Progeria Research Foundation
FOR THE CHILDREN ♥ FOR THE CURE

WWW.PROGERIARESEARCH.ORG • SEPTEMBER 2025

news

Collaboration = Progress

The Progerinin trial is a partnership between PRF, trial sponsor PRG Science & Technology (a Korean- based biotech company), Boston Children's Hospital (the trial site), Brigham and Women's Hospital (site for some trial tests), and of course the families who are with us every step of the way!

Progerinin Clinical Trial Running Full Speed Ahead!

Last fall, we announced that PRF was partnering with PRG S&T (Sponsor) and Boston Children's Hospital to launch a new Progeria clinical trial to test a drug called Progerinin. We are thrilled to announce that the Progerinin Clinical Trial was launched in 2025 and is fully enrolled and running full speed ahead – what an amazing accomplishment for an ultra-rare disease! Progerinin was created in the laboratory of PRF-funded researchers, so it has not been given to children before. This first-ever Progerinin trial is looking at two different doses, their side effects and whether either dose shows initial signs of benefit. It's a wonderfully busy time for the trial team!

See page 3 for details on next steps and how we will determine whether Progerinin, in combination with the FDA-approved lonafarnib (brand name Zokinvy) treatment, will be even more beneficial than lonafarnib alone.

We also have lots to share about PRF's gene therapy development…**Our mission to treat and cure Progeria continues in full force!**

In January, 21-year-old Kaylee and 23-year-old Merlin became the first to enroll in the Progerinin clinical trial. They celebrated by taking their first doses in shot glasses featuring Boston's Bruins and Patriots sports teams!

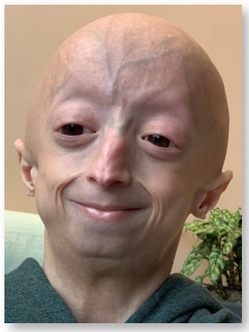
Progeria is a rare, fatal, "rapid-aging" condition.

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:

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There are SO many involved with PRF, we can't fit them all! For more details on our amazing volunteers, please visit ***https://www.progeriaresearch.org/our-people/***

A Message from the President and Executive Director

Hello Everyone,

PRF's on-line Charity Navigator report* states the following:

Charity Navigator

Rating Information

Great

This charity's score is 100%, earning it a Four-Star rating. If this organization aligns with your **passions** and **values**, you can give with **confidence**.

What an awesome reflection of what YOU & PRF – staff, board, researchers & research-related program partners, donors, volunteers, the families and other supporters – make possible every day!

Here's what this perfect score says about all of us:

- **100%:** Everyone connected to PRF is fully committed to one goal - finding treatments and the cure for Progeria. That singular focus drives everything we do.
- **Passion:** We all share an intense enthusiasm for PRF's mission. The love and energy are palpable in every aspect of our activities, as seen in the pages that follow.
- **Values:** PRF operates in a fast-paced, ever-evolving environment grounded in innovation, collaboration, and integrity. Transparency is also core to our approach, along with deep respect for the privacy of donors and the families we serve.
- **Confidence:** This consistent 4-star rating reflects more than just wise management – it's a testament to PRF's extraordinary record of success, and the strength of its mission and the people who drive it.

Every day, I'm reminded how lucky I am to serve as PRF's Executive Director – because ***every day,*** I witness the commitment, passion, and values that fuel our shared confidence that **together, we *WILL* find the cure!**

Thank you all for your incredible support.

Audrey Gordon, Esq.
President and Executive Director

**https://www.charitynavigator.org/ein/043460220*

Research Updates

Progerinin Clinical Trial is Well Underway!

We're thrilled to report that the first Progerinin clinical trial stage – determining the side effects and optimal dose of Progerinin – is running smoothly. The Progerinin clinical trial includes ten children and young adults with Progeria, from seven countries. Eight are taking Progerinin in combination with lonafarnib, and two are receiving only lonafarnib.

Patients travel to Boston for testing and drug supply every 4 months. Those taking Progerinin have completed their enrollment visit, which is when they started a low dose of Progerinin. They have all returned after 4 months for testing and transition to a higher dose of drug. They are now beginning to return for their 8-month trial visits to Boston Children's Hospital (BCH).

Progerinin trial participants Bastian, 6 years old from Mexico, and Juan Carlos, 10 years old from Colombia, give their new Boston Red Sox hats the thumbs up during their 2nd trial visit in May.

We estimate that within the next year, we and our partners, Sponsor PRG S&T and BCH, will evaluate whether Progerinin shows promise as a possible new treatment for Progeria, mainly by looking for a decrease in progerin levels in the blood. If Progerinin is well-tolerated and shows initial signs that it may improve disease, we anticipate moving into a larger and more extensive trial to determine effectiveness in treating Progeria – stay tuned!

Progerinin has been shown to increase lifespan in a Progeria mouse model by 50%, compared to lonafarnib mouse studies that showed a 25% increase. Thus Progerinin – a small molecule drug that targets the toxic, disease-causing progerin protein in a different way than lonafarnib – shows promise as a possible new treatment for Progeria in combination with lonafarnib. Our pursuit of better treatments and the cure continues...!

Gene Therapy: PRF's Path to CURE PROGERIA is Big News!

Path to CURE PROGERIA

After 6 years of intense research, PRF and its partners have created a drug designed to permanently correct the Progeria gene mutation at the DNA level. While lonafarnib (and possibly Progerinin) are designed to attack the disease-causing protein progerin after it is made, PRFs gene editing therapy is designed to prevent progerin from ever being made by the cell.

THE NEW YORKER

On August 11th, ***The New Yorker*** featured an in-depth article detailing PRF's history, accomplishments, and present focus on the Gene Editing Therapy that could bring us closer to curing Progeria. Our extraordinary journey is reaching millions more throughout the world!

To read the full article, go to ***https://www.newyorker.com/magazine/2025/08/18/how-an-ultra-rare-disease-accelerates-aging***

Why Progeria Base Editing Gene Therapy Could Work:
Base editing gene therapy uses CRISPR technology to target and permanently correct specific DNA base (or DNA letter). **Because Progeria is caused by a change to a single letter in the DNA, it is perfectly suited for this type of gene therapy.** A Progeria base editor increased the lifespan of a Progeria mouse model by 140% – the mice were approaching old age!

PRF is now working on drug manufacturing, US FDA interactions, and clinical trial development to bring this promising technology to children and young adults with Progeria. This transformative program is a multi-stage, technically complicated, expensive, and time-consuming process – **and PRF is up to the challenge!**

Moreover, the success of this project could have widespread implications:

"The hope of our team is to use base editing to fix the root cause of Progeria, providing a pathway to a potential cure for children with progeria, as well as advancing treatments for millions of other children with fatal genetic diseases."

– **David Liu, PhD**, Broad Institute of MIT and Harvard University, Progeria Gene Team member, and developer of Base Editing Gene Therapy Technology.

In addition to curing Progeria, we are forging a path for others to take – how incredibly exciting!

Gene therapy is the most promising and the most expensive type of drug to manufacture. The unwavering commitment of PRF's passionate supporters like you will make Progeria gene editing a reality. **Go to *www.progeriaresearch.org* to find out more about PRF's Path to CURE PROGERIA!**

PRF On The Move!

PRF continues to lead the way in rare disease research, with our representatives participating in prestigious international conferences. Our groundbreaking work is being recognized around the globe, helping to raise awareness of Progeria and spotlighting PRF's critical mission. Here's a look at where we've been this past year:

In September 2024, **PRF Medical Director Dr. Leslie Gordon**, **PRF Executive Director Audrey Gordon**, and **PRF Ambassador Sammy Basso** presented to families and researchers in **Shanghai** at the **China Rare Disease Summit**, a prestigious event bringing together over 1,000 participants and hosted by the Chinese Organization for Rare Diseases (CORD). It was incredibly special to connect in person with more than a dozen families, and we thank CORD CEO Kevin Huang and his team for making that possible. Leslie and Audrey will also forever treasure the time spent with Sammy and his parents, just a month before Sammy's passing.

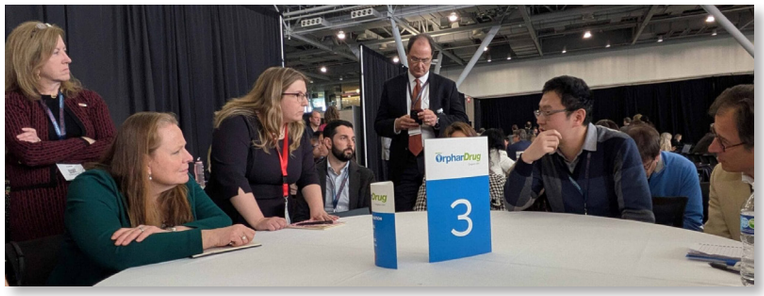
On Rare Disease Day 2025, Dr. Gordon and **PRF Board Chair Scott Berns** were keynote speakers at **The Center for Research in Molecular Medicine and Chronic Diseases (CiMUS)** at the University of Santiago in **Spain**. Addressing research advancements and the hope they bring to affected children and their families, Drs. Gordon and Berns were touted as "two leading figures in the fight against Progeria", and co-founders of "a pioneering organization in the development of progeria treatments on a global scale."

Drs. Gordon and Berns with event organizers and fellow speaker Esther Martínez Gracia, mother of Alexandra and co-founder of the Asociación Progeria in Spain, who supports families and raises awareness in Europe.

In May, Dr. Gordon was back in Europe presenting *"Current major development efforts towards the cure for Progeria"* at the **5th International Meeting on Laminopathies** in **France**. This prestigious meeting brings together leading international experts in nuclear envelope proteins and related diseases, including accelerated aging syndromes like Progeria.

PRF's Chief Business Officer Barbara Natke has been connecting with dozens of business and research leaders at major US conferences. These efforts are essential for building strong partnerships and staying current with the latest research and industry developments – both crucial for accelerating our progress.

At the **BIO International Convention**, Barbara engaged with over 50 leaders from biotech and pharmaceutical companies, nonprofit and patient advocacy organizations, and scientific service providers – WOW!



At the **American Society of Gene and Cell Therapy** meeting, Barbara attended ground-breaking sessions and met with leaders in the field of Gene Therapy. And at this year's **World Orphan Drug Congress**, Barbara (2nd from left) participated in a roundtable discussion on Gene Therapy for Rare Diseases. Insights gained and connections made at these events will be critical for driving PRF's Gene Editing program to the clinical trial stage.

FUNdraising Events - Fun Times, Fabulous People!

Over the past year, PRF was involved in three amazing races that brought together our extraordinary community of athletes, donors, and volunteers. **Together, we're *racing* toward the cure!**

Team PRF Falmouth runners are ready to run!

On a picture-perfect summer day on Cape Cod in August 2024, eighteen **TEAM PRF runners** took on the iconic 7-mile **Falmouth Road Race**. Dressed in **bold highlighter green** tank tops, they stood out in a sea of 11,000 participants and raised nearly $20,000 for Progeria research – ***SO Awesome!***

PRF Ambassador Carlos inspires the crowd!

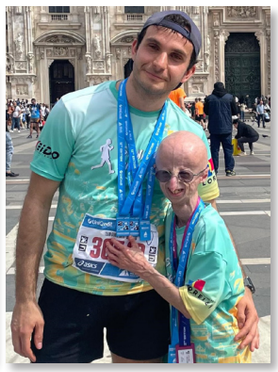
…the following month, over 300 runners and walkers gathered in Peabody, MA for **PRF's 23rd Annual International Race for Research.** Centered around the theme of **love** – a key ingredient needed to treat and cure Progeria (along with research and $$$!) – this heartfelt event was filled with energy and determination to cross the finish line in celebration of PRF's tremendous progress – ***How Wonderful!!***

Canadian Kindness at its Finest Each year, the **École primaire de l'Escalade school in Quebec** chooses a cause that's meaningful to the students, to benefit from their annual event, **Escacourse**. This year, in honor of Nevena and her family, students supported PRF. Nevena and her brother Lazar (pictured) carried the event medal and were proud to be the top fundraisers. ***How inspiring – cheers to our wonderful northern friends!***

Lazar and Nevena are all smiles after Escacourse's huge success!

Chip Foose, RealTruck, and Barrett-Jackson Keep on Truckin' for PRF Long-time PRF supporter and custom car designer extraordinaire Chip Foose partnered with RealTruck to create a custom Ford F-150 that was auctioned off for $150,000 at Barrett-Jackson's 2025 event in Arizona to benefit PRF – WOW! A heartfelt thank you to Chip for dedicating his beautiful craftsmanship, to RealTruck for sponsoring the rebuild, to Barrett-Jackson for hosting the auction, and to ***highest bidder John Burkland for your incredible generosity!***

On April 21, 2025, TEAM PRF was honored to be part of the **Bank of America Boston Marathon Charity Program** with 9 incredible runners from Italy, Israel, and 4 US states who trained rigorously for months, all while raising $115,000 for PRF – ***Astounding!!!***

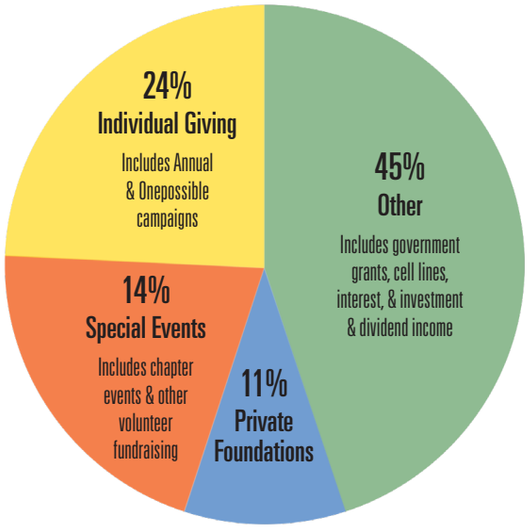


Among our runners were Tappa (left) and Tony (right) from Italy, who ran in honor of their dear friend Sammy Basso ♥

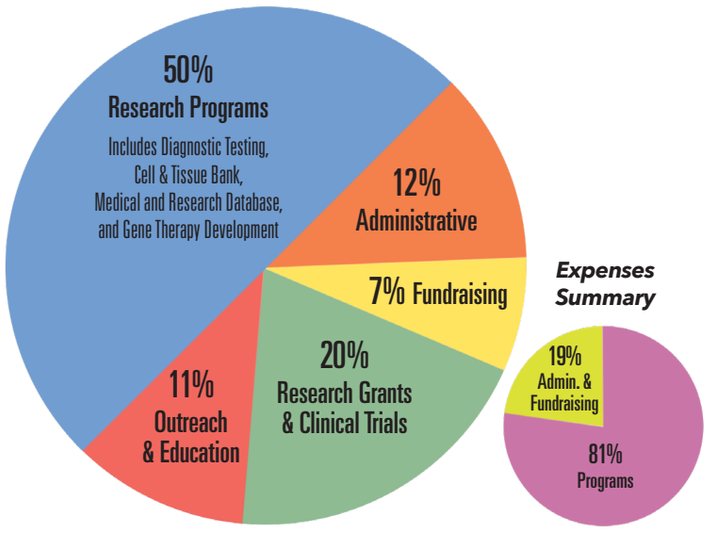
Marathon runners gathered the day before the race, with PRF co-founders Audrey, Scott and Leslie joining to give the "thumbs up"!

PRF 2024 Annual Report

Total Revenue $3,780,945



Total Expenses $5,259,932



Net Assets = $47,400,671, mainly attributable to proceeds from the 2021 sale of the Priority Reveiw Voucher (PRV) issued after the historic FDA approval of lonafarnib as a treatment for Progeria. PRF's 10-year average net assets prior to this historic payment = $4.63 million.

***Does not include in-kind services.** In 2024, PRF received approximately $567,000 in pro bono legal, business and research consultant, and other professional services – thank you to everyone who donated their time and talents! If factored in, PRF's program ratio would exceed 83%.

Thanks to your support, progress in 2024 brought us ever closer to better treatments and the CURE!

Here are some 2024 highlights…

18 children and young adults from **11** countries travelled to Boston for PRF-funded clinical trial that provides the only way they can get the life-extending treatment lonafarnib.

26 new children from **15** countries joined PRF's Patient Registry a *20% increase* over 2023 and one of our highest years ever! PRF can now provide them with important information and services needed to live longer, healthier lives.

98 PRF Cell Bank samples — a **32%** increase over the prior year – were sent to 24 researchers in 7 countries. These are the vital biological tools they need for lifesaving research.

2 programs advanced at a remarkable rate: RNA and DNA Gene Therapy development. These cutting-edge methods could bring us closer to curing Progeria

1 new clinical trial launched with the drug *Progerinin* Trials are essential to discovering more treatments and the cure.

PRF's other programs include **Research Grant Funding**, **Medical & Research Database**, **International Scientific Workshops**, and **Public Awareness**. For more information on these programs, go to *progeriaresearch.org/prf-by-the-numbers/*.

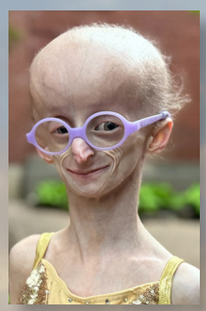
Gone from our sight, but never our memories~
Gone from our touch, but never our hearts.

In Loving Memory...

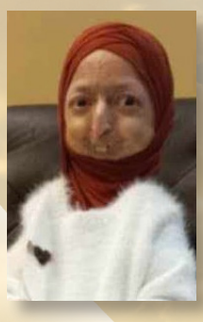
Sammy
28 years old
From Italy

Rafiq
9 years old
From Suriname

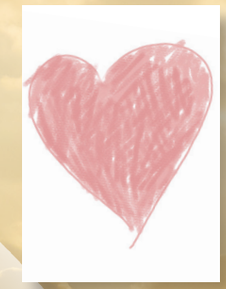
Beandri
19 years old
From South
Africa



Carly
14 years old
From USA



Hamida
41 years old
From Algeria



Amira
11 years old
From Libya

Nisha
19 years old
From Nepal

Mateo
22 years old
From Argentina

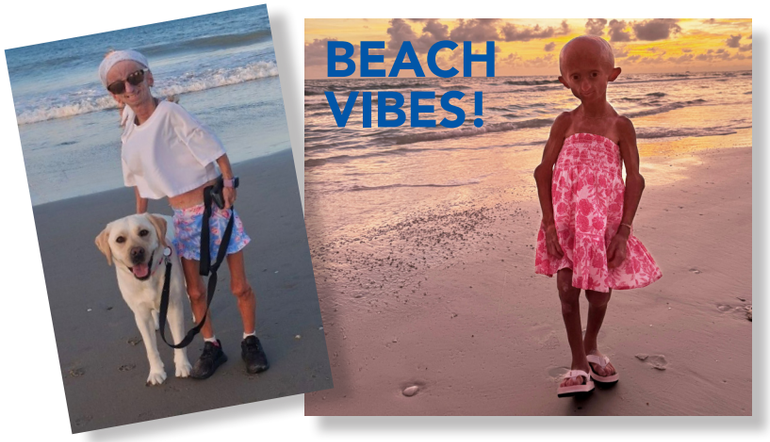
Forever part of our community and our purpose,
their light will continue to shine in our hearts and minds forever.

Progeria Research Foundation
FOR THE CHILDREN ♥ FOR THE CURE

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**



Fashion and favorite place come together this summer, with Zoey in sunset-blending pink, and Kaylee (with Iris ♥) matching the water's whites and blues.

LAST CHANCE TO REGISTER!

THE PROGERIA RESEARCH FOUNDATION 12TH INTERNATIONAL SCIENTIFIC WORKSHOP

COMING of AGE

October 29–31, 2025
Boston Marriott Cambridge
Cambridge, MA

PRF's scientific conference is just a few weeks away. RESEARCHERS, be part of our journey to the cure for Progeria! Join Progeria and aging researchers from around the globe for an unforgettable 2 ½ days of the latest on new drug development, cutting-edge basic science advances and more. NON-RESEARCHER SUPPORTERS, come experience the opening evening's moving presentations by young adults with Progeria.

To register and for more details, visit prfworkshop.org

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youtube.com/user/progeria123