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Scientific Workshop SAVE THE DATE	BACK COVER

PRF's 2-drug Clinical Trial Visits Complete; Monotherapy Trial continues!

In April 2021, after more than a year of COVID-related travel restrictions, PRF welcomed back children and young adults from around the world to Boston for their final, 2-drug clinical trial visits. This trial will determine whether a new drug (everolimus), in conjunction with lonafarnib (also known as Zokinvy—the current standard of care for Progeria) is more effective than lonafarnib alone. **Sixty children from 27 countries participated—amazing!** The trial team is now hard at work analyzing data, and plans to publish their findings in a peer-reviewed, scientific journal.

Because access to the life-extending drug lonafarnib is vital to those with Progeria, PRF has extended the trial, enabling 2-drug trial participants, and others who came to PRF after enrollment ended, to roll over into a monotherapy (1-drug) trial extension. This is especially important for children who cannot get the drug locally.

2-drug trial participants, siblings Jeshiah and Jeanezharette from the Philippines, completed their final visit in late 2021.

See page 8 for more details.



We did it yet again!

PRF joins just 5% of reporting charities in accomplishing this highest distinction for **9 straight years**.

A Message from the Executive Director

Hello Friends,

As the world adjusts to post-pandemic life, including the easing of travel and meeting restrictions, PRF is taking full advantage in every way possible, including:



Children and young adults who cannot obtain the treatment lonafarnib in their home countries are traveling to Boston, to participate in the clinical trial that enables them to receive this life-extending drug;



Our superhero supporters gathered in Boston for the first time in four years for our signature Night of Wonder gala, generating more excitement and funds than ever before; and



Researchers from all over the world will soon be in Boston for the first in-person scientific workshop since 2018.

As demonstrated in the pages that follow, PRF is teeming with activity in our core areas of Progeria research and global awareness, enabled by your extraordinary support through fundraising events and campaigns. Staff, Board of Directors, donors, runners, volunteers, translators, our social media community, and more all contribute to the cause — **it is truly a TEAM PRF effort!**

Why are we all so passionate about PRF's mission? Well, I think pages 6-7 say it all. Just look at what these extraordinary children and young adults are accomplishing and enjoying! PRF's mission is to enable them to realize their dreams and enjoy long, healthy lives. Thank you for being with us as we work to make these dreams, and a future cure, possible.

Together, we *WILL* find the cure!

Audrey Gordon, Esq.
President and Executive Director

7 AM on August 21, 2022: getting ready for the Falmouth Road Race with fellow TEAM PRF runners.



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.

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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.

Night of Wonder 2022: A SUPER Success!



PRF's signature gala and auction was a MARVEL-ous opportunity to come together for our first in-person

Night of Wonder in four years, bringing in an astounding \$580,000! This year's theme, **Calling All PRF Superheroes**,

celebrated 350 do-gooders who showed up to POWER PRF to the next level, toward the cure for Progeria.

PRF Ambassador, 21-year-old Meg Waldron (pictured above with gala attendee Rich Reed) gave a rousing speech about being one of the first lonafarnib clinical trial participants in 2007, and how that drug has helped Meg live an active life. With humor yet persistence, Meg also encouraged the crowd to bid high, and bid often!

Funds raised at Night of Wonder 2022 will go directly to PRF's worldwide research initiatives, where the ideas that will lead us to the cure are born. Thank you to all of our DEFENDERS: sponsors, auction donors, advertisers, volunteers and guests—you are our TRUE SUPERHEROES!

This FANTASTIC event is held every two years, so we hope to see you at Night of Wonder 2024!



A special thank you to our major superhero sponsors:

The Morrison Family
Cathy and John McCarthy
Debbie Mendelson Ponn



Amy Award winners Tom and Robyn Milbury were honored for their many and long-time contributions to PRF.



Gala event committee members Jodi Mitchell, Martha Holden and Kim Paratore, with Chuck Holden, celebrating the culmination of all their hard work.



Science and Medicine (SAM) Award winner Tom Mathers (far right) with his family and Wonder Woman.



Sponsor and gala committee member Debbie Mendelson Ponn (pictured above on the upper right), with friends.

PRF On The Move!

Honoring the special friendship between Dave Matthews and "Kit Kat Sam"

In January, Sam Berns' dad Dr. Scott Berns, PRF Co-Founder and Board Chair, recorded a wonderful tribute to Sam, which aired for two weeks on SiriusXM's Dave Matthews Band (DMB) Radio. Dave has supported PRF for many years and shared a close bond with Sam, including an appearance in the award-winning HBO documentary, *Life According to Sam* where Dave dedicates the song "Kit Kat Jam" to Sam at a concert.



Sam and Dave enjoy some time together back stage in 2013

Sam developed an indelible love of DMB with his dad. The two attended many concerts, exchanged play lists, and built endless memories around this love, which shines through in Berns' heartwarming tribute radio show.

Fan feedback...

"What a great show! Sam's set list was amazing, along with your memories at concerts all over the country. Listened twice with smiles and tears remembering Sam. It's wonderful to see (and hear) your family's story again, knowing that you are giving more and more families hope and healing around the world in yet another wonderful tribute to Sam in such a cool way."

Exciting Updates in Progeria Research Delivered by Dr. Francis Collins

Dr. Francis Collins, Acting Science Advisor to the President and former Director of the National Institutes of Health, spoke to an audience of researchers in May 2022, at The Frank and Joan Rothman Lecture at Brown University.



After being introduced by longtime colleague and friend, PRF Co-Founder and Medical Director Dr. Leslie Gordon, Dr. Collins shared the latest breakthroughs in genetic editing and drug development in a panel entitled, **"Genomic Therapeutics: Seeking a Cure for Premature Aging."** He mapped out the most transformational pathways being explored in Progeria research toward additional treatments and the cure, some with astounding survival benefits that seemed nearly impossible a decade ago. These pathways have helped to position

Progeria as a pioneer in the cutting-edge field of genetic editing and rare disease drug development, backed by mouse studies that indicate potential for much wider application in other areas. It was no surprise his talk was met with a standing ovation!

PRF Medical Director: Thought Leader in Rare Disease Drug Development at the STAT News Conference



In May, Dr. Leslie Gordon was invited to speak in Boston to an audience of biotech and pharma executives on the challenges and triumphs of drug development. Sharing the floor with pharma executives who've led pivotal trials through development for the

COVID-19 vaccine and other conditions, Dr. Gordon told the story of lonafarnib's path to FDA approval in a panel entitled, **"Battle Scars: The Journey from Lab to Patients' Bedside."** The conference was hosted by leading healthcare publication and media group STAT News.

Con-grad-ulations, graduates!

19-year-old Kaylee from Ohio (right) graduated high school this year with a 4.2 GPA! She plans to start college in the spring and will study human resources.

18-year-old Lindsay from Michigan (left) is off to college this fall, pictured here with her acceptance letter. She graduated in the top ten of her high school class and delivered a commencement speech!

You go, girls!



Game on!

15-year-old Zach from Kentucky is happiest when he's gaming. He likes to play Minecraft and Fall Guys with his friends, but his absolute favorite game is Mario Odyssey. He loves to speed run through the game and beat his personal best times!



Fun on four wheels 7-year-old Adrian from Spain loves all kinds of sports and driving his go-kart. His mother tells us that he lives his life by three key tenets:

- * Enjoy every day.
- * There are no limits to what you can like.
- * The only goal in life is to be happy.

Thanks for the positive words, Adrian!



Getting snuggly with furry friends

6-year-old Alexandra from Spain (left) never stops

playing with her new pet hamster, "Princess Jasmine," named after the iconic character from Disney's Aladdin. To see Alexandra and Princess Jasmine in action, go to <https://bit.ly/3dNLL0P>.

7-year-old Juan Carlos from Colombia (right) enjoys playing with his adorable dog, Dulce (which means "Sweet" in Spanish)!



PRF Kids Young On



Surf's up! 12-year-old Carlos from Massachusetts learned how to swim this summer! His favorite pool activities are being tossed by his dad from inside the pool and jumping off the diving board.

Stay cool, Carlos!



A star is born 11-year-old Enzo from Australia co-starred in an uplifting documentary called 'Embrace Kids,' about empowering young people to embrace their bodies and themselves in order to reach their full potential. In the film, Enzo says with a warm smile, "it doesn't matter if you are big or you're small, I just like being different and I never want to change." **What a great attitude!**

Photo credit: Sam Oster

Man about town 26-year-old Sammy from Italy is everywhere! He played a pivotal role in passing a new national law to make Italy one of the most cardio-protected countries in the world, creating an impactful video and gathering 40,000 signatures. The law will place defibrillators in more public places, improve defibrillator training, and encourage the development of apps that indicate defibrillator locations in emergency situations.

He also runs his own radio show, where he comments on "deep and serious arguments with a slice of craziness!"



To top off the summer, Sammy was brought on stage by Italian musical sensation Jovanotti before a cheering crowd of 50,000 fans. The singer told the crowd that Sammy is a scientist who has done extensive research in Progeria, and that there has been very exciting progress. To see this thrilling moment in action and hear Sammy's inspiring words, check out <https://bit.ly/3Clo768>.



Astemir is looking handsome in a bow tie for his Kindergarten graduation in May.

and Adults The Move!

New best buddies

6-year-old Astemir (left) enjoyed meeting 8-year-old Artem, both from Russia. Their doctor in Moscow had scheduled the families' pickup of Ionafarnib to coincide so they could meet at the hospital—how thoughtful!



Clinical Trial Updates

continued from front cover

Ensuring continued lonafarnib access to all...



Typically, outside of a trial, a drug is only attainable by prescription or through a Managed Access Program (MAP), which enables patients to get the treatment through their local physician. Now that the 2-drug trial visits have ended, and new children have been joining the PRF community after enrollment ended, we needed a way to get lonafarnib to those who cannot access it through those other means. Thus, PRF and partner Boston Children's Hospital have been supporting a "monotherapy" trial for lonafarnib only, so that group of children and young adults with Progeria can get this life-extending, heart-strengthening drug, and PRF can continue to collect important data on lonafarnib's effects. It is a novel approach, consistent with how we roll: *Where there's a will, there's a way!*

These innovative solutions to sustaining continuous global lonafarnib access ensure that all those with Progeria have a chance at longer, healthier lives, while the search for better treatments and the cure continues.



Kahlil (age 4, from Guyana) joined the PRF family in July—long after enrollment for the 2-drug trial ended—and is now receiving a steady supply of lonafarnib through the monotherapy extension trial.

There are currently 35 children from 18 countries participating in the monotherapy trial.



3-year-old Mirela from Brazil is one of the newest members of PRF's community. She made her first trip to Boston for the monotherapy extension trial in August.

The Progeria clinical trials offer the best chance to accomplish our mission, representing the culmination of years of research designed to discover ways to improve the health and lifespan of those with Progeria. The trials have also provided vital information on the disease process, including heart disease, paving the way for new drug candidates and other findings that can advance the field of Progeria and aging-related conditions that impact us all. ***These clinical trials are the reason we have a treatment, and future trials will one day be our ticket to the cure!***



Livia (age 9, from Brazil) completed her final 2-drug trial visit in October 2019, and has since rolled into PRF's monotherapy trial to continue receiving lonafarnib regularly.

New Research Grants

Over the past 23 years, PRF has provided **\$9.2 million** to fund **84 grants** for Progeria-related research projects performed at **54 institutions** in **15 countries!** These projects have resulted in key findings in Progeria, heart disease, and aging, and have brought our understanding of Progeria to new heights.

Thanks to your support, we're funding three new, international research projects that will be conducted in Germany, France and Spain:



Karima Djabali, PhD

Technical University of Munich

"Treatment of Hutchinson-Gilford Progeria Syndrome with two FDA-approved drugs combined – Lonafarnib and Baricitinib, specific inhibitors of farnesyltransferase and JAK1/2 kinase respectively"

Dr. Djabali's project will test in a mouse model of HGPS whether treatment with a combination of lonafarnib and baricitinib, an anti-inflammatory drug, will delay development of the typical HGPS pathologies, namely vascular disease, skin atrophy, alopecia, and lipodystrophy. Her previous findings link the JAK-STAT pathway with inflammation and cellular disease features of HGPS. In vitro administration of baricitinib with lonafarnib improved some HGPS cellular phenotypes over and above lonafarnib alone.



Laurence Arbibe, MD, PhD

Institut Necker-Enfants Malades (INEM) – Paris

"Unraveling accelerated intestinal ageing in HGPS physiopathology: an integrative approach"

Dr. Arbibe's lab recently showed that chronic inflammation extensively alters quality control of pre-mRNA splicing in the gut, one of the consequences being the production of the toxic, disease-causing progerin protein. Now, she will explore the impact of progerin toxicity on intestinal homeostasis and implement a reporter mouse model enabling in vivo tracking of the progerin-specific splicing event. This project will address the consequences of Progeria disease on the integrity of the gut, while also providing the scientific community with new resources for investigating tissue- and cell-specific drivers of accelerated ageing in HGPS.



Silvia Ortega Gutiérrez, PhD

Complutense University – Madrid

"Reduction of progerin levels by small molecules as a new approach for treating Progeria"

Dr. Ortega Gutiérrez will focus on the design and synthesis of a class of compounds called proteolysis-targeting chimeras (PROTACs). PROTACs can bind to specific proteins and tag them for degradation, effectively removing them from a cell. She will identify PROTACs that can bind to progerin, thereby reducing progerin levels and improving disease. After identifying a promising PROTAC compound, she will test it to see whether it can improve disease in a mouse model of Progeria.

Historic Summit Held

PRF's First Progeria Aortic Stenosis Intervention Summit was a great success!



**Progeria
Aortic Stenosis
Intervention Summit**



With the children living longer due to lonafarnib therapy, physicians are seeing aortic stenosis (narrowing of a critical heart valve) as a problem in the older children and young adults with Progeria that may be amenable to lifesaving surgery. In May 2022, PRF partnered with **Boston Children's Hospital** and **Brigham and Women's Hospital** to host a specialized meeting of high-level cardiologists and researchers to discuss surgical intervention for critical aortic stenosis in Progeria.

Led by Drs. Leslie Gordon, Ashwin Prakash, and Marie Gerhard-Herman, 24 invited experts joined the summit. They discussed the challenges around two main strategies for treatment: aortic valve replacement and implantation of a vascular conduit. Two patients have undergone each of these two procedures, and the group will work towards strategies for successfully evaluating and implementing these surgeries for others who will need these lifesaving procedures in the future.



Close friends Zach and Kaylee, the first two to undergo aortic stenosis surgery within days of the summit, are true success stories! Here they are kicking back together at a restaurant near Boston Children's Hospital, several days after Zach's procedure, as Kaylee was gearing up for hers. Zach is 15 years old, and Kaylee is 19.

Chapter, Family and Annual PRF Events

We're back! Chapter and other events have returned to in-person formats over the past year—many offering a virtual option as well, so everyone can participate however they're most comfortable. Special events are the community glue that connects thousands of PRF supporters each year, all of whom have one goal: to help cure Progeria.

THANK YOU for being with us together again—*for the children, for the cure.*

A time to celebrate: PRF's 20th Annual International Race for Research! Last September, we were thrilled to welcome back 224 runners and walkers to our home turf in Peabody, MA, as well as dozens of virtual participants who helped us celebrate 20 years of running from neighborhoods far and wide.



Carlos Luiz Silva, PRF Executive Director Audrey Gordon, Kevin Tierney from Lead Sponsor North Shore Bank, and PRF Ambassador Meg Waldron take the stage to announce the winners

Casting Lines for the Cure in North Carolina

The 6th Annual **Deallaney Hudson Honorary King Mackerel Fishing Tournament** in the Southern Outer Banks of NC had a great turnout in November 2021, with an amazing 54 fish-loving boaters attending the event. The winner went home with a 77-pound fish—holy mackerel!



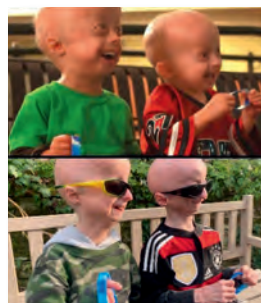
Deallaney with the oh-FISH-ial sponsor list

Cruising on Two Wheels for Progeria!

It was a "wheely" cool day to be a biker when a group of motorcycle enthusiasts once again hit the streets in July in support of the **12th Annual Zach Attack Ride for Progeria** in Lexington, KY. On top of a great afternoon out with friends, riders enjoyed food, a silent auction and raffles after their journey.



Zach Attack's team of bikers cruising the roads of Kentucky for Zach and his friends



Best of all, Zach's good buddy Cam joined him from Pittsburgh, PA, to cheer on the bikers. The two have been close friends since they were 3 years old.

Cam and Zach in 2010 (top), and recreating the pose in 2022 (bottom)

A Golf Par-tee in NJ 80 enthusiastic golfers played a strong game in support of Zoey and PRF's New Jersey Chapter, at **Team Zoey's 12th Annual Golf Outing** at Preakness Hills Country Club last September.

This year's September outing will be the last one on the course, bringing together Zoey's amazing community of support one last time in celebration of her 13th birthday!



Zoey and her brothers Gavin and Aidan having a tee-rrific time together



Special Thanks to Our Boston Marathon Runners

Paul Michienzie (left), and Lucas Paratore (right) really went all out for children and young adults with Progeria by taking on the esteemed 26.2-mile course from Hopkinton, MA to Boston this past April, going the distance and raising over \$25,000 — **WOW!**



Members of TEAM PRF take a bite out of victory at the finish line of the 2021 Peabody course!

TEAM PRF Goes the Distance at the 2021 and 2022 Falmouth Road Race

In August 2021, 11 TEAM PRF members ran the 7-mile Falmouth at-home edition from a variety of locations. This year, a record high 27

TEAM PRF runners gathered in Falmouth and raised a record-breaking \$35,000 — **AMAZING!**



Making Waves with Nathan and Bennett

The 11th Annual **Make-a-Splash** event hosted by our PA East Chapter was held virtually once again last November. A new and exciting addition this year was a live trivia game—a true test of knowledge for the many participants who helped bring in \$30,000 on behalf of the Fighting for their Future community.



The Falcones sure know how to make WAVES with their friends and family



A Hoppy Occasion—Wormtown Brewery Teams up with PRF

In February, our friends at Wormtown Brewery in Foxboro, MA, opened their doors to celebrate a limited release brew,

'Sam's Hoppy Life,' in honor of Sam Berns (see Sam's "My Philosophy for a Happy Life" TEDx Talk). The beer was inspired by many flavor profiles and aromatics that Sam loved. The event included live music from Sam's childhood friend Matt Michienzie, inspiring words from PRF co-founders, and beer lovers from near and far. **Cheers!**



PRF Co-Founders Drs. Leslie Gordon and Scott Berns, toasting to Sam with Wormtown Brewery owner David Fields and PRF Executive Director Audrey Gordon

SAVE THE DATES!

HaTs ON
for PROgeria

Friday, October 21, 2022

Wear your favorite hat and support PRF with hashtag #HatsOnProgeria
Details at: progeriaresearch.org/hats-on-for-progeria/

Golf for PRF

Monday,
May 15, 2023

Ipswich Country
Club in MA

Come play
FORE the cure!
prfgolf.org



Miracle Makers

Our Miracle Makers—PRF supporters who go above and beyond for those with Progeria—are instrumental in our fight for the cure! These amazing people give their time and talents to help PRF in unique and vital ways. Here are just a few of our heroes—we wish we could include them all!

To learn how YOU can become a Miracle Maker, visit progeriaresearch.org/be-a-miracle-maker/



Lonafarnib access far and wide

In 2019, PRF partnered with Dr. Jianhua Mao, Professor and Chief Physician at The Children's Hospital of Zhejiang University School of Medicine, to find and help children with Progeria in China. Since then, Dr. Mao and his colleague, Dr. Jingjing Wang, have helped make it possible for children in China to obtain lonafarnib through a Managed Access Program (MAP). With MAP, the children can access lonafarnib through their physicians, instead of seeking access through the clinical trial, which would necessitate traveling to Boston—an option unavailable since March, 2020 due to COVID. Over these three years, he has also helped PRF find an unprecedented 14 children with Progeria in China (compared with 21 total in the past decade!), and has helped 20 children in various ways, connecting them with PRF's vital programs and services, as well as the opportunity to join future clinical trials.

Sammy Runners – Marathons for Milestones

Sammy Runners are a group who have come together over the years in support of their friend, 26-year-old Sammy Basso of Tezza sul Brenta, Italy. The team has run numerous road races across Italy to raise Progeria awareness and exemplify the importance of having fun, being together, and always being ready to go the distance for their friend. Sammy is able to be with his team throughout every race in a special wheelchair, pushed by the legs, lungs and hearts of his dedicated and growing group of friends. Che meraviglia!



A posthumous tribute to the wonderful Anne French

In February, a group of Nathan and Bennett Falcone supporters led by Anne French, a close friend of the Falcone family who has sadly since passed away, raised \$15,000 in a Superbowl square fundraiser.

She loved the boys deeply and wanted to honor them in the best way possible—funding research toward the cure. We're grateful for Anne's steadfast commitment to these boys, and her selfless drive to advance PRF's mission. You can honor Anne and the boys by organizing a 2023 Superbowl square fundraiser, too!



All in the family

For the Michienzies, supporting PRF has always been a family affair. Parents Debbie and Paul, and their three children Chris, Emily and Matt, lived down the street from Sam Berns and his parents, PRF Co-Founders Drs. Leslie Gordon and Scott Berns (all pictured, above). The Michienzies have supported PRF in numerous ways over the years: Debbie has co-chaired PRF's Night of Wonder gala; Chris and Emily have run on PRF's Falmouth Road Race team; Matt provided the musical talent during 'Sam's Hoppy Life' beer release party, and Paul has taken to the streets for PRF many times in Falmouth as well as the Boston Marathon. Needless to say, the Michienzies are a dedicated bunch, and we are so grateful!

Milestone Mania

As we reflect on the past year at PRF, we celebrate the many exciting milestones we've achieved—stepping stones in our journey to cure Progeria, each its own unique emblem of the progress we've made.

Here are just a few of our latest...

✓ For the first time, PRF is aware of more than 200 individuals living around the world with Progeria (defined as the classic, Hutchinson-Gilford Progeria Syndrome -- HGPS) or Progeroid Laminopathies (PLs). Presently, we are aware of 140 with Progeria and 65 with PLs.

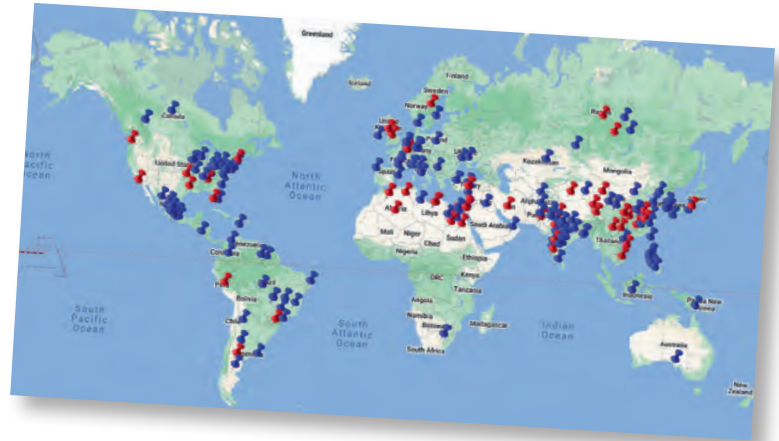
Today, more children and young adults than ever are getting the unique care they need, thanks to PRF's efforts to find them.

✓ There are various pathways to diagnostic testing in Progeria, one of which is through PRF's Diagnostic Testing Program. In June, PRF diagnosed **the 100th individual with classic Progeria**. That child now has a definitive diagnosis that will enable access to lonafarnib and future clinical trials, as well as access to any new information that may benefit her in the future.

✓ To keep up with our growing workload, our staff is the fullest yet, with 14 part- or full-time employees, including two new, fully remote members, which we've never had until now. PRF is adapting to the times, and ensuring administrative progress toward the cure is advancing as effectively and efficiently as possible.



✓ PRF is publishing our **first-ever Portuguese Clinical Care Handbook - 2nd Edition**, bringing the total number of languages this important guide is available in to 5. From basic health facts to daily care recommendations to extensive treatment guidelines, this 131-page handbook helps answer many questions for caring for those with Progeria.



✓ The 100th Progeria clinical trial participant came to Boston to be treated with lonafarnib. Clinical trials are the key to discovering treatments and the cure.

✓ In a beautiful story about the magnificent life of Sammy Basso and his extensive research into his own condition, **PRF was featured in the New York Times for the 5th time**. Media exposure is key to finding those with Progeria, and to gaining support for PRF's work.



✓ We've logged the **highest-ever number of children and young adults in PRF's registry program** in a single year (29 enrolled in 2021). For comparison, PRF registered only 38 children and young adults with Progeria in its first ten years of existence.

✓ As this newsletter goes to print, Sam Berns' TEDx talk, 'My Philosophy for a Happy Life,' will soon hit **50 million total views**. Combined views of Sam's talk on TEDx.com and



TED.com will soon add up to a collective total of 100 million (anticipated in a matter of months). **Sam continues to inspire the world!**

PRF 2021 Annual Report

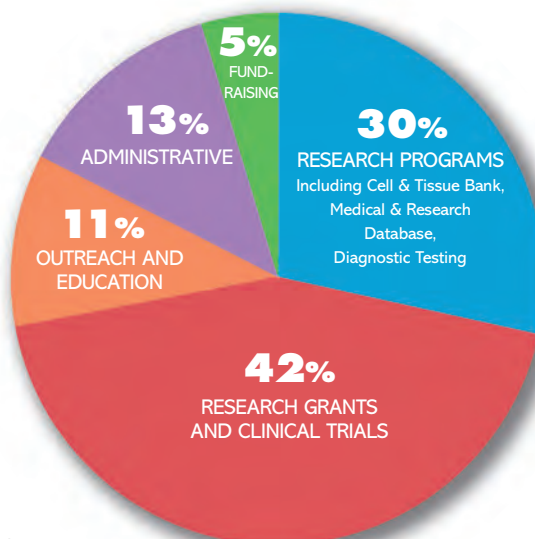
INCOME ANALYSIS



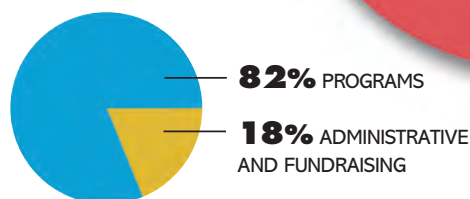
TOTAL OPERATIONAL REVENUE*
\$1,671,264

TOTAL EXPENSES
\$2,946,682

EXPENSE ANALYSIS



PROGRAM RATIO



*Operational revenue excludes the proceeds related to the sale of the Priority Review Voucher (PRV) issued after FDA approval of lonafarnib as a treatment for Progeria. The total 2021 revenue, including net assets and investment income related to the non-recurring PRV, = \$49,739,365. PRF's 10-year, average income prior to this historic payment = \$2.6 million.

Core PRF Activities That Support Our Mission

2021 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY (1999 - 2021)
29 children from 19 countries a new record and 9.1% increase over 2020!	International Patient Registry (includes unconfirmed cases)	348 children with Progeria from 70 countries and all continents have registered with PRF
96 cell lines sent to 16 labs in 9 countries; 7 tests completed	Cell & Tissue Bank and Diagnostic Testing Program	1,360 cell lines sent to 208 teams in 26 countries; 159 children tested through PRF
12 children enrolled; treatment guidelines provided to all	Medical & Research Database	199 enrolled; treatment guidelines sent to families and physicians in 53 countries and 1 U.S. territory
Everolimus & lonafarnib Trial final visits continue; 2 new children enrolled in Monotherapy Extension	Clinical Trial Funding & Co-coordination	100 children and adults from 40 countries have participated in PRF clinical trials, initiated in 2007
10 research grants ongoing (extensions granted due to COVID-19); includes 2 new research grants awarded	Research Grant Funding	81 grants in 14 countries; funding total: \$8.7million
Planning for 2022 Workshop began	International Scientific Meetings	14 meetings: 10 general workshops and 4 subspecialties
9 events, 85 new volunteers (including 24 new volunteers in PRF's translator program!)	Volunteer-Led Events and Other Activities	Over 5,000 volunteers worldwide have helped raise awareness and funds
12 children visited the U.S. and required an interpreter	Translations Program	PRF's program and medical care materials are translated into 39 languages
25 new children identified	Public Awareness	196 children and young adults living with Progeria and Progeroid Laminopathies from 50 countries

In Loving Memory...

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



Aaron

2 years old
From Argentina



Adalia

15 years old
From the United States



Ahmad

8 years old
From Palestine



Bakshi

25 years old
From Sweden



Claudia

23 years old
From Portugal



Heba

10 years old
From Egypt



Joel

14 years old
From Argentina



Julieta

19 years old
From Argentina



Leticia

14 years old
From Brazil



Mostafa

7 years old
From Saudi Arabia



**Nur Alya
Sabrina**

17 years old
From Malaysia



Qonita

11 years old
From Indonesia



Rayasat

14 years old
From Sri Lanka



Retal

1 year old
From Egypt



Saju

13 years old
From Bangladesh

*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
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5-year-old Zein
from Egypt is ready
to rock a new year
at school!

Due to our concern for the environment and rising paper costs, WE'RE GOING PAPERLESS NEXT YEAR! We'll still email you a copy of the newsletter, and you can always find it on our website. Not sure if we have your email? Subscribe to the newsletter at the bottom of our homepage: www.progeriaresearch.org. Don't miss out!

THE PROGERIA RESEARCH FOUNDATION 11TH INTERNATIONAL SCIENTIFIC WORKSHOP



We're **starting our engines**
and **revving up!**

**REGISTER
NOW!**
prfworkshop.org



Since its inception in 1999, our scientific workshops have provided a platform that fosters collaboration among top minds in Progeria research, cardiovascular disease and aging.

Join us to meet children and young adults with Progeria, and interact with the Progeria Basic and Clinical Scientific Communities.

Stay current with cutting-edge scientific presentations, poster sessions, and high-level collaborative discussions that will lead to the next wave of treatments and to the cure!

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