**
- **Dedicated mobile app** to engage with fellow *Progeria Connect* members while on-the-go!

Families interested in joining can register for free at *www.progeriaconnect.sciensus.com.*

PRF *On The Move!*

Brilliant Minds at Work

In June, PRF Ambassador Sammy Basso was invited to speak at the **Brilliant Minds – 'Future-Gazing'** event in Stockholm, Sweden, along with friend and colleague Dr. David Liu, Richard Merkin Professor and vice chair of faculty at the Broad Institute of MIT and Harvard, a pioneer in the field of genetic editing in rare disease. Brilliant Minds brings together world-class innovators across all walks of life in an exclusive annual showcase of breakthrough ideas that shape the future for mankind. It was no surprise that Sammy and Dr. Liu's talk on the power of base editing in Progeria research and its potential curative impact received a standing ovation!

Check it out at *https://bit.ly/3qxMrhF*.

PRF Medical Director Presents as Expert in Aging Research

PRF co-founder and Medical Director, Dr. Leslie Gordon, known as the world's expert on Progeria, is often asked to speak as a thought leader in various facets of rare disease research. In March, she was a panelist at the **Wall Street Journal Health Forum**, addressing an audience of senior healthcare business executives and medical practitioners. In a session entitled **"Breakthroughs in Aging Research,"** Dr. Gordon (pictured 2nd from the right) shared the latest findings in the science of aging, gleaned from decades of Progeria research.

PRF Hires New Chief Business Officer – Barbara Natke

As PRF's research activities continue to expand, our organizational structure must adapt to keep up with this growth. To that end, we recently welcomed Chief Business Officer Barbara Natke to our team. Barbara has a strong scientific background and over 20 years of biotech/pharma experience, including extensive knowledge of the drug development process. Our expanding team is truly on the move. Welcome, Barbara!

PRF Presents at International Meeting on Laminopathies in Madrid, Spain

In May, Dr. Leslie Gordon spoke as an expert in Progeroid Laminopathies at the 4th International Meeting on Laminopathies in Madrid, Spain. In a talk entitled **"Progeria and the Lonafarnib Journey to Approval — Lessons for Future Treatments and the Cure,"** Dr. Gordon shared insights on PRF's pathway from the early phases of drug exploration with lonafarnib, through FDA approval. Pictured here, PRF co-founder and Executive Director Audrey Gordon (left) and Dr. Gordon (right) huddle up at the conference with our European friends, 27-year-old Sammy Basso from Italy and 37-year-old Ivana from Serbia.

PRF Welcomes New Board Member Mark Kieran, MD, PhD

Dr. Mark Kieran recently joined our team of volunteer Board of Directors. During his 20 years as Director of Pediatric Neuro-Oncology at the Dana-Farber Cancer Institute and Boston Children's Hospital, Dr. Kieran served as the Principal Investigator on the first-ever lonafarnib clinical trials in Progeria from 2007 – 2017. He brings immense knowledge from decades of experience as a physician-scientist, as well as more recent industry involvement through his current position as VP of Clinical Development at Day One Biopharmaceutics, a company focused on the development of targeted drugs for children. We're thrilled to welcome him to the team!

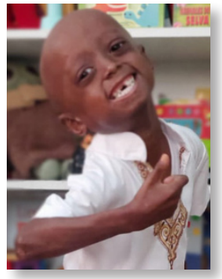
NORD Educational Series Bridges the Gap in Rare Disease Research

In May, Dr. Gordon participated in an educational video series produced by the National Organization for Rare Disorders (NORD), along with her long-time friend and colleague, Dr. Francis Collins. Addressing an audience of rare disease patient organization leaders, Drs. Gordon and Collins shared the story of their journey in lonafarnib drug development, from the founding of PRF, to the Progeria gene discovery, clinical drug trials and FDA approval of lonafarnib. We hope their story inspires innovations and partnerships in other areas of rare disease research!

Kids & Young Adults *on the Move!*

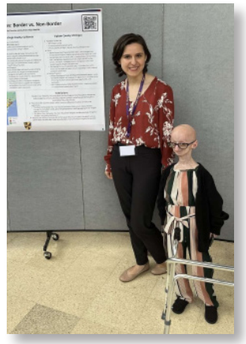
Here's a glimpse into the activities and personalities of those we strive to treat and cure. Your support is making their healthier, longer lives possible – THANK YOU.

This is why we do what we do. ♥



According to his mom, **5-year-old Kahlil** from Guyana is best known for his "ability to effortlessly make friends and infuse every room he enters with an infectious brightness." ***Thank you, Kahlil, for all the joy you bring to the world!***

Although a Jenga enthusiast at heart, **10-year-old Vyan** from India can solve a Rubik's Cube game in lightning speed! He's also an avid YouTube gamer, operating under the username "No Raze." ***Check him out!***



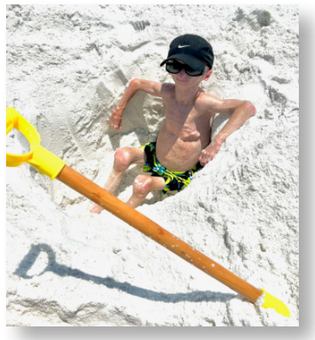
Lindsay is crushing college! At the **Challenging Borders International Human Rights Conference** at Albion College in April, the 19-year-old freshman from Michigan presented on the different policies in sanctuary counties in the U.S., interacting with people from 9 countries and 18 universities. **It was truly a cultural experience!**

Now entering his final year of primary school, **12-year-old Enzo** from Australia is pictured here with his dog, Jasper, whose bright, spunky and positive attitude has been Enzo's "perfect match." ***Woof!***

27-year-old Sammy from Italy was invited to the Italian Parliament, where the President of the Chamber dedicated the entire Christmas concert to Sammy and Progeria research. ***È fantastico!!***

4-year-old Bastian from Mexico graduated pre-school in the spring and is ready to take on the world! ***Sigue avanzando hacia tus sueños!*** (Keep moving towards your dream!)

Best friends and brothers from Pennsylvania, **Nathan** (age 18, on the right) is ready to crush his senior year in high school and will be busy college-planning this fall, while **Bennett** (age 14) looks forward to starting high school. ***Kudos to both of you for being such enthusiastic students!***



16-year-old Zach continues to thrive in high school, finishing his sophomore year with an impressive 4.0 GPA. Zach enjoys gaming, cooking and traveling with his family, having visited Cancun, Mexico this summer. ***He loves the sand between his toes!***

When she's not studying to be a paralegal, **20-year-old Kaylee** from Michigan enjoys hanging out with friends, driving her van and traveling. Her favorite trips so far have been Savannah, GA and Phoenix, AZ. ***Bon voyage, Kaylee!***

This past year has had **45-year-old Tiffany** from Ohio busy growing her business, Wanderlust Studio, making new connections, and being filled with reminders of gratitude. In her words, "I am excited about what's ahead and plan on creating & exploring life as much as I possibly can." ***Tiffany, you are an inspiration!***

In his father's words, **5-year-old Jedidiah** from Papua New Guinea "is very talented, funny and playful, and can remember everything! He loves drawing with pencils and chalk. He can run, jump and play, is an amazing dancer, and makes friends easily. We all love him dearly!" ♥

A highlight of the year for Belgium natives **Amber** (age 17) and her brother **Michiel** (age 25), was their trip to Lake Garda in Italy, where they ate delicious food and went on lots of adventures by motorbike and on foot. ***Happy trails to you two!***

Chapter, Family and Annual PRF Events

Special events connect our wonderful, diverse communities of supporters in active and fun ways, all while powering lifesaving research. To the organizers, participants, volunteers and sponsors - THANK YOU for uniting in our shared goal to cure Progeria. **For the children, for the cure.** ♥

Carlos Luiz Silva kicks back with his family after the race (left), while PRF Ambassador Merlin Waldron (right) has a far-out time chilling with supporters on race day.

PRF's 21st Annual International Race for Research Gets Groovy

Last September, PRF's annual road race rocked a far-out '60s vibe, complete with tie-dyed shirts, totally tubular tunes and a super rad morning of fun for all.

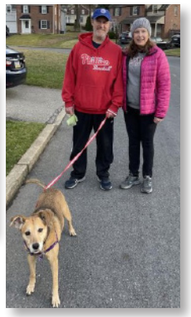
We were so jazzed to have nearly 300 runners and walkers both on our home turf in Peabody, MA, and virtually from neighborhoods across the US. *It was outta sight!*

Cycling for Science

Motorcycle enthusiasts hosted by the Sons of Solomon Motorcycle Group once again hit the pavement on July 29 for the **13th Annual Zach Attack Ride for Progeria** in Lexington, KY. Riders were treated to food, music, a silent auction, raffles and more, in honor of local Kentuckian Zach and others around the world with Progeria. We're so grateful for the steadfast support – *this crew ROCKS!*

Zach Attack's team of bikers gather around him in support of breakthrough research toward the cure!

30-Mile Movement Challenge Makes our Hearts Happy



Some of the 30-Mile Movement Challenge participants in action.

PRF celebrated February's National Heart Month with a 30-Mile Movement Challenge, encouraging participants to complete 30 miles in 30 days through any movement (walking, running, biking, etc.) while raising funds and awareness for PRF. Together, we brought in over $13,000 through online donations to participants' fundraising campaigns. *Way to move it!*

PRF's Falmouth Runners Hit the Streets for the Cure

TEAM PRF 2023 gears up for the big race ahead!

In August, 26 TEAM PRF members ran the challenging roads of Falmouth, MA, in the annual **7-mile Falmouth Road Race**. TEAM PRF also surpassed their team fundraising goal, raising over $30,000! **Great job, runners, on and off the course!**

An Un-fore-gettable Golf Tournament in NJ

Last September, golf enthusiasts came together in support of Zoey and PRF's New Jersey Chapter for **Team Zoey's 13th Annual Golf Outing,** at Preakness Hills Country Club.

Happy 13th birthday, Zoey!

It was truly a double celebration! Zoey's amazing golfing community also celebrated her entry into the teenage years. TEAM ZOEY supporters are a close-knit, wonderful group that has come together since Zoey was 1 year old – WOW!

Playing FORE the Cure!

PRF's 4th **Cure Cup Classic Golf Tournament** brought dozens of golf-loving PRF supporters out on a beautiful, sunny day at Ipswich Country Club.

Foursome Kevin Clarke, David Hoar, Jodi Mitchell and Cindy Cole had a blast on the course. Jodi had an especially victorious day, winning the women's longest drive!

PRF's Boston Marathon Runners Cross the Finish Line Towards the CURE!

In April, Bobby Nadeau (left), and Paul Michienzie (right) totally crushed it in the intense 26.2-mile course from Hopkinton to Boston, MA, going the distance to raise over $23,000 – AMAZING!

Miracle Makers

PRF's Miracle Makers — those who go above and beyond in support of our mission to cure Progeria - are a critical part of PRF's progress. They give their time, talent, or treasure to help PRF in unique ways, often making lifelong impacts on the children and families they support. Enjoy getting to know a few of these heroes!

Pediatrician on a mission

Dr. Mayank Nilay, a pediatrician and clinical geneticist in Noida, India, is passionate about forging collaborations that help rare disease patients. He works closely with four children with Progeria in India, and has been instrumental in connecting them with the lonafarnib Managed Access Program (MAP) in order to maintain a steady supply of this life-extending drug. He's also connected PRF with several new Indian families, enabling their children's access into PRF's vital programs and services.

Teachers are superheroes!

ESL teacher Hannah Weigert of Everett Public Schools in Massachusetts has supported 13-year-old Carlos since he moved to the U.S. from Brazil in 2019. Not only has she seen him make great strides in his public speaking (check out his Instagram @carloseduardoluizsilva to see him in action!) but she's been a steadfast PRF supporter, organizing 'Hats ON' days and other fundraisers at the school. Ms. Weigert also runs with her family in PRF's annual road race as part of Team Amigos de Carlos. Hats off to you, Hannah!

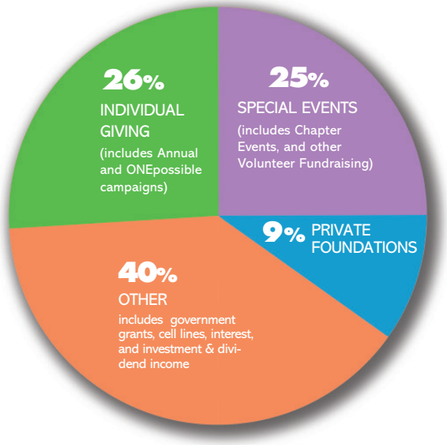
Pictured here, Juan snuggles with her daughter, Yan Huangxi, who also has a rare disease, Mitochondrial DNA depletion syndrome.

Chinese Social Media with a Smile

Since 2019, Juan Huang from the Chinese Organization for Rare Disorders (CORD) has provided fantastic social media support for PRF from her office in Beijing, China. PRF's WeChat and Weibo social media accounts are our primary means for raising awareness in China, where we believe there may be 50-60 children with Progeria still undiagnosed and untreated. Juan's 'insider knowledge' and resourcefulness has been paramount to PRF's awareness efforts across China, as she facilitates video production, assists with family translations, and shares cultural insights necessary to help PRF optimize our search for children.

PRF 2022 Annual Report

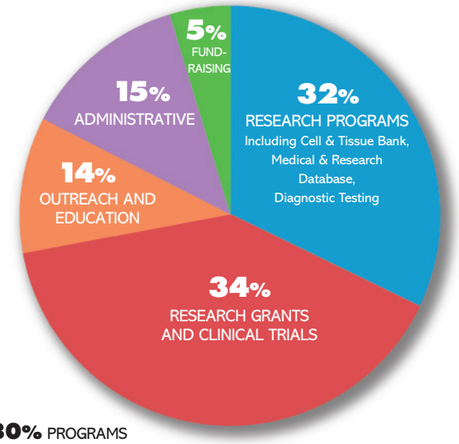
INCOME ANALYSIS



TOTAL OPERATIONAL REVENUE*
$3,187,832

TOTAL EXPENSES
$2,911,725

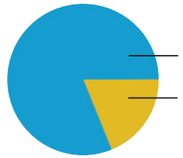
EXPENSE ANALYSIS



*Above chart excludes net investment losses of $5,773,600

Net Assets = $46,824,384, mainly attributable to proceeds from the 2021 sale of the Priority Reveiw Voucher (PRV) issued after FDA approval of lonafarnib as a treatment for Progeria. PRF's 10-year average net assets prior to this historic payment = $4.63 million.

PROGRAM RATIO

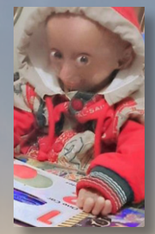


Core PRF Activities That Support Our Mission

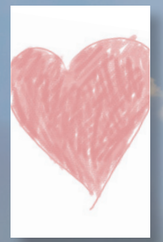
2022 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY (1999 - 2022)
23 children from 9 countries registered	International Patient Registry (includes unconfirmed cases)	371 children with Progeria from 70 countries and all continents have registered with PRF - amazing statistics for an ultra-rare disease!
100 cell lines sent to 21 labs in 12 countries; 7 tests completed	Cell & Tissue Bank and Diagnostic Testing Program	1,460 cell lines sent to 226 teams in 28 countries; 166 children tested through PRF
8 children enrolled; treatment guidelines provided to all	Medical & Research Database	207 enrolled from 53 countries and 1 U.S. territory; treatment guidelines sent to 859 families, physicians and other caretakers
4 new children from 2 new countries enrolled in Everolimus & Lonafarnib Trial	Clinical Trial Funding & Co-coordination	104 children and young adults from 42 countries have participated in PRF clinical trials, initiated in 2007
9 research grants ongoing (extensions granted due to COVID-19); includes one new research grant awarded	Research Grant Funding	84 grants in 14 countries; funding total: $9.1m
124 registrants from 14 countries attended PRF's 2022 workshop	International Scientific Meetings	16 meetings: 11 general and 5 subspecialty workshops
12 events, 55 new volunteers (including 11 additions to PRF's translator program!)	Volunteer-Led Events and Other Activities	Over 5,000 volunteers worldwide have helped raise awareness and funds in furtherance of PRF's mission.
5 children visited the U.S. and required an interpreter	Translations Program	PRF's program and medical care materials are translated into 39 languages
28 children identified; 1 country added – Papua New Guinea	Public Awareness	212 children and young adults living with Progeria and Progeroid Laminopathies from 51 countries, speaking 31 languages

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

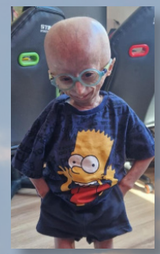
In Loving Memory..!



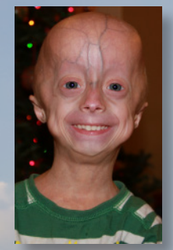
Ahmad
6 years old
From Pakistan



Elijah
9 years old
From the United States



Adrian
8 years old
From Spain



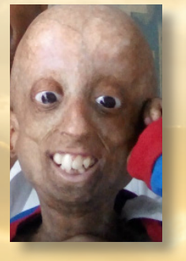
Cameron
16 years old
From the United States

Evra
17 years old
From Turkey

Sakshi
12 years old
From India

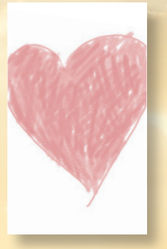
Maryam
13 years old
From Pakistan

Talha
18 years old
From Pakistan



Jomar
13 years old
From Dominican Republic

Andrea
7 years old
From the United States



SiXuan
8 years old
From China

Jonathan
33 years old
From the United States

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

Progeria Research Foundation
FOR THE CHILDREN ♥ FOR THE CURE

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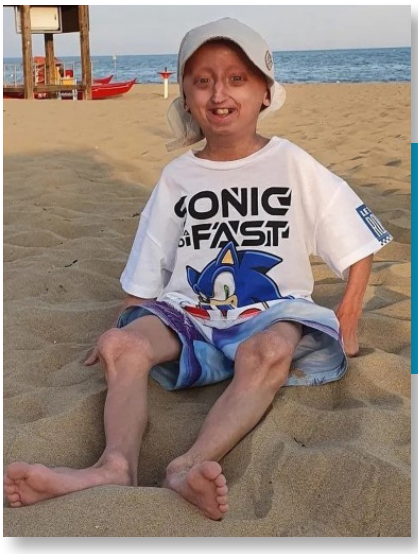
info@progeriaresearch.org or call **978-535-2594**



10-year-old Mischa from Germany is soaking up the last of the summer sun's rays before a new school year!

Love PRF's annual newsletter but want to help save trees? Subscribe on our home page at progeriaresearch.org to get next year's copy emailed!

SAVE THE DATE!

Night of Wonder Gala

Saturday, April 20, 2024
Renaissance Boston Waterfront Hotel

BE A GAME-CHANGER for children & young adults with Progeria at PRF's signature ***Night of Wonder Gala.***

Roll the dice all evening, enjoying fabulous **food**, marvelous **music**, spectacular **speeches**, and the most amazing **live auction** you'll ever experience.

Don't miss your turn – come PLAY to WIN with PRF!
Visit *prfnow.org* for details.

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