



Progeria

Research Foundation

FOR THE CHILDREN ♥ FOR THE CURE

WWW.PROGERIARESEARCH.ORG • SEPTEMBER 2020

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SAVE THE DATE (again!)

COVER

The world has changed. Our mission has not.

As we all navigate this uncertain time, our fight against Progeria remains steadfast. PRF staff and the clinical trial team have been working closely with our Progeria families around the world, to ensure their continued access to PRF's vital services. Our programs have been operating at full capacity, as we continue to provide researchers with the tools they need to proceed *full steam ahead to the cure!*

Thankfully, we do not know of any children with Progeria who have COVID-19, and our staff remains healthy as well.

Throughout the pandemic, PRF's work has not stopped – and the families of children and young adults with Progeria have not stopped needing and hoping for the cure. THANK YOU for your dedicated support, which makes the **work**, the **progress**, and the **hope** possible.



10-year-old Aaditya from India takes precautions wherever he goes.



★★★★★
CHARITY NAVIGATOR
Four Star Charity

We did it again!

PRF joins just 7% of reporting charities in accomplishing this highest distinction for **7 straight years**.

The age of gene therapy for Progeria is approaching...

PRF continues to support exploration of the cutting-edge gene editing field for Progeria, which is advancing at a rapid pace.

See page 6 for more details.

A Message from the Executive Director

Hello everyone,

To say this year has been challenging – for the entire world – is certainly an understatement. Through it all, the staff, Board of Directors, trial team, grantees, volunteers, families, and donors have remained focused and determined in the respective ways they support PRF's mission. Whether it's adjusting to working remotely, strategizing how best to manage the budget in the wake of revenue reductions, ensuring trial patients have enough drug until travel resumes, changing in-person fundraising events to virtual, donating whatever they can, given the economic downturn, or taking extra precautions to keep those with Progeria COVID-free, our army of supporters is ALL IN. How lucky we are to experience such devotion to these remarkable children!

As the pages that follow demonstrate, our work and our progress continue at an extraordinary rate. Some of the most striking evidence of this can be found in our 2-drug clinical trial infographic on page 5: to date, just for this one trial, participating children and parents have flown over 1.5 million miles. Such a testament to the hard work of the trial team, and the bravery and commitment of the families!

Lonafarnib continues to have a positive impact in fighting Progeria, and as a result, we devote lots of time, energy and resources to ensure access to this drug. This past year has brought many new examples of why we can now refer to those we help as children and **young adults** with Progeria:



Fatma from Libya, Niccolo from Italy, and Lindsay from Michigan all turned 16. Lindsay, a National Jr. Honor Society member throughout middle school, advanced to National Honor Society as she begins her junior year of high school this month – all A's, all the time!



19-year-old Meghan from Boston completed her freshman year at college.



20-year-old Megan from Wisconsin has been realizing her dream of participating in horseback riding events.



Sammy from Italy was awarded knighthood by the Italian Republic, and celebrated his 24th birthday in December 2019.

Many others are celebrating similar milestones and achievements. Their futures are brighter than ever, thanks to your support and dedication to our mission. **We look forward to continuing our treasured partnership with you – For the Children ♥ For the Cure.**

Audrey Gordon, Esq.

President and Executive Director



It was so wonderful to spend time with Ana Clara (Brazil) and Mio (Japan), who came to Boston for their clinical trial visits in January.

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. *Executive Director*

Gina Incrovato *Director of Operations*

Kelsey Tuminelli *Patient Programs Coordinator/Family Liaison*

Michelle Fino *Senior Development Officer*

Lynne MacKenzie *Donor Relations Director*
and *Social Media Coordinator*

Eleanor Maillie *Communications Manager*

Kristine Valente *Executive Assistant to the ED/Office Manager*

Lynn Doucette *Executive Assistant*

Christina Sollecito *Executive Assistant to the Medical Director*

Odette Kent *Data Specialist*



Progeria is a fatal, “rapid aging” condition that afflicts children who, without treatment, 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 heart disease and aging, what we learn from Progeria research has the potential to benefit all of us.

VOLUNTEERS:

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Clinical Trial and Lonafarnib News

Everolimus plus Lonafarnib 2-Drug Trial in Final Stages

Our trial team continues to ensure that the current PRF-funded clinical trial is running effectively and efficiently. Just over 2/3 of those enrolled have finished their two trial visits in Boston, where they underwent a series of tests and got new drug supply.

Due to the COVID-19 pandemic, only one child, traveling locally, has been able to come to Boston since March. Travel will resume for the remaining 18 when further lifting of travel restrictions and adherence to all recommended safety precautions will allow us to welcome them back. Once trial visits are complete, the trial team will conduct data analysis to determine whether the 2-drug combination is a more effective treatment regimen for Progeria than lonafarnib alone.

Access to lonafarnib: present and future efforts

What happens when children finish the trial? What about children found after trial enrollment for the everolimus plus lonafarnib trial was completed? Typically, outside of a trial, neither group would be able to obtain the drug(s) being tested. But lonafarnib has been shown to give the children stronger hearts and longer lives, so access to it is vital.

As announced last September, thanks to our partner Eiger BioPharmaceuticals, there is a Managed Access Program (“MAP”) that enables access to lonafarnib in home countries where MAP is available. For countries that do not offer MAP, PRF is funding a new, monotherapy (1-drug) extension of the current trial, that provides lonafarnib to children who would not otherwise be able to get this important drug. These innovative solutions to drug access ensure that all those with Progeria have a chance at longer, healthier lives, while the search for better treatments and the cure continues.



Jumping for joy! Carlos, who lives in the Boston area, was the first to resume his clinical trial visits at Boston Children's Hospital after the pandemic-related restrictions began. Carlos was so comfortable in his hospital gown that he asked if he could keep it!



Taking a break from their final trial visit in February 2020 with a trip to the Museum of Science, 13-year-old Adalia from the US and 10-year-old Anna from Russia give each other a big HIGH FIVE!

Applications to FDA and EMA for lonafarnib approval are IN!

In March, Eiger BioPharmaceuticals completed submission of a New Drug Application, seeking approval – in Europe (European Medicines Agency, or EMA) and the US (Federal Drug Administration, or FDA) – of the drug lonafarnib as a first-ever treatment for Progeria. This submission is the culmination of 12 years of research data and 4 clinical trials, all funded by PRF and made possible by the courageous children and their families, our partners at Boston Children's Hospital, Brigham & Women's Hospital, and Eiger BioPharmaceuticals, and PRF's wonderful community of donors. THANK YOU ALL, and we will keep you updated on this developing news.

Our hope is that lonafarnib will be approved by the FDA and EMA, an historic feat that would enable many of those with Progeria to access this drug by prescription, instead of through a clinical trial.

5 AWESOME facts about PRF'S 2-DRUG TRIAL to date

Children from
27 countries
flown to Boston!

20
languages
spoken!

hello! hola!
bonjour! ciao!
salut! ni hao!



Over **1,568,011**
miles traveled!
(that's almost 63 times around
the globe!)

60 Visas
secured!

798 nights
in hotels!



all
THANKS
to **YOU!**

Cover story (continued)

Gene Therapy for Progeria?

In his April 2019 TED talk, *Can we cure genetic diseases by rewriting DNA?*, chemical biologist David Liu, PhD, Professor at Harvard and the Broad Institute, shared a breakthrough on Progeria:



'Children with progeria are born with a T at a single position in their genome, where you should have a C, with the devastating consequence that these wonderful, bright kids age rapidly and pass away by about age 14. Throughout the history of medicine, we have not had a way to efficiently correct point mutations in living systems, to change that disease-causing T back into a C... perhaps, until now; Scientists have succeeded in achieving a critical milestone towards that goal... recently delivering a base editor into a mouse with progeria, changing that disease-causing T back to a C and reversing its consequences at the DNA, RNA and protein levels. Additional work lies ahead before base editing can realize its full potential to improve the lives of patients with genetic diseases...'

Dr. Liu's research on Progeria is funded in part by The Progeria Research Foundation. He will be presenting his latest findings on gene therapy for Progeria at PRF's 10th International Workshop Webinar November 2-3, 2020. We hope to 'see' you there!

PROGERIA RESEARCH FOUNDATION 10TH INTERNATIONAL SCIENTIFIC WORKSHOP

researching possibilities
extENDING lives
WEBINAR VERSION



Register Now

prfworkshop.org

November 2 and 3, 2020

10:00 am -1:00 pm each day

US Eastern Standard Time



Together we *WILL*, find the cure!



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

Stay current with cutting edge scientific presentations on genetic therapies, new medical and surgical treatments and more!

Look forward as we preview our next in-person workshop planned for **November 8-10, 2021** in Cambridge, Massachusetts.

Research Grants

Worldwide Research Continues: PRF Awards 5 Grants to Researchers in 5 Countries!

Continuous and innovative scientific study of Progeria is vital to discovering more effective treatments and the cure. Since 1999, PRF has awarded 77 grants to researchers working in 14 countries – a true reflection of the international reach the field has achieved. These devoted, diligent scientists have led the field 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 in Progeria research that will lead to novel, cutting-edge treatments and the cure.

Thanks to your support, we were able to fund five more projects:



Colin Stewart, PhD

*Institute of Medical Biology,
Immunos, Singapore*

**“Breaking the LINC to
Suppress Progeria.”**

Dr. Stewart is a highly experienced investigator in the field of Progeria research. Over the last decade, his research has centered on laminopathies, a heterogeneous collection of diseases all arising from mutations in the LMNA (Lamin A) gene that affect aging, cardiovascular function, and muscular dystrophy. He and his colleagues have shown that deletion of a protein called SUN1 reverses weight loss and increases survival in progeria-like mice. He will now perform drug screening based on this finding, examining thousands of chemicals for any that may disrupt SUN1 and could potentially serve as new drugs to treat children with Progeria.

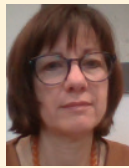


Bum-Joon Park, PhD

*Pusan National University,
Republic of Korea*

**“Effect of progerinin (SLC-D011)
and lonafarnib on HGPS: A com-
bined in Vitro and in Vivo.”**

Dr. Park has developed a drug called progerinin that inhibits progerin and improves disease in progeria cells in mice. Dr. Park will now investigate the synergistic effects of progerinin with lonafarnib. He will compare single drug treatment (lonafarnib) and a combination treatment (progerinin and lonafarnib) to determine which is most effective. If the drug combination has low toxicity, a combined clinical trial of progerinin and lonafarnib could be on the horizon!



Giovanna Lattanzi, PhD

*CNR Institute of Molecular Genetics Unit
of Bologna, Italy*

**“Improving the quality of life in Progeria: A first trial in
the murine LmnaG609G/G609G model.”**

Dr. Lattanzi will address quality of life in Progeria, which is related to a chronic inflammatory state. Normalizing the inflammatory state may help patients face pharmacological treatments; if their health state is improved, they can attain better efficacy and extend lifespan. Dr. Lattanzi will test strategies for reducing chronic inflammation in a Progeria mouse model, with the goal of transferring results to patients.



Vicente Andrés, PhD

*Centro Nacional de Investigaciones Cardiovasculares (CNIC),
Madrid, Spain*

**“Generation of transgenic Lamin C-Stop (LCS) and CAG-
Cre Yucatan minipigs to breed HGPS Yucatan minipigs for
preclinical trials”**

A key area of research in Dr. Andrés' lab is directed towards the generation of new animal models of Progeria. Large animal models recapitulate the main hallmarks of human disease much better than mouse models, allowing us to investigate cardiovascular disease and test therapies. Dr. Andrés' model will improve upon a new minipig model of Progeria that was previously funded by PRF.



Elsa Logarinho, PhD

*Aging and Aneuploidy Group, IBMC – Instituto de
Biologia Molecular e Celular, Porto, Portugal*

**“Small-molecule enhancement of chromosomal
stability as senotherapeutic strategy for HGPS”**

Dr. Logarinho's project aims to explore the effects of a small-molecule agonist of the microtubule (MT)-depolymerizing kinesin-13 Kif2C/MCAK (UMK57), to counteract HGPS cellular and physiological features. Her previous findings grade Kif2C as a key player in both genomic and chromosomal instability, which are causally linked, and also established as primary causes of progeroid syndromes. Stabilizing Progeria chromosomes at the cellular level aims to improve disease throughout the body.

From our ONEpossible 2020 fundraising campaign: A Parent's Journey...

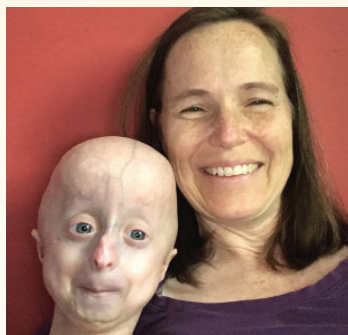
We asked parents what PRF has meant to them, and were overwhelmed by their responses! ***This is why we do what we do.***



Zach and his mom; USA

"Connecting with PRF filled us with such relief – someone was out there who cared about my son, who was fighting for a cure. They give us peace of mind, knowing there are world-class researchers in our corner, working to cure this fatal disease. I'm so grateful to PRF for fighting for Zach every day, and for making Zach and our family's future brighter."

– **Tina**, Zach's mom



Cam and his mom; USA

"We are so lucky to have The Progeria Research Foundation – they give us hope... thank you to all the people who support us, making our journey with Progeria lighter."

– **Catherina**, Enzo's mom



Enzo and his parents, Australia

"I frequently tell people who don't know about PRF how lucky we are to have you helping our kids. Please know that you have made a huge difference in our lives."

– **Stephanie**, Cam's mom

"While I wish PRF had existed for my daughter Amy, who passed away in 1985, I was blessed to have her, and it's a blessing that PRF is now here for the children and their parents."

– **Terry**, Amy's mom



Amy's mom and her friend, Zach; USA



Nathan, Libby and Bennett, with mom and dad; USA

"We want the same as everyone else – we want our boys to grow up... PRF is our hope and keeps us going."

– **Phyllis** (Nathan and Bennett's mom)



Brennen and his mom; USA

"PRF has blessed us with the gift of hope in our fight to find a cure for Progeria. When Brennen was first diagnosed, we were lost and distraught, not knowing where to turn to next, but the caring staff and loving families we've met through PRF have stood beside us every step of the way. We have truly gained a Progeria community family." – **Erin**, Brennen's mom

"We're eternally grateful to PRF for giving us the light and hope we needed when we realized that our 2-year-old daughter, Alexandra, was the only case of Progeria in Spain. The wonderful PRF team and their network of incredible professionals welcomed us with open arms. To those who've supported PRF along the way, we greatly appreciate your help so that Alexandra and her peers have a bright future." – **Cedric**, Alexandra's dad



Alexandra and her parents; Spain



Kaylee celebrates her 17th birthday; USA

"To me, The Progeria Research Foundation means hope and support. It's scary dealing with a rare disease, and I know that if I have any questions, they'll do their best to help. They've also helped parents with Progeria connect to share their experiences. I know they're working hard to find new treatments and a cure, and I can't imagine life without The Progeria Research Foundation."

– **Marla**, Kaylee's mom

"PRF is our lifeline... a family... our hope for amazing things to come."

– **Laura**, Zoey's mom



Zoey and her parents; USA

From all of us at PRF, as well as the children and their families, THANK YOU to everyone who donated to our 2020 ONEpossible campaign. We CRUSHED our \$110,000 goal – which will help offset funds lost from canceled events due to COVID-19. Each of you is ONE that will make the cure POSSIBLE!

To read more kind words from families, check out our "Meet the Kids" section of our website at progeriaresearch.org/family-quotes

Chapter, Family and Annual PRF Events

Special events are vital to our fundraising and awareness efforts. The energy and love from participants is palpable! From all of us at PRF, and from the children with Progeria and their families: THANK YOU to those who made these events such a hit. You are ALL helping us get closer to the CURE!

Most events featured took place in 2019, before COVID-19, and many this year were virtual or had to be canceled. We look forward to when we can all gather together again – **for the children, for the cure.**

7 Years Going the Distance! TEAM PRF Finishes Strong at the 2019 Falmouth Road Race

Last August, 15 PRF supporters were among the 13,000 runners who endured the 7-mile rigorous race course lined with tens of thousands of spectators. Shining bright in their orange shirts, TEAM PRF crushed their team fundraising goal. Way to go, runners!



Miles for Miracles 2020

This year's Miles for Miracles in May took on a different format, due to COVID-19. While the usual walk was canceled, the Ratcliffes' amazing supporters in Flatrock, MI came together – at a distance – and made the day a rousing success! Between some creative fundraising challenges – like Joe eating spicy Doritos – and a parade of cars streaming by the Ratcliffe house, not only did the event raise over \$20,000, but it also reflected the unwavering spirit of this community.



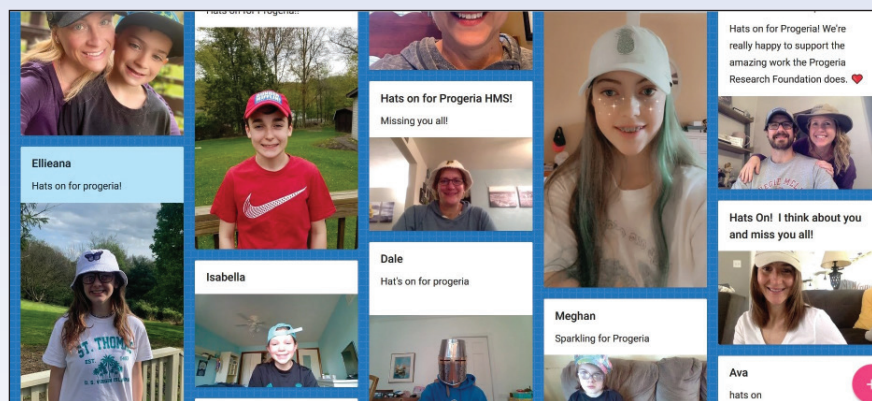
Joe, Lindsay and Kristy Ratcliffe look forward to next year's Miles for Miracles!



Another Tee-rific Year of Golf in NJ

Team Zoey's 10th Annual Golf Outing was held just after Zoey's 10th birthday, so it was truly a double celebration! At the post-golf dinner, Dr. Leslie Gordon updated this

crowd of steadfast supporters on PRF's progress, followed by singing and cake for the birthday girl. The 2020 tournament is ON for September 21st – so golfers, get ready for another fantastic year!



Although schools were closed, students and teachers from Hampton Township Middle School in Pennsylvania showed their support virtually for this year's HatsON to celebrate their buddy Cam. Thanks so much for the love, guys!

Tip of the Hat to This Year's HatsON Supporters

We're celebrating eight years strong of HatsON for Progeria! Classrooms, businesses, groups of friends and PRF social media followers once again joined us virtually in their favorite hats, in a show of solidarity and to help raise awareness for their friends in the Progeria community.

Please join us for our **Fall HatsON event on October, 16, 2020**, our **Spring HatsOn event on March 12, 2021**, or ANY day of your choosing! Don't forget to send us your photos by tagging your social media posts with **#HatsONProgeria!**

SAVE THE DATES!



**International Race for Research
AT HOME EDITION**

September 26-27, 2020

Wherever you live, you can run
or walk for PRF!

Visit **PRFroadrace.org** for details.

**PRF's Cure Cup
Classic Golf Tournament**
May 17, 2021

Ipswich Country Club in MA

Come play FORE the cure!

prfgolf.org





Some of the many bikers who hit the streets to join Team Zach's gang in Lexington, KY.

A Decade of Love and Support – On Two Wheels!

It was a beautiful day for the many bikers who came out to support the 10th Annual Zach Attack Ride for Progeria in July. Hundreds of riders enjoyed live music, food, and auction items. Keep on riding, Kentucky!

Super Star Zach Rocks the Stage

Team Zach Attack's first annual Rock the Night party was off the charts! Zach wowed the crowd of over 200 fans with his rock idol costume changes as they enjoyed live music, amazing auction items and raffle prizes, and much more. While the 2020 party had to be postponed, PRF's Kentucky chapter looks forward to picking right back up again when it's safe to party on!



Cold weather knows better than to mess with THIS Turkey Trot!

A Thanksgiving Weekend Tradition

Verona Park, NJ, was full of NJ chapter supporters from near and far for the 9th annual "Miles of Hope" Team Zoey Turkey Trot. Even though it was a bit chilly, everyone buttoned up their parkas and hit the streets in a show of

love and support for their girl Zoey. And such a great way to burn off those Thanksgiving calories!

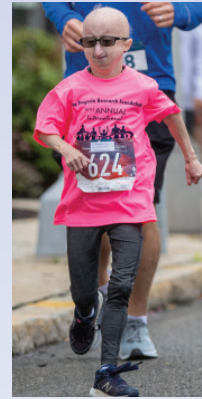


Make a Splash? More like a Tidal Wave!

This annual signature event hosted by our PA East Chapter is always a hit, but Make-a-Splash 2019 was truly spectacular! The *Fighting for their Future* community came together with outdoor activities for kids and adults alike, raising lots of awareness and funds for PRF. Special guest, mascot Phillie Phanatic, was on hand for all the fun too!

PRF's Race for Research Continues to Inspire Our Local Community

PRF's Annual International Race for Research is the biggest and longest-running road race in PRF's hometown of Peabody, MA. Last September there



was threat of a hurricane on race day, but the rain and wind held off, the race went off without a hitch, and it was a record-breaking success!



PRF Ambassador, Meghan Waldron (left), gave a rousing welcome speech, and Jomar from the Dominican Republic (right) had fun handing out the trophies.

Hooked on the Cure

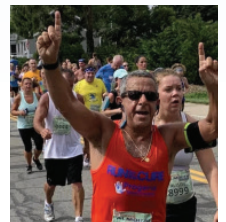
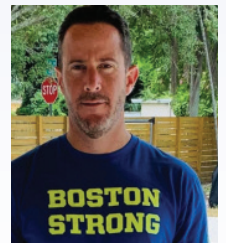


Deallaney and her team prepare to weigh in their catch.

The fourth annual Deallaney Hudson King Mackerel Fishing Tournament brought an even bigger turnout than last year, with hundreds of participants casting their lines from 39 competing boats, and backed by over 50 sponsors! The beautiful weather, combined with a very fruitful catch, made for a wildy successful tournament. Holy Mackerel!

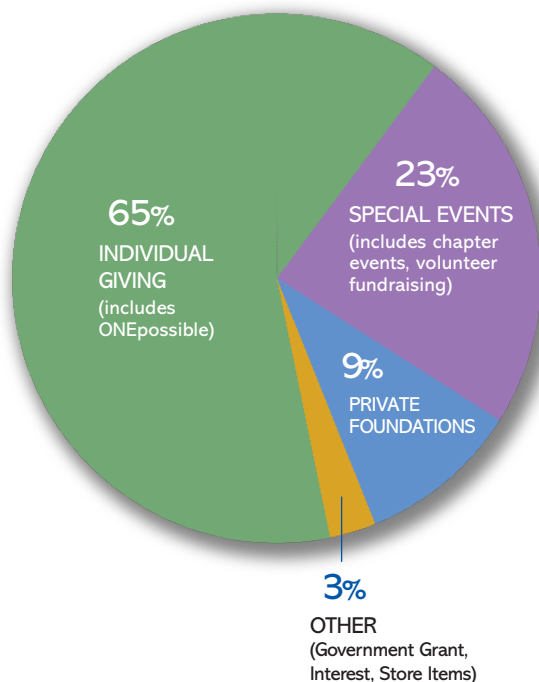
Special Thanks to Our Boston Marathon Runners

We are super excited that, for the first year, PRF received two coveted charity bibs! Even though the 2020 Boston Marathon was canceled, we are so grateful to our team – Bryan Dempsey (top) and Paul Michienzie (bottom) – who were willing to go the distance and run this historic marathon. Thank you for your commitment to PRF, the kids, and your training for 2021!



PRF 2019 Annual Report

INCOME ANALYSIS - 2019

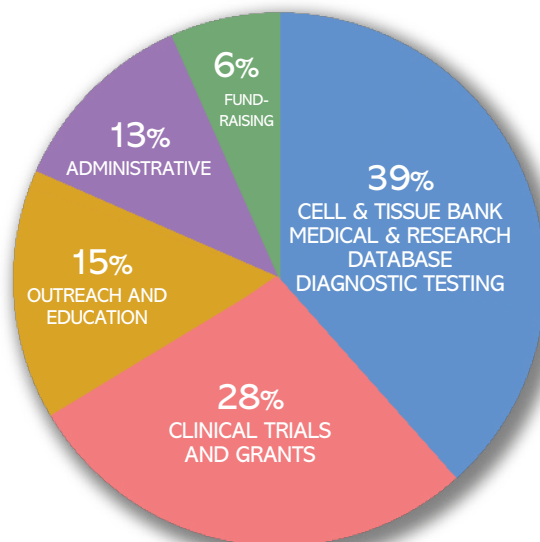


TOTAL REVENUE
\$5,046,788*

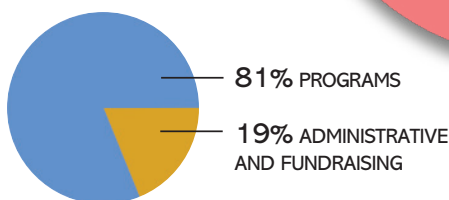
*Includes 1-time, directed gift of \$2.5m for new trial research

TOTAL EXPENSES
\$2,646,487

EXPENSE ANALYSIS - 2019



PROGRAM RATIO



Total net assets: \$6,942,443. Much of these funds are designated for future clinical trials and drug development costs.

Core PRF Activities That Support Our Mission

2019 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY (1999 - 2019)
12 children from 10 countries	International Patient Registry	285 children with Progeria from 64 countries and all continents have registered with PRF
153 cell lines sent to 27 labs in 6 countries	Cell & Tissue Bank Diagnostic Testing Program	1180 cell lines sent to 188 teams in 25 countries; 149 children tested through PRF
8 children enrolled; treatment guidelines provided to all	Medical & Research Database	184 enrolled; treatment guidelines sent to the families in 51 countries and 1 U.S. territory
Everolimus + Lonafarnib Trial, phase II continues	Clinical Trial Funding & Co-coordination	97 children from 38 countries have participated in PRF clinical trials, initiated in 2007.
5 research grants awarded	Research Grant Funding	76 grants to 50 labs in 15 countries; funding total: \$7.7m
Planning for 2020 International Workshop began	International Scientific Meetings	13 meetings: 9 general workshops and 4 subspecialties
18 events, 100 new volunteers	Volunteer Program	4,900 volunteers worldwide have helped raise awareness and funds
18 children who visited the U.S. and required an interpreter	Translations Program	PRF's program and medical care materials are translated into 38 languages
10 new children identified	Public Awareness	Presently: 176 living children from 54 countries – an all-time high!

PRF On The Move!

PRF's Cofounders Deliver Keynote Address at PCORI 2019

PRF's cofounders, Drs. Leslie Gordon and Scott Berns, are highly sought after speakers for healthcare and other conferences. In September 2019, they delivered a keynote speech for the Patient-Centered Outcomes Research Institute (PCORI) annual meeting. Their powerful presentation, "Making a Difference for Children with Progeria: From Obscurity to Treatment and Beyond," gave an overview of PRF's rich history in establishing the only organization in the world dedicated to curing children with Progeria. It's no surprise that they got a standing ovation!



Find the Children Campaign Finds Success in India and China!



This year has brought exciting progress in our quest to find and help children with Progeria worldwide. We've built a partnership with the Chinese Organization for Rare Disorders (CORD), which is helping to connect PRF with children in China. PRF also established a WeChat channel, China's widely used social media platform, to broaden outreach and connect with new families.

In India, we were able to achieve a stronger media presence this year. Highlights include Shreyash meeting his hero, the country's most famous actor, Amitabh Bachchan. Also, Dr. Leslie Gordon introduced Progeria as a featured theme to an audience of 6,500 pediatricians at PEDICON, the most prestigious annual



At the PEDICON conference, Aaditya was held by the Indian Health Minister, making headlines across the country

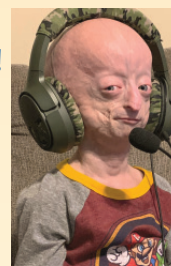
conference of the Indian Academy of Pediatrics.

Since the start of our *Find the Children* campaign last fall, PRF's Google search volume in India has doubled! A true sign of increased awareness.



Any Tiltify gamers out there? You can play live for Progeria!

Gamers, unite! You can now flaunt your gaming prowess in support of PRF. From latest hits like Fortnite, Minecraft, and Overwatch, to a simple game of cards, we invite you to live-stream your favorite games while helping raise funds and awareness for PRF. To learn more or to kick off your first gaming fundraiser, check out the link and FAQ's at <https://www.progeriaresearch.org/play-live-for-progeria/>.



Zach loves to play Minecraft!

Dr. Gordon Presents at Kings College in London

Last fall, PRF's Medical Director, Dr. Leslie Gordon, presented: "Progress, Lessons and the Future of Clinical Trials for Hutchinson-Gilford Progeria Syndrome" at the 3rd International Meeting on Laminopathies at Kings College in London.

Pictured here at the meeting is Professor Francesco



Musumeci (center), joined by Leslie and Scott (on the left), along with Sammy Basso and his parents, Laura and Amerigo, of the Associazione Italiana Progeria.

PRF is the 2020 EURORDIS photo contest winner!

For the first time ever, PRF submitted a photo in the annual photo contest held by the European Organization for Rare Diseases (EURORDIS), an alliance of patient organizations representing hundreds of rare diseases worldwide – and our photo took first place!

The contest gives those touched by a rare disease the opportunity to visually express what it means to live with that condition. Out of over 400 submissions, our photo of Zein from Egypt and his mother Dina, taken by volunteer PRF photographer Cheryl

Holbert Millard, received the highest vote count. Thank you to all who voted, helping us put Progeria in the spotlight!

Our Miracle Makers

PRF's Miracle Makers Lighten Loads and Brighten Lives

Our miracle makers are amazing! These volunteers give their time and energy to help PRF in unique and wonderful ways. Photographers, graphic designers, translators, fundraisers, ambassadors, event staff and more – cheers to all of you whose support is so important to our mission.

Want to learn how you can be a Miracle Maker? Visit progeriaresearch.org/be-a-miracle-maker/



Bek Wyzykowski (left), a talented crocheter of animal figures, came to PRF because, in her words, 'I've been deeply touched by the work you do for the kids. I'm grateful that you have such a dedicated team searching for ways to treat and cure these kids, allowing them more time to be kids.' Below, Carlos enjoys cuddling with a sloth, one of Bek's original creations.



Stephanie Johnson has volunteered as a PRF Ambassador for five years, providing transportation for children traveling to Boston for their clinical trial visits. Pictured here, Stephanie is returning from a December 2019 trip to the airport, dressed as Santa Claus! She's happy to help, and always with a smile – even for 5:30am arrivals!



'For my senior leadership project, I hosted a charity softball/ baseball game at my school to raise money and awareness for people with Progeria.

When I was in middle school, there was a girl named Hayden, and she had Progeria. She had a huge impact on me. Even though she passed away a few years ago, her love and determination still live with me. She was the most energetic, inspirational human being ever, always positive even in the tough times, and always found the good in things. Because of Hayden, I deeply care about this cause.'

– **Jared D.**, high school senior,
Summerville, SC



Michelle Kirschner (3rd from left) with her 2019 Make-a-Splash team. As president of the PA East chapter, Michelle tirelessly helps raise funds in honor of her close friends, Nathan and Bennett, and to support the Progeria community.



It doesn't get any SWEETER than this! THANK YOU to Gavin, Lucas, Adam, and Josh for their lemonade stand. They raised \$160 in support of Nathan and Bennett and PRF's Pennsylvania Chapter.

In Loving Memory...

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

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



Austin

6 years old
from the United States



Abdur

16 years old
from Bangladesh



Mats

17 years old
from Belgium



Kokona

12 years old
from Japan

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

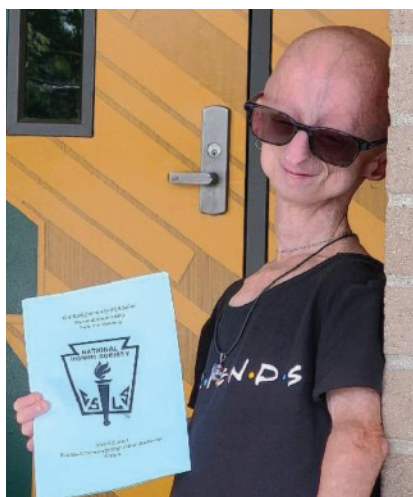


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*Lindsay looks forward to the new school year
as a member of the National Honor Society!*

NEW LAUNCH DATE FOR *VIRTUAL* NIGHT OF WONDER - DEC. 5, 2020!

Get ready to **blast off** for PRF's signature event,
as we hold a special **virtual** celebration on
December 5, 2020. We'll have exciting research
updates, fabulous auction items, special messages*
from the children you are helping, and lots more.

What a great opportunity for everyone to
support our mission to cure Progeria!

Help us get to the cure with **flying colors**.
Visit prfnow.org for details

Questions? Email prfnow@gmail.com or call 978-535-2594.



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