

news

Message from the President	2
The PRF Team	3
Clinical Trial Updates	4
Cover Story cont'd: FDA Approval and PRV news	5
PRF International Workshop	6-7
New Research Grants	8
Cover Story cont'd: Genetic Therapy Progress	9
Special Events	10-11
Annual Report 2020	12
ONEpossible	13
PRF On The Move! Our Miracle Makers	14
In Loving Memory	15
Night of Wonder SAVE THE DATE	BACK COVER

First-ever Treatment for Progeria Receives FDA Approval!

On November 20, 2020, in partnership with Eiger Biopharmaceuticals, PRF achieved an important piece of our mission: lonafarnib, the first-ever treatment for Progeria, was granted U.S. Food & Drug Administration (FDA) approval. *Progeria now joins fewer than 5% of the 7,000 known rare diseases with an FDA-approved treatment.*

See page 5 for more details on this historic news.

Breakthroughs in the Cutting-Edge Fields of Genetic and RNA Therapies

There has been tremendous progress recently in two types of therapy that could one day translate into the cure for Progeria. One study of **genetic editing** in a mouse model of Progeria corrected the mutation that causes Progeria in many cells, improved key disease symptoms and dramatically increased lifespan in the mice. Two other studies on the use of **RNA therapeutics** showed significant reduction of the toxic progerin-producing RNA, and substantial increase in survival in the mice.

Continued on page 9



We did it again!

PRF joins just 6% of reporting charities in accomplishing this highest distinction for **8 straight years**.

A Message from the Executive Director

Dear Friends,

When you look at Mio on the cover page, doesn't she make you **smile?!** Wearing bright pink boots and a pink, hand-knit hat, holding on tightly to a cherry-flavored Tootsie Pop and a sign with the most wonderful news. WE DID IT – an FDA-approved treatment for Progeria! Cause for celebration and reflection, for sure, but not for pause. As you'll see in the pages that follow, we are moving full speed ahead on discovery of other treatments that, in combination with Zokinvy (lonafarnib), could be even more effective. And we are exploring genetic therapies that could one day cure Progeria – incredible!

As we celebrate Zokinvy, we reflect on the many years of hard work that got us to this first treatment, and remain steadfast in our mission to cure children and young adults with Progeria throughout the world.

In this issue, we highlight other recent milestones that are cause for celebration, reflection, and determination to press onward:



PRF held its **10th International Scientific Workshop**, despite the cancellation of the in-person event due to COVID-19. We adjusted to a virtual format, and had a record turnout of 377 participants from 30 countries. Many called it the best virtual scientific conference – substantively and logistically – they had ever attended. Our very first workshop was in 2001 – a one-day gathering of a few dozen courageous researchers committed to the work, the mission and the children. How this vital event has grown!



We're completing the final patient visits for our current, **2-drug clinical trial**, and the next phase of data analysis will soon begin. We'll then know if the combination of lonafarnib and everolimus is more effective than lonafarnib alone. The Progeria clinical trials are the reason we have a treatment, and future trials will lead us to the cure.



With the increase in on-line communication due to COVID-19, we have been able to **reach more families** than ever before. Zoom has opened up a whole new level of connection with and among the families, which is so important when faced with a fatal, ultra-rare condition.



This year marks PRF's 20th annual **International Race for Research** in its hometown of Peabody, Massachusetts, USA. I remember the very first race – 200 people showed up, and we raised \$5,000. Each year, my nephew Sam and I would pick out the trophy styles. When he got older, we worked together on the speeches he and I would make to the crowd. Who could have ever imagined that he would become an internet sensation with one, 12 ½-minute **TEDx talk**, now on the verge of **100 million views?!** Sam loved the races, and I always think of him with love – and a **smile** – on race day.

Audrey Gordon, Esq.

President and Executive Director



Me and Sam at the 9th Int'l Race for Research in 2010. PRF will hold its 20th race on September 18th.

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*
Justine Maestranzi *Clinical Trial Coordinator*
Michelle Fino *Senior Development Officer*
Eleanor Maillie *Communications Manager*
Jennifer Brickley *Events & Communications Specialist*
Robyn Milbury *Development Officer*
Karen Gordon Betournay *Social Media Coordinator*
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 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.

VOLUNTEERS:

Medical Director:

Leslie B. Gordon, MD, PhD

Corporate Officers:

Audrey Gordon, Esq., *President*
Paula L. Kelly, CPA, *Treasurer*
Barbara F. Gordon, Esq., *Clerk*

Board of Directors:

Scott D. Berns, MD, MPH, FAAP, *Chair*
Karen N. Ballack, Esq.
Sandra Bresnick, Esq.
Paula L. Kelly, CPA
John Marozzi
Larry Mills
Liza Morris
Kim Paratore
Matthew Winters, Esq.

Board of Advisors:

Roger Berkowitz
Monica Kleinman, MD
Rabbi Harold Kushner, PhD
Suzette Kushner, MS
George M. Martin, MD
Robert K. Morrison
Elizabeth G. Nabel, MD
Bob Schwartzberg, CFRE

PRF Ambassadors

Meghan Waldron
Sammy Basso

Public Awareness Committee:

Liza Morris, *Chair*
Scott D. Berns, MD, MPH, FAAP
Audrey Gordon, Esq.
Leslie B. Gordon, MD, PhD
Eleanor Maillie

Medical Research Committee:

Bryan P. Toole, PhD, *Chair*
W. Ted Brown, MD, PhD
Judith A. Campisi, PhD
Maria Eriksson, PhD
Vicente Andres Garcia, PhD
Thomas W. Glover, PhD
Leslie B. Gordon, MD, PhD
Christine Harling-Berg, PhD
Monica Kleinman, MD
Tom Misteli, PhD
Marsha Moses, MD, PhD

Development Committee:

Audrey Gordon, Esq., *Chair*
Michelle Fino
John Marozzi
Kim Paratore

Finance Committee:

Paula L. Kelly, CPA, *Chair*
Scott D. Berns, MD, MPH, FAAP
Audrey Gordon, Esq.
John Marozzi

Chapters & Their Leaders:

California, Terry Foose
Kentucky, Tina Pickard
Michigan, Kristy & Joe Ratcliffe
New Jersey, Barbara Batesko
Ohio, Marla & Tim Halko
Pennsylvania West, Jim Schoonover,
Stephanie Howard
Pennsylvania East, Phyllis & Mark Falcone

Legal:

Arent Fox LLP,
Washington, DC

Goodwin Procter LLP
Boston, MA

Quinn Emanuel Urquhardt & Sullivan LLP
New York, NY

Weil, Gotshal & Manges, LLP
Silicon Valley, CA and New York, NY

Web Site:

Karen Gordon Betournay, *Coordinator*

Graphic Design:

Marie Migliaccio, Topsfield, MA
Julie Pritchard, Foxboro, MA
Regina Printing, Middlesex, NJ

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. All but 5 of the 53 trial participants have completed their visits to Boston, where they underwent a series of tests and got new drug supply. Since the COVID-19 pandemic began in March 2020, 21 children have safely traveled to Boston Children's Hospital. The remaining trial participants live in countries that have presidential proclamations blocking travel (China, Germany, India and Italy), and we hope they will be able to return in the near future.

In addition to seeing those who come in for their visits, the trial team is hard at work conducting data collection, the next step in determining whether the 2-drug combination of everolimus and lonafarnib is a more effective treatment for Progeria than lonafarnib alone.



Diego, from New Jersey, is all smiles on a trip to LEGO-LAND during his latest clinical trial visit in Boston. He just turned 12 in mid-August – Happy Birthday, Diego!

Alptug (left, age 5) and Veysel (age 7) in Boston, April 2021. They are both from Turkey and met during their first trial visit in 2018. For the past 3 years, their families have stayed in touch. The boys were very excited to see each other and spend the week together in Boston – such a sweet friendship!

Keeping Family Connections Strong

Did you know that PRF holds periodic group Zoom meetings with families and their local doctors? We have separate meetings for those who speak English, Spanish, Chinese, and one for all other languages. With well over 100 children and young adults identified, there's a lot to talk about. This past year, the group Zoom calls were held in December and June. We connected with those at the forefront of PRF's mission: the children and young adults with Progeria.

The goals of the meetings are to give everyone the opportunity to check in, ask questions, and stay up-to-date on the latest developments in our journey to new treatments and the cure. Most importantly, we all had a chance to visit with our global "Progeria Family". We are stronger together, and – Together, we *WILL* find the cure!

"I've never met an organization and [Gordon-Berns] family that has worked so hard for such a small group of people. We're so lucky to have this team that has supported us and found a treatment that prolongs Cam's life."

- Stephanie Howard, Cam's mom (top far right, appearing on the US-based family call)



Our Global Progeria Family: One of many screen shots from the PRF family Zoom meetings

FDA Approval of lonafarnib, brand name Zokinvy®

continued from front cover.



We are thrilled that lonafarnib, the drug tested in the PRF-funded clinical drug trials beginning in 2007, has been approved as a treatment for Progeria by the U.S. FDA. Lonafarnib is now referred to as Zokinvy, its brand name.

FDA approval gives access to this life-extending drug for children and young adults with Progeria through prescription in the U.S. Before approval, the only way children in the U.S. could access Zokinvy was through PRF's clinical trials. We are particularly grateful to Eiger Biopharmaceuticals (Eiger), for their sponsorship of the application for FDA approval, and for Eiger's ongoing efforts and commitment to ensuring access to Zokinvy to children and young adults with Progeria around the world.

Without this treatment, the average life expectancy of those with Progeria is 14½ years. Zokinvy improves many aspects of Progeria, including stronger hearts and bones, and extension of lifespan by an average of at least 2.5 years. With longer, healthier lives as a result of this treatment, many with Progeria are graduating from high school, attending college, working and living on their own, and developing into magnificent young adults.

Lonafarnib is the result of many years of drug development by countless scientists at Schering-Plough Research Institute and

Merck. Following the Progeria gene discovery in 2003, PRF worked with and funded researchers who tested the effects of lonafarnib on Progeria cells and mice. Thanks to all of these scientists, lonafarnib was "repurposed" from a cancer trial drug to a Progeria trial drug, starting in 2007.

This momentous milestone comes as a result of 13 steadfast years of clinical trials all co-ordinated and funded by PRF. All trials were conducted at Boston Children's Hospital (BCH) with clinicians who have become the world's experts on Progeria – Thank you BCH trial team!

All of this amazing progress was only possible because of your generous support, and the courageous children and their families who traveled many times from 37 countries to Boston for their trial visits.

Progeria's first-ever drug approval is a testament to the power of partnerships between a patient-centered nonprofit, academic researchers, hospital-based clinical trialists, and a pharmaceutical company – a strategy PRF will continue to pursue in the search for even more effective treatments, and ultimately the cure.

Priority Review Voucher Sale Brings Transformational Funds to PRF

The scientific excellence and resources PRF poured into making the Zokinvy FDA approval possible also brought another remarkable development: significant funds to invest in Progeria research, enabling PRF to accelerate our pace toward better treatments and the cure.

How did this happen? Under Section 529 of the Federal Food, Drug, and Cosmetic Act (FD&C Act) of 2012, when a drug is approved for a rare pediatric disease, the FDA issues a Priority Review Voucher (PRV) to the company that sponsored the application for approval (for Zokinvy, the company sponsor was Eiger). A PRV allows a pharmaceutical company to expedite the FDA approval process for any drug it chooses, so that a drug gets to market more quickly. The PRV program was created by Congress to incentivize companies to develop drug treatments for rare pediatric diseases that, because of the low number of patients and resulting small market, are typically not given the same attention as more common conditions. The owner of a PRV often sells it, as Eiger did. The net proceeds from the PRV sale were split evenly between Eiger and PRF, and PRF received approximately \$47 million!

Though drug development in gene therapy (p9) and small molecule/drug discovery (clinical trial updates, p4) will likely cost much more than \$47 million, **these new funds and your continued support guarantee progress in this exciting, innovative work towards treatments and the cure for every child and young adult with Progeria.**

PRF'S First-ever Web-based Scientific Conference a

PROGERIA RESEARCH FOUNDATION 10TH INTERNATIONAL SCIENTIFIC WORKSHOP

researching possibilities
exTENDING lives
WEBINAR VERSION



PRF's 10th International Scientific Workshop, originally intended to be held in person, pivoted to a first-ever virtual conference. This format attracted a record 377 registrants from 30 countries! Leading PRF-funded Progeria physicians, scientists and preclinical investigators, joined by families of children with Progeria, came 'together' to share and discuss the latest findings in Progeria research and hear from those they're helping: the children and their families.

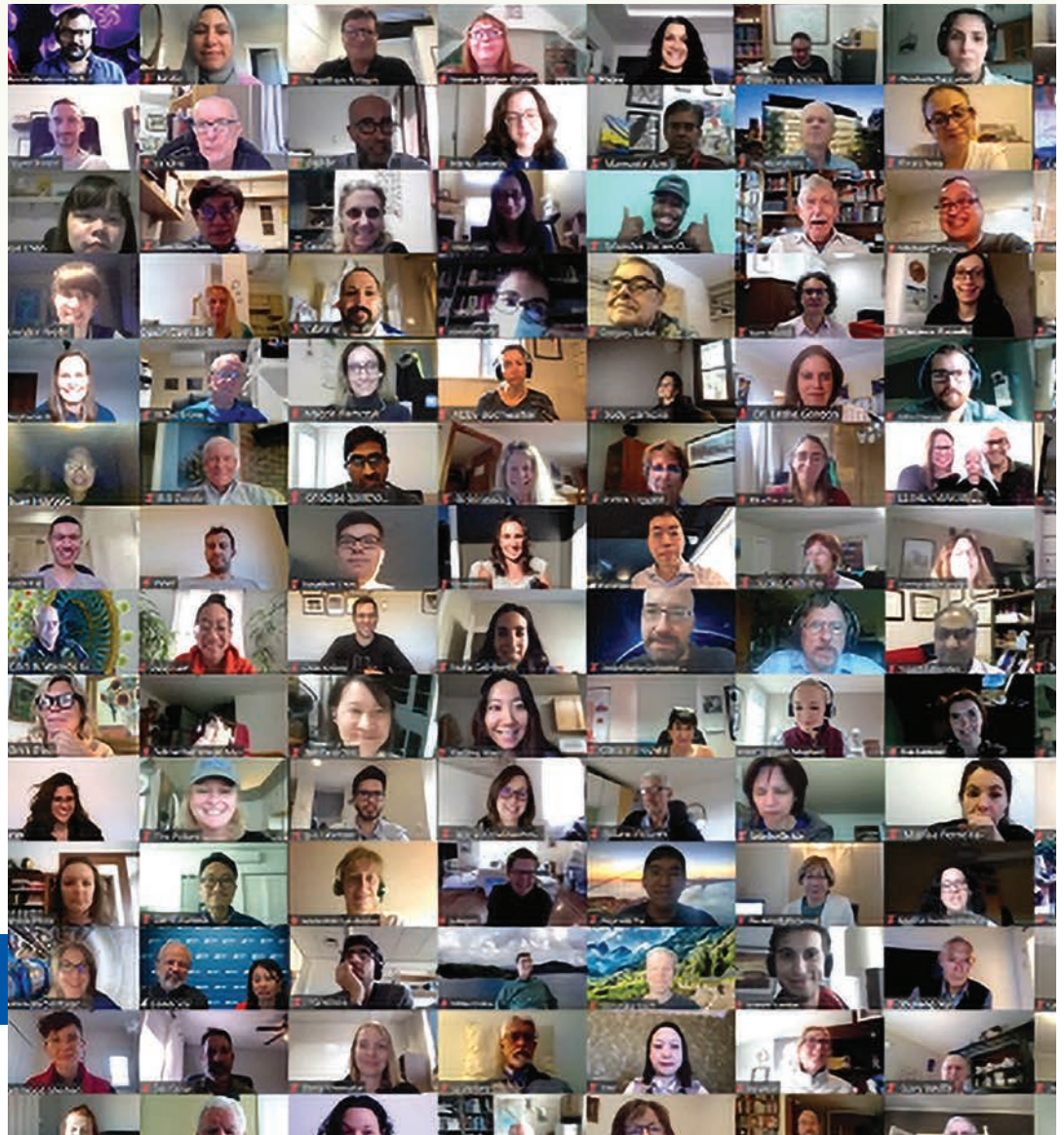
This event took place over two days, from November 2 – 3, 2020, via Zoom, and kicked off with a family panel moderated by PRF Medical Director Leslie Gordon, MD, PhD. Researchers had the unique opportunity to get a first-hand account of two families' journeys with Progeria: one with a young adult with Progeria, and the second with a young child, in a session entitled, "The Journey from No Hope, to Clinical Trials, and Entering Young Adulthood – Living Life to Its Fullest."



Family Panel Participants, above: Sammy (25 y.o.), Amerigo, and Laura Basso, Italy; below: Alexandra (6 y.o.), Cédric, and Esther Peraut, Spain



Screen shot of just a few of the 377 attendees from 30 countries



a Resounding Success!

Focusing on the most recent developments in biological and clinical research on Progeria, 16 speakers from across the care continuum delivered scientific presentations that shed light on new cutting-edge treatment investigations.

Day 1: Treatments and Trials on the Horizon

Moderator: Mark Kieran, MD, PhD, Day One Biopharmaceuticals, San Francisco, CA

This session began with a presentation on Zokinvy (lonafarnib) and its journey to FDA approval, followed by updates on the ongoing Progeria clinical trial testing the combination of lonafarnib plus everolimus. Talks then shifted to new, small molecule based therapies that target either the functional progerin protein pathway (progerinin and ICMT inhibitors), or improve various downstream damaging consequences of progerin production, such as inflammation (LINC and interleukin-6 receptor inhibitors).

Day 2: Gene and RNA Therapeutics for HGPS

Moderator: Francis Collins, MD, PhD, National Institutes of Health; Bethesda, MD

This session focused on treatments that block progerin's production at the RNA level, and at the genetic level. Genetic editing actually changes the progeria cells' DNA to a normal state so that the cell does not have Progeria anymore!

Panel Discussion: Cause and Treatments: Cardiovascular Disease in HGPS and Ageing

Moderator: Leslie Smoot, MD, Boston Children's Hospital; Boston, MA

The final session maximized communication between basic scientists and clinical investigators by exploring the newest research on the natural history of cardio-neurovascular disease in HGPS, and how we can use this clinical knowledge to improve trial outcomes and assessments. Special presentations of the first Progeria heart valve replacement surgeries showed how these advances are helping young adults with Progeria live longer, healthier lives.



We're deeply grateful to all who participated in this workshop. The support and interest in Progeria grows each year, and we look forward to the next phases of progress towards a cure, together, at the 2022 PRF workshop (see page 8 and prfworkshop.org for details).

Many of the exciting studies on new treatment strategies have extended lifespan in Progeria mice

Percent Increase in Progeria mouse lifespan compared to untreated Progeria mice

DNA base editor	140%
RNA therapeutic	61.6%
progerinin	50%
lonafarnib	24.9%

Results from the 2020 workshop were published in the journal *Aging*, available at: www.aging-us.com/article/202835.

Special thanks to our Workshop Organizing Committee...

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

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

Judith Campisi, PhD, Buck Institute for Research on Aging, Novato, CA

Mark Kieran, MD, PhD, Day One Biopharmaceuticals, San Francisco, CA

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

Lynn Doucette, Meeting Coordinator, PRF, Boston, MA

Madeline Schwartz, Kelly Edwards, Meghan Johnson, Technical Team; National Institute for Children's Health Quality (NICHQ)

And our Workshop Co-Supporter:

Associazione Italiana Progeria Sammy Basso



PRF Research Grants Program

PRF announces funding of two new grants to researchers in Spain and Italy!

Thanks to our incredible community of support, PRF has awarded two more research grants this year. Since our inception in 1999, PRF has provided \$8.7 million in funding for 80 grants to researchers in 15 countries! Our latest grant recipients are exploring novel ways to understand Progeria biology, and create pathways toward more effective treatments and the cure, as PRF continues to invest in the most promising Progeria research.



Mario D. Cordero, PhD

Institute of Biomedical Investigation & Innovation – INiBICA, Spain

“Inflammasome inhibition and polypill strategy in the treatment of HGPS”

Dr. Cordero’s project will explore key ways in which inflammation creates disease in Progeria. His previous findings show a possible role of specific inflammatory pathways affecting disease, and Dr. Cordero will examine mechanisms behind how lonafarnib might improve this inflammation. He will then expand the possible Progeria treatment profile by examining new inflammation-reducing drugs that could boost the beneficial effects of lonafarnib.



Chiara Lanzuolo, PhD

Institute of Biomedical Technologies (ITB) – CNR, Italy

“Monitoring genome structure and function’s recovery upon pharmacological treatments in Hutchinson Gilford Progeria Syndrome”

Dr. Lanzuolo is an expert in the field of DNA 3-Dimensional structure. Her group recently reported that abnormalities in DNA structure in Progeria can be found very early in disease. In this project, she will use cutting-edge technologies to understand why the DNA structure in Progeria is abnormal, and test various possible treatments’ ability to correct this early disease manifestation.

Hear from these scientists and other world-class leaders in Progeria research at next year’s International Progeria Scientific Workshop! See below for details.

THE PROGERIA RESEARCH FOUNDATION 11TH INTERNATIONAL SCIENTIFIC WORKSHOP

RACE Progeria to the Cure

May 2-4, 2022
Cambridge, MA



We’re **starting our engines** and **revving up** for

The Progeria Research Foundation’s
11th International Scientific Workshop:
RACE Progeria to the Cure!

May 2-4, 2022
Royal Sonesta Hotel
Cambridge, MA, USA



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!

REGISTER NOW!
prfworkshop.org

Breakthrough Findings in Gene Therapy Studies

continued from front cover

► **Gene Editing:** Last year, we introduced you to the work of genetic editing expert Dr. David Liu of the Broad Institute of Harvard and MIT. Dr. Liu's research was published this year – a dramatic study demonstrating that a single injection of a genetic base editor (similar but more targeted than CRISPR-CAS9) programmed to correct the disease-causing mutation resulted in **mice surviving 2.5 times longer** than untreated Progeria mice, to an age corresponding with the start of old age in healthy mice! Importantly, treated mice also **retained healthy cardiovascular tissue**, a significant finding, as cardiovascular (heart) disease is the main cause of death in children with Progeria. This amazing collaborative work was conducted by



Dr. Collins with Sam Berns, son of Drs. Leslie Gordon and Scott Berns, and the inspiration behind the founding of PRF, on stage at TEDMED 2012.

researchers at the Broad Institute, Harvard University, National Institutes of Health, Vanderbilt University, Baylor College of Medicine, University of Maryland, and PRF.*

"Five years ago, we were still finishing the development of the very first base editor," said Dr. Liu. "If you had told me then that within five years, a single dose of a base editor could address Progeria in an animal at the DNA, RNA, protein, vascular pathology, and lifespan levels, I would have said 'there's no way.' It's a real testament to the dedication of the team that made this work possible."

"To see this dramatic response in our Progeria mouse model is one of the most exciting therapeutic developments I have been part of in 40 years as a physician-scientist," said Dr. Francis Collins, Director of the National Institutes of Health and co-leader of the gene editing study.

"This has the feel of something that could someday be a true genetic cure with a single injection."

- PRF's Co-Founder and Medical Director, Dr. Leslie Gordon, quoted in an article in *STAT News/The Boston Globe*

► **RNA Therapy:** Two RNA therapeutics studies, led by researchers at the National Institutes of Health (NIH), were equally exciting. Though each study used a different drug delivery system, both studies targeted the same basic treatment strategy, inhibiting production of RNA coding for the abnormal disease-causing protein in Progeria, progerin:

One study, led by Dr. Collins, showed that treating Progeria mice with a drug named SRP-2001 **reduced the harmful progerin mRNA and protein expression in blood vessels, as well as in other tissues**. At the end of the study, the blood vessels remained strong and the mice demonstrated an increased survival of over 60% compared to untreated mice.**

The other study, led by Tom Misteli, PhD, Director of the Center for Cancer Research, National Cancer Institute, NIH, showed a **90-95% reduction of the toxic progerin-producing RNA** in different tissues after treatment with a drug called LB143.***

Furthermore, researchers found that a combination treatment with RNA therapeutics and Zokinvy reduced progerin protein levels in liver and heart more effectively than either single treatment on its own.

*Koblan LW, Erdos MR, Wilson C. et al. In Vivo Adenine Base Editing Rescues Hutchinson-Gilford Progeria Syndrome *Nature*. 2021 Jan 6.

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

***Puttaraju, M., Jackson, M., Klein, S. et al. Systematic screening identifies therapeutic antisense oligonucleotides for Hutchinson-Gilford progeria syndrome. *Nat Med* (2021).

All three studies were co-funded by The Progeria Research Foundation, and the publications were co-authored by PRF's Medical Director, Dr. Leslie Gordon.

There is still much work to be done before these gene therapy treatments can be tested safely in human clinical trials. We look forward to keeping you informed as further progress is made!

Chapter, Family and Annual PRF Events

Pivoting for Progeria: Our amazing chapter leaders and other organizers of fundraisers adjusted to the COVID-19 pandemic restrictions, switching to a virtual format whenever possible.

Although sad we could not be together, our virtual events - including an at-home edition road race and an online gala - allowed supporters from across the globe to partake in these festivities for the first time. These successful events were only possible because of your participation and generosity in the midst of the extraordinary challenges the world faced throughout most of 2020. THANK YOU for your steadfast support during these challenging times, and getting us closer to the CURE!

We're excited to now be together more – for the children, for the cure.

Worldwide support for PRF's 19th Annual International Race for Research: 2020 AT-HOME Edition!

Last September, 125 runners and walkers took to their neighborhood streets from 21 U.S. states and as far and wide as Australia, South America and Europe, to support children and young adults with Progeria.



Proud bib displayers from around the world

Save The Date – in-person AND virtually!

WE'RE BACK!!! We're so excited to be together in-person again for this year's road race! Please join us on our Peabody, MA race course (or virtually from ANYWHERE) for PRF's **20th Annual International Race for Research – Peabody & AT-HOME EDITION: September 18, 2021!** Visit PRFroadrace.org for details.



Fundraising E-fish-ently is What it's All A-Boat!

The 5th Annual Deallaney Hudson Honorary King Mackerel Fishing Tournament in November finished strong with over 40 sponsors supporting the event. The fish were jumping, and a great time was had by all!



Deallaney and friends score the catch!

Team Zoey's Turkey Trot Goes Virtual

NJ chapter supporters from near and far came together virtually for the **2020 Team Zoey Turkey Trot**. Registrants had the month of November to complete the course on the streets of their hometowns and to show their love and support for Zoey.



Zoey and friends enjoy the Turkey Trot in 2019

Golfing for Good in New Jersey

Team Zoey's 11th Annual Golf Outing last September was another successful event despite COVID-19 restrictions. While they did not have the usual full dinner and auction, 80 golfers had a wonderful day on the course, followed by an outdoor cocktail hour.

This year's outing will be held on September 20 – so golfers, get ready for another amazing year!



Zoey, her grandfather, PRF Board member John Marozzi, and brothers Gavin and Aidan enjoy the afternoon reception at Preakness Hills Country Club



Lindsay Ratcliffe (in red), with her friend, Kaylee, and Flat Rock police officers

A Different Kind of Miles for Miracles

This year's Miles for Miracles took place in May, and due to COVID-19, pivoted from the usual walk to a Road Rally with a driving scavenger hunt – so creative! The Ratcliffes' amazing community of supporters in Flat Rock, MI came together after the scavenger hunt for socially-distanced raffles, refreshments and prizes for best team costumes. They 'rallied' this year – and can't wait to return to the walk format in 2022!

10th Annual Make-a-Splash Event Goes Virtual

Nathan and Bennett's 10th Annual Make-a-Splash event hosted by our PA East Chapter was held virtually in 2020 and still garnered tremendous support from the *Fighting for their Future* community.

Leading up to Make-a-Splash, Nathan and Bennett collected coins from neighbors to help with

the national coin shortage and raised a total of \$3,828 to donate to PRF. Way to go, boys!

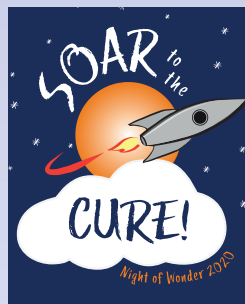


Always there to support PRF, the Falcone family masks up for this year's family photo

TEAM PRF Finishes Strong at the 2020 Falmouth Road Race AT-HOME Edition

Last August, 15 TEAM PRF members went the distance, running a 7-mile local race course after the in-person race went virtual.

Awesome job, runners!



We SOARED to new heights at PRF's 2020 Night of Wonder Gala – SOAR to the CURE!

WHAT A NIGHT! PRF's signature gala, originally scheduled in Boston for April 2020, went virtual,

in December. If the chat room, auction bidding and *Circle of Hope Society* (\$250 and up donations) were any indication of the evening's success, this SOAR to the CURE event was astronomical, fueled by your love for the children, and our collective mission to cure them.

Special thanks to our major sponsors:

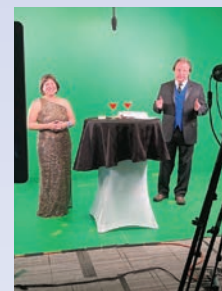
BIG BANG: The Morrison Family

SUPER NOVA:

Leslie Gordon & Scott Berns
Debbie Mendelson Ponn

AURORA BOREALIS:

Alice & Lew Berns
Boston Bruins Foundation
Marlene & John Marozzi
Linda and Robert Mendelson
Linda & Larry Mills
New England Patriots & The Kraft Family
Tom O'Brien
Putnam Investments



PRF Executive Director Audrey Gordon and auctioneer Paul Zekos emcee the 2020 Night of Wonder gala and auction from a local studio.



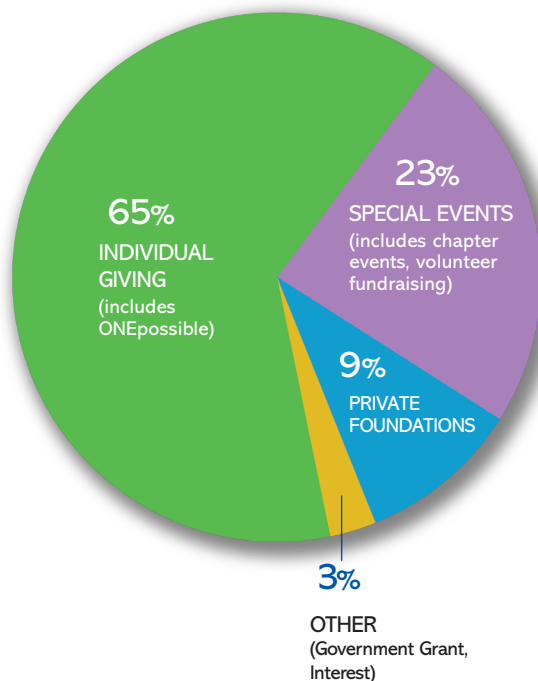
The upcoming gala on April 9, 2022 will once again bring our supporters back in person! See back cover for details.



Audrey and Paul celebrate the evening's success with "Cosmo-naut" drinks.

PRF 2020 Annual Report

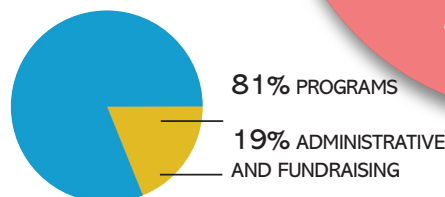
INCOME ANALYSIS - 2020



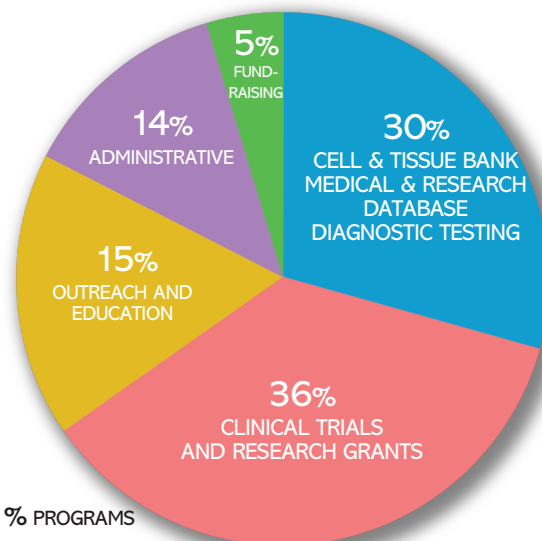
TOTAL REVENUE
\$2,227,158

TOTAL EXPENSES
\$2,591,622

PROGRAM RATIO



EXPENSE ANALYSIS - 2020



Net Assets: \$6,532,044 - much of these funds are designated for future clinical trial and drug development costs.

Core PRF Activities That Support Our Mission

2020 ACTIVITY	PRF PROGRAM	CUMULATIVE ACTIVITY (1999 - 2020)
28 children from 15 countries - a record high!	International Patient Registry (includes unconfirmed cases)	319 children with Progeria from 65 countries and all continents have registered with PRF
86 cell lines sent to 20 labs in 8 countries; 4 children were tested	Cell & Tissue Bank Diagnostic Testing Program	1,264 cell lines sent to 197 teams in 25 countries; 152 children tested through PRF
4 children enrolled from 4 countries	Medical & Research Database	187 enrolled from 51 countries and 1 US territory
Translation of Progeria handbook in Chinese began	Progeria Treatment Guidelines Handbook	Available in four languages - English, Spanish, Portuguese, Japanese; sent to families and their doctors in 65 countries and 1 U.S. territory
Everolimus + lonafarnib Trial, final visits continue; Trials led to FDA approval for lonafarnib!	Clinical Trial Funding & Co-coordination	98 children from 38 countries have participated in PRF clinical trials, initiated in 2007
7 research grants awarded	Research Grant Funding	80 grants to 54 labs in 15 countries; funding total: \$8.7 million
373 registrants from 30 countries attended virtually	International Scientific Meetings	13 meetings: 10 general workshops and 3 subspecialties
11 events, 80 new volunteers	Volunteer Program	Nearly 5,000 volunteers worldwide have helped raise awareness and funds
9 families requiring an interpreter traveled to the U.S.; one more language added to program material translations (Persian/Farsi)	Translations Program	PRF's program and medical care materials are translated into 39 languages
1,310 media mentions (online publications)	Public Awareness	8,500 media mentions (online publications) - resulting in millions reached worldwide

Growing Progeria Research: the KEY to the CURE!



This year, 100% of the funds raised through PRF's annual ONEpossible campaign will be devoted to research activities, as PRF 'plants the seeds of research' in the most cutting-edge areas of science. With your help, we will cultivate the CURE!

Thanks to our devoted community of donors, Progeria researchers are able to contribute to PRF's tremendous progress toward treating, and one day CURING, children and young adults with Progeria. Here are just a few expressing why they're so deeply committed. Visit progeriaresearch.org/meet-the-researchers to see more.

"While all of us on the Boston Children's Hospital (BCH) team are committed to science that underlies Progeria, we do it ALL for the kids! There is nothing more gratifying than being in clinical research, where we have the ability to weave together scientific principles and concepts and also meet these wonderful children and their families."

– **Dr. Catherine Gordon**, Progeria Clinical Trial Team Member, Endocrinologist and Bone Health Specialist, BCH



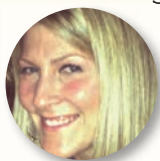
"We are convinced that, as in many other diseases, a combination of drugs will be necessary for the cure. Finding the right combination requires a great effort of researchers – we and colleagues worldwide are working hard!"

– **Giovanna Lattanzi, PhD**, PRF Research Grantee from CNR Institute of Molecular Genetics Unit, Bologna, Italy



"I'm forever grateful to be a part of a special team here at BCH... The work and effort that everyone has invested for these children, young adults, and their families, inspires me every day to continue pushing forward to find a cure. The resilience and strength that these kids and families demonstrate as they adapt to new and often unpredictable circumstances is extraordinary."

– **Christine Dube MS, BSN, RN**, Progeria Clinical Research Nurse, BCH



"I love being able to work with the children and their families who come to Boston Children's Hospital for treatment. The most apparent similarity I have noticed about the children is that they don't let the disease take control of their lives. If something is a challenge because of Progeria, they find a way to overcome it. The children are resilient, brave, and hopeful which is a true testament to their characters."

– **Tim O'Toole**, Trial Coordinator for the Progeria Clinical Trials, BCH



"It's inevitable that we will find a cure, eventually... we will never stop, and the people who join the Progeria family... are in it for good."

– **Leslie Gordon MD, PhD**, PRF Co-Founder and Medical Director



From all of us at PRF, as well as the children and their families, THANK YOU to everyone who donated to our 2021 ONEpossible campaign. We crushed our goal, raising over \$120,000! Each of you is ONE that will make the cure POSSIBLE!



PRF On The Move!

Breakthroughs in Gene Editing

In July, PRF Medical Director, Dr. Leslie Gordon spoke on a panel at the virtual **STAT Breakthrough Science Summit** with her colleagues, Dr. David Liu of the Broad Institute of MIT and Harvard, and Dr. Francis Collins, Director of the National Institutes of Health, as well as 25-year-old PRF spokesperson, Sammy Basso. Their panel covered the stunning results of genetic editing in correcting the gene responsible for Progeria in mice, and was hailed as a conference favorite - not only for its groundbreaking potential to serve as the first iteration of a technology that could one day be the cure, but also for Sammy Basso's charm when he shared that, "Now, we are not alone [...] and every scientific discovery is like a party for these families."



Top: Leslie Gordon, Sammy Basso, David Liu; Bottom: STAT Panel moderator Meghan Molteni, Francis Collins

"There's a relentless focus and commitment inspired by patients like Sammy."

— Dr. David Liu

"In 2003, nobody would have imagined we'd have the elegant [technology] that David Liu and his lab have made possible, where you do a find-and-replace on DNA like you would in your word processor."

— Dr. Francis Collins

Expertise in Rare Disease Clinical Trial Design and Execution

In March, Dr. Leslie Gordon also presented virtually to hundreds of MD's, PhD's, and regulatory FDA staff, including the Rare Diseases Team at the FDA's Center for Drug Evaluation and Research, detailing her journey leading 13 years of clinical trials that brought Zokinvy to market as the first-ever treatment for Progeria, and on its profound impact on the Progeria community.

PRF's Executive Director Audrey Gordon Featured on Great.com podcast

In June, 2021, Ms. Gordon was interviewed in a podcast hosted by Great.com, a philanthropic organization that aims to help nonprofits raise awareness by giving them a platform to share their story. Audrey spoke about PRF's founding, its rich history bringing Progeria from total obscurity to the first treatment's FDA-approval, and much more. To hear the 25-minute interview, google: "Great.com Talks With #254."



Sam Berns' Eternal Legacy

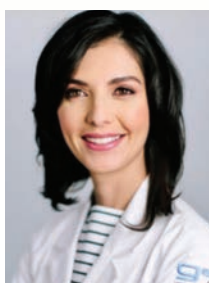
At 42M views on TED.com, Sam Berns' inspiring **'My Philosophy for a Happy Life'** has now surpassed Bill Gates' epidemic-related TED talk. Between TED.com and TEDx.com, Sam's talk has a cumulative 89M views, which includes views on the TEDx MidAtlantic channel, to become the eighth most-viewed talk on the TED channel. It's been translated into 39 languages! Watch for news of a special celebration when the talk – which has inspired millions around the world to live life to the fullest – hits 100 million views!



3 Cheers for PRF's Miracle Makers!

PRF's research breakthroughs are possible, in part, because of our wonderful Miracle Makers – those who give their time, energy and talent in a variety of ways to raise awareness and funds in support of our mission. Miracle Makers include fundraiser organizers, translators, photographers, Ambassadors, and much more.

Here are just a few of our most recent heroes:



Dr. Melania Abreu, a Mexican Geneticist, helps patients in Mexico to access the PRF Genetic Testing Program. A confirmed diagnosis of Progeria is the first step in getting the children the unique care they need. Thank you for giving these children a chance at longer, healthier lives, Dr. Abreu!



Classmates for a cause! These amazing kids, all friends of Verona, NJ, resident **Zoey Penney**, have put their hearts and souls into fundraising to help their friend since 2016. Dozens of events and many lasting memories later, the members of 'VeronaRocks' are celebrating five years making a difference for Zoey. This energetic group of friends has raised funds through bike ride events, fashion shows, car washes, food drives, holiday caroling, and lots more – **WOW, they ROCK!**

YOU can be a Miracle Maker, too!

Visit progeriaresearch.org/be-a-miracle-maker to learn how.

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

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



Ashanti
18 years old
from England



Aahan
7 years old
from India



Emily
13 years old
from the United States



Essau
11 years old
from Tanzania

In Loving Memory...



Rupesh
23 years old
from India



Rania
16 years old
from France



Omaima
14 years old
from Pakistan



Nethi
13 years old
from Thailand



Iryna
10 years old
from Ukraine

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



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

RETURN SERVICE REQUESTED

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

NON-PROFIT
US POSTAGE
PAID
NEW BRUNSWICK, NJ
PERMIT NO. 1512



*Fresh off a Zoom class, 9-year-old Enzo
is all smiles as he gets his school work done!*



SAVE THE DATE!

Night of Wonder 2022 - April 9th in Boston

You ALL have a **superpower**: supporting children with Progeria. Unite with your fellow superheroes at PRF's signature **Night of Wonder** gala in April 2022.

Swoop in for our **Marvel-ous** live auction, gourmet food, and fun entertainment, and hear directly from the children you're helping.

Join us for our Night of Wonder - where **capes** are optional, but **big hearts** are a must!

Visit **prfnow.org** for details.

Questions? Email **prfnow@gmail.com** or call **978.548.5303**.



NIGHT OF WONDER 2022

Come find us:



[facebook.com/
ProgeriaResearch](https://facebook.com/ProgeriaResearch)



twitter.com/progeria



[instagram.com/
progeriaresearch](https://instagram.com/progeriaresearch)



[youtube.com/user/
progeria123](https://youtube.com/user/progeria123)