Progeria Research Foundation®

WWW.PROGERIARESEARCH.ORG • SEPTEMBER 2018

news

FANTASTIC NEWS! Study Published in JAMA Finds Treatment Extends Survival

In a spectacular development, a new study published in The Journal of the American Medical Association (JAMA) shows that treatment with lonafarnib extends lifespan in children with Progeria. This is the first evidence that lonafarnib alone improves survival for this fatal disease.

On the heels of the JAMA study, PRF and Eiger BioPharmaceuticals announced they will partner to pursue FDA approval of lonafarnib so that children may access the drug with a prescription instead of through a clinical trial.

Read about this groundbreaking study and the PRF-funded clinical trials that make this progress possible on pages 4-7.

Meghan, then six years old, was one of the first children to enroll in the clinical trial in 2007 that led to the recent breakthrough on lonafarnib. She is pictured on the left getting a lesson in how to take lonafarnib, holding the drug for the very first time. On the right, after ten years of taking lonafarnib, Meghan is finishing strong in PRF's Annual Race for Research in 2017.

A Message from the President and Executive Director:

Dear Friends,

PRF's mission is clear: to find life-changing treatments and the cure for Progeria. Every day, our research makes strides towards this goal – thanks to YOU, our steadfast and dedicated supporters.

The past year has been an incredibly active year for PRF, full of exciting progress. This newsletter highlights many of these notable achievements:

- In April 2018, a study published in JAMA led by PRF's Medical Director, Dr. Leslie Gordon, and funded by PRF found that lonafarnib extends survival in children with Progeria – a remarkable feat that demonstrates our work is resulting in longer, stronger lives for children with Progeria.
- In May 2018, PRF and Eiger BioPharmaceuticals partnered to pursue FDA approval for lonafarnib as a treatment for Progeria, marking the first Progeria therapy to be submitted for FDA approval.
- Our two-drug clinical trial is moving full speed ahead, with new children enrolling and some now coming back to Boston to complete their final trial visits.
- We "Rocked the Cure" at Night of Wonder 2018, raising a record-breaking $580,000 for new research.

None of this progress would have been possible without all of you. Your donations, fundraising events and volunteer efforts help PRF raise the funds needed to fuel our research, allowing us to continue to strive for the next breakthrough in our race to cure Progeria.

As PRF's Executive Director, I have the privilege of meeting children and families touched by Progeria. As I tell many people, this is the best part of my job. I meet extraordinary families filled with love, hope, optimism and trust in our mission. Families inspire us every day, and I am proud to be playing a part in this fight. I pledge to continue this progress, helping raise awareness of this rare disease and bringing together the people who can help us find the CURE!

Thank you all for your support,

Meryl Fink

Meryl Fink, Esq.
President and Executive Director

Meryl with 8 year old Aaditya from India during his trial visit to Boston Children's Hospital in May 2018.

PRF's Vision:
A world in which every child with Progeria is cured.

PRF's Mission:
To discover treatments and the cure for Progeria and its aging-related disorders, including heart disease.

Progeria is a fatal, "rapid aging" condition that afflicts children, who die of heart disease at an average age of 14 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.

PRF-Funded Study Finds Lonafarnib Increases Lifespan for Children with Progeria

Next step: seeking FDA approval.

The best news yet...

The study using lonafarnib, a type of farnesyltransferase inhibitor (FTI), shows extension of lifespan in children with Progeria. Authors compared children who received lonafarnib alone to those similar in age, sex and continent of residency who were not part of the clinical trial and therefore did not receive lonafarnib.

The results demonstrate that treatment with lonafarnib alone compared with no treatment was associated with a significantly lower mortality rate (3.7% vs. 33.3%). The study, which was **funded by The Progeria Research Foundation**, was published April 24, 2018 in *The Journal of the American Medical Association** (JAMA), one of the most prestigious scientific journals in the world.

The research was led by PRF's Medical Director, Leslie B. Gordon, MD, PhD, and conducted by PRF-funded investigators from Brown University, Boston Children's Hospital, Harvard Medical School and Boston University.

**Gordon et. al., Association of Lonafarnib Treatment vs No Treatment With Mortality Rate in Patients With Hutchinson-Gilford Progeria Syndrome, JAMA, April 24, 2018 Volume 319, Number 16*

Zoey is an active 8 year old from New Jersey. She has been taking gymnastics for over 3 years and loves training, performing and competing with her fellow teammates. Zoey has been taking lonafarnib since joining the PRF-funded clinical trials 4 years ago.

A link to PRF's press release about the study and more details of the findings and their impact can be found at www.progeriaresearch.org

JAMA
The Journal of the American Medical Association

"This study published in JAMA shows evidence that we can begin to put the brakes on the rapid aging process for children with Progeria. These results provide new promise and optimism to the Progeria community."

- Leslie Gordon, MD, PhD, co-founder and Medical Director for PRF, and lead study author.

"My lab did some of the original research on cellular and mouse models that showed potential benefit of this class of drugs for Progeria. It was encouraging to see those results translated into a clinical trial. Yet demonstrating effectiveness of treatments in this small population of children with this rare fatal disease is a major challenge. Thus, I'm particularly encouraged by these latest findings."

- Dr. Francis S. Collins, MD, PhD, National Institutes of Health Director

THE PATH TO Finding the Cure for Progeria

What is Progeria?

Children with Progeria die at an average age of just **14 years**.

{HGPS}

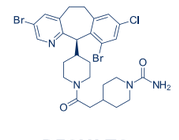
Progeria, also known as **Hutchinson-Gilford Progeria Syndrome (HGPS)**, is a rare, fatal genetic condition of accelerated aging in children.

There are **no** current, FDA-approved **treatment options**.

2018: Journal of the American Medical Association

GLOBAL STUDY
Finds Treatment with Lonafarnib increases Survival in Children with Progeria Syndrome

- Study demonstrated a link between drug therapy and increased survival
- Lower mortality rate after a median of 2.2 years follow-up
- Treatment with lonafarnib alone compared with no treatment was associated with a significantly lower mortality rate (3.7% vs 33.3%)



RESULTS:
Study shows that treatment with the drug, lonafarnib, helped increase survival in children with Progeria

JAMA
The Journal of the American Medical Association

PRF's Unprecedented Progress Against a Fatal Disease

1999 — **The Progeria Research Foundation (PRF) was founded** in response to the complete lack of progress being made to help children with Progeria

2003 — Discovery of **Progeria Gene**

2007 — **First-ever clinical drug trial** for a potential treatment

2012 — Clinical trial with lonafarnib reveals **improvement in cardiovascular disease**

2016 — **Ongoing new trial** adds a drug that attacks progerin from another route

2018 — JAMA **Study published in JAMA** shows decreased mortality rate when using lonafarnib as a treatment option

PRF and Eiger BioPharmaceuticals Partner to Pursue FDA Approval of Lonafarnib

Wonki from South Korea and Miguel from Columbia were all smiles while in Boston for their first trial visits in 2014, when they began taking lonafarnib.

On the heels of the study published in April 2018 in *JAMA*, PRF partnered with Eiger BioPharmaceuticals to pursue FDA approval of lonafarnib as a treatment for Progeria – the first therapy to be submitted to the FDA for this purpose. Lonafarnib is not currently approved for use outside of clinical trials. **If approved, children may access this drug by prescription instead of having to do so through a clinical trial**. This would allow PRF to devote more resources to new trials in order to explore additional treatment options and ultimately discover the cure.

EIGER BIOPHARMACEUTICALS

This road towards FDA approval of lonafarnib, in partnership with Eiger, is made possible by the success of all of PRF's research programs:

- **The PRF International Progeria Registry** identified children who went on to receive lonafarnib as part of our clinical trials.
- Only children with a definitive diagnosis of Progeria can enroll in the trials, and the **PRF Diagnostics Testing Program** provided the diagnosis for many trial participants.
- Analysis of medical records gathered through the **PRF Medical & Research Database** provided critical health data as children entered the lonafarnib trials.
- Cells from the **PRF Cell & Tissue Bank** were instrumental in the gene discovery and studies of lonafarnib as a potential treatment.
- PRF-funded **research grants** showed that lonafarnib had a positive effect on Progeria cells and in Progeria mouse models, paving the way towards clinical trials.
- Results from PRF-funded **clinical trials** showed that lonafarnib improved weight gain, bone structure, vascular disease, and increased lifespan in children with Progeria.

Two-Drug Trial Continues

Progeria clinical drug trials are the culmination of years of research focused on discovering treatments and the cure for children with Progeria. Your support has allowed PRF to coordinate and fund our third experimental treatment in eleven years – a remarkable feat for a relatively small organization focused on a rare disease. Children in this trial take two drugs: lonafarnib, the medication that gives the children stronger hearts and longer lives; and everolimus, a drug that shows promise in laboratory studies. The trial has two phases. Phase 1, completed in June 2017, determined the best dose of everolimus to give the children. Phase 2 is occurring now, and will determine whether the combination therapy is more beneficial than lonafarnib alone.

It has been a busy year! Since last fall, more than 40 children have travelled to Boston from 20 countries, for a total of over 60 children currently enrolled in this trial. Enrollment will be completed this year – an amazing accomplishment!

Miguel (l) from Columbia and Danielle Kim (r) from the Philippines, both 12 years old, were together in Boston in January 2017 when they enrolled in the two-drug trial. Coming to Boston in pairs gives children with Progeria and their families an unique opportunity to meet in person.

Mio, 13 years old, traveled from Japan to Boston to participate in the two-drug trial.

Trial Expansion Update:

As children complete their last visits for the two-drug trial, PRF has made sure they will still be able to receive lonafarnib. Usually, clinical trials run their course and the patients are taken off all the drugs until the data is analyzed to see if the drugs are helping - that can take years. PRF and the Boston Children's Hospital team have ensured that will not happen here, by adding up to 4 ½ years of lonafarnib treatment. This extension allows additional time to explore the full benefits, including lifespan, and track any long term side effects of lonafarnib. Moreover, it allows us to continue exploring additional treatment options while giving the children the opportunity to continue receiving this proven treatment.

Progeria clinical drug trials are the path to the cure.

PRF is funding all aspects of the trial, including travel, lodging, testing and staff. We must raise over $1 million each year just to fund these trials, and we rely on the generosity of our wonderful contributors to PRF's ONEpossible and end of year campaigns, as well as participants in the many special events that occur throughout the year around the world.

THANK YOU for making this trial – and progress toward the cure for Progeria - POSSIBLE!

In Loving Memory...

Lucy
8 years old,
from the United Kingdom

Rafaely
12 years old,
from Brazil

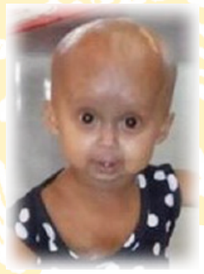
Nova Mae
8 years old,
from the Philippines

Elias
12 years old,
from Mexico

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

Natsuki
17 years old,
from Japan

Luis Javier
10 years old,
from Guatemala



Eloysa
2 years old,
from Brazil

Magaly
16 years old,
from Columbia

PRF Announces Updated Grant Program

Since its inception in 1999, PRF has awarded 71 grants to researchers spanning 14 countries to advance the field of Progeria research. The work produced by these scientists has shaped the field and led to important discoveries about Progeria, heart disease, and aging.

(Top row l to r): Tom Glover PhD, Vicente Andrés Garcia PhD, Tom Misteli PhD, Maria Eriksson PhD, W. Ted Brown MD, PhD, Frank Rothman PhD (emeritus), Bryan Toole PhD (chair)

(Front Row l to r): Monica Kleinman MD, Christine Harling-Berg PhD, Judy Campisi PhD, Leslie Gordon MD, PhD, Marsha Moses PhD

Over this past year, PRF's 11-member Medical Research Committee (MRC) held two meetings to revise PRF's grant program. Based on these discussions, PRF has implemented a two-step granting process: a letter of intent, followed by an invitation for selected full proposals. In addition, a member of the MRC will conduct regular advisory meetings with each funded investigator, to discuss progress, pitfalls, changes in project direction, and provide assistance in any way possible to facilitate successful research. These changes will assure that PRF's grant program remains on the cutting edge of science and continues to propel us towards new treatments and the cure.

For more details on the application process and the grants PRF has funded please visit:
www.progeriaresearch.org/research_funding_opportunities

Researchers, Still Time to Register!

THE PROGERIA RESEARCH FOUNDATION PRESENTS

9TH INTERNATIONAL SCIENTIFIC WORKSHOP

September 20-22, 2018
Royal Sonesta Hotel, Cambridge, MA USA

Meeting Mission:
To promote collaboration between basic and clinical scientists toward progress in Progeria, cardiovascular, and aging research

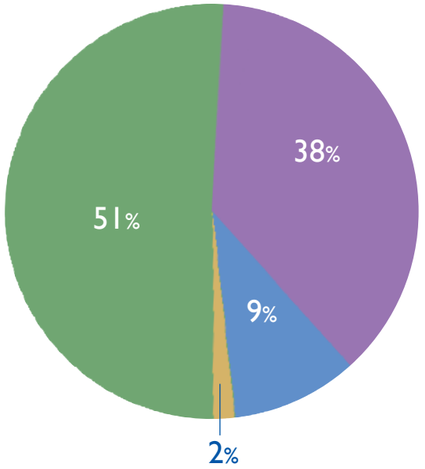
Featured Presentations:
Progeria Families Inspiring the Research Community
Plenary Session by Eric Lander, Broad Institute, MIT
Presentation by Francis Collins, NIH Director

Session Topics Include:
Disease Mechanisms in Progeria, Heart Disease and Aging
Therapeutic Approaches to Progeria
Spotlight on Genetic Therapies

Register at *www.prfworkshop.org*

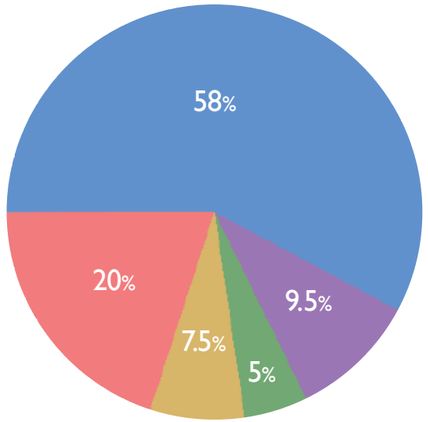
Questions? Contact us at
workshop@progeriaresearch.org
or call 978 535-2594

PRF 2017 Annual Report



REVENUE 2017

INDIVIDUAL GIVING (includes in-kind donations)	$1,497,241
SPECIAL EVENTS (includes PRF's gala, chapter events, other volunteer fundraising, and ONEpossible campaign)	$1,123,766
PRIVATE FOUNDATIONS	$279,005
OTHER (Interest, Store Items)	$58,315
TOTAL REVENUE	$2,958,327



EXPENSES 2017

RESEARCH PROGRAMS Clinical Trials, Diagnostic Testing Medical & Research Database, Cell & Tissue Bank	$1,847,965
RESEARCH GRANTS	$626,671
ADMINISTRATIVE	$303,134
PUBLIC AWARENESS	$242,106
FUNDRAISING	$154,027
TOTAL EXPENSES	$3,173,903

NET ASSETS:
$4,178,941*

*Much of these funds are designated for future clinical trials and drug development costs

PROGRAM RATIO SUMMARY

CHARITY NAVIGATOR ★★★★ Four Star Charity

SEAL OF TRANSPARENCY GOLD GuideStar

Night of Wonder 2018:
A Rockin' SUCCESS!

Our signature gala and auction rocked the house, raising a record-breaking $580,000! This year's theme was **Rock the Cure**, with 450 PRF supporters celebrating the historic "hits" PRF has achieved, while seeking to ensure we keep "climbing the charts" to the cure.

PRF's Youth Ambassador 17-year-old Meghan Waldron stole the show with her speech, sharing the importance of PRF's work and urging everyone to contribute to the **Circle of Hope** so that the ***hope*** of a cure can become a reality:

"I want you all to know that I believe I wouldn't be able to do all the things I love if it weren't for the treatment. I feel that it's given me a healthier and longer life, and for that I am truly thankful."

PRF will use the funds raised at Night of Wonder 2018 to find new treatments that may help the children even more than lonafarnib alone. Thank you to our sponsors, auction donors, advertisers, volunteers and guests – **We sure do make beautiful music together!**

This phenomenal event is held every two years, so we hope to see you at Night of Wonder 2020!

Andrea Hayward is all smiles as she places the winning bid on one of the many fantastic auction items.

Gala co-chairs Martha Holden (seated, front) and Mary Bellavance (directly behind Martha) enjoyed the evening – and the culmination of all their hard work – with their spouses and friends.

A special thank you to our major sponsors:

The Morrison Family

Leslie Gordon & Scott Berns

Debbie Mendelson Ponn

(pictured above on the far left, with family and friends)

PT Sanders

Science and Medicine (SAM) Award winner John Seng celebrates on the dance floor with his wife Christine.

Chapter, Family and Annual PRF Events

Special events are the community glue that holds together thousands of people each year, all of whom have one goal: to help cure Progeria. They participate in road races, tournaments of all kinds, car shows, pool parties, and more for children with Progeria. THANK YOU ALL for your special support.

Another strong finish for Team PRF at the Falmouth Road Race

You could see TEAM PRF from a mile away in their neon orange shirts at the 2017 Falmouth Road Race. PRF's largest team to date (18 runners – wow!) ran past thousands of spectators along the 7-mile rigorous race route, raising awareness and funds for Progeria research. Congratulations, team!

We welcome runners nationwide to participate in marathons and other races on behalf of children with Progeria, and will work with you to get a bib number if needed.
Contact Michelle at mfino@progeriaresearch.org to help win the ultimate race – for the cure!

16 Years Strong: Race for Research continues to inspire our local community

The Annual International Race for Research is the biggest and longest-running road race in PRF's hometown of Peabody, MA. Last September's race welcomed two very special guests: **Meghan Waldron**, longtime race runner and PRF's Youth Ambassador, and **Sammy Basso**, here from Italy to enroll in the two-drug clinical trial. We appreciate their motivating speeches at the starting line, and all our local supporters who join in each year!

Sammy (l) and Meghan (r) welcome the runners and walkers before the start of the 2017 race.

The Falcone family is all smiles posing poolside!

Make A Splash!

Always a fun event for both kids and adults, the 7th annual Make-a-Splash pool party in support of our PA East Chapter was the biggest yet! Costume characters, log rolling, magic, games, music, amazing auction items and more, this event is a great way to end the summer and support Nathan and Bennett.

HatsON for Progeria is now in its 6th year!

In our widest-reaching year yet, students and companies in 27 states and 21 countries donned their favorite hats in April for PRF's annual version of Dress Down Days. We love to see them sharing photos on Facebook, too! This event is so popular, we've added a fall date of October 19th – contact Lynne at *lamackenzie@progeriaresearch.org* to join in on the fun then, or next year on March 9th!

PPG employees with Cam and his dad Jason. PPG participates in HatsON every year and doubles their impact by matching all gifts!

HaTs ON for PROGeRia

Laura Marozzi Penny, Zoey's mom, with sponsors and organizers from Dynamic Speed and Fitness.

1st Annual Team Zoey - Verona Cornhole Tournament a sellout!

It began as a conversation at the gym and escalated to a charitable tournament that sold out in under two hours – wow!

"We saw a Cornhole tournament on ESPN and said 'hey, we could do that for charity,'" said one of the organizers, Shawn Luftglass. With 32 teams and dozens of sponsors, he describes the event as a "true team effort" from residents and the business community. We can't wait for next year's event!

Have you heard about Cornhole? It's a super fun lawn game in which players take turns throwing bags of corn at a raised platform with a hole in the far end, earning points for landing on the platform and through the hole. Why not organize your own "Cornhole for a Cure" tournament?!

2nd Deallaney Hudson King Mackerel Tournament makes it official: another fantastic annual event for PRF!

Thanks to the many people involved in this event – participants, sponsors, donors, volunteers, and t-shirt purchasers – everyone had a great time and raised awareness and funds for Progeria research. It takes a (fishing) village!

Deallaney (front left) poses with the winners.

Do we have your email address? We send notices of special events in your area so you don't miss out on all the fun.

Do you want to start your own PRF event tradition?
Contact us at info@progeriaresearch.org to sign up for our mailing list and get more involved TODAY!

Zach Attack Bowl-a-Thon

Every lane was full of Zach fans wearing their red "Keep Calm and Zach On" t-shirts! This sold-out Kentucky Chapter event is a blast that bowlers look forward to each year. "We are so grateful to everyone who shows up, year after year, as the research they help fund brings more and more good news for Zach", says mom Tina.

Zach with his well-deserved trophy, surrounded by volunteers and supporters.

First-rate support from first responders at MI Chapter's 13th MILES FOR MIRACLES

The rain couldn't dampen the spirits of steadfast supporters of this fantastic event that features a walk, raffles, music, food and loads of love! A huge turnout of local firefighters and other first responders joined Lindsay, her parents, friends and family to raise money for the clinical trial Lindsay is enrolled in.

Lindsay with her firefighter friends.

Miracle Makers

Volunteers making a difference in the lives of children with Progeria.

This very special group of people raises awareness by sharing our mission, helps defray costs by donating their time and talents, and raises critical funds through handmade craft sales, wedding and birthday fundraisers, and lots of other creative ways. You can become a Miracle Maker, too – visit *progeriaresearch.org/miracle_makers* to learn how!

Friends Forever

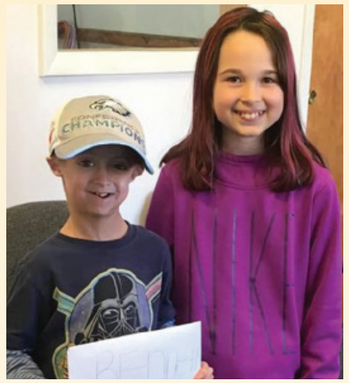
Enzo's schoolmate Charlotte is a determined 6 year-old with a huge heart. Charlotte started her own fundraising project, selling hand-made bracelets. Her project went viral and she raised over $400 for Team Enzo – now that's what friendship is all about!

Talented Photographer Takes Aim for PRF

For the past year, Gleb Budilovsky has volunteered his time and talents to PRF to photograph dozens of children who are in Boston for the clinical trials. Gleb is always eager to help, and his photos capture the special personalities of the children!

A Selfless Act of Friendship

Birthdays are special, especially for kids who look forward to the presents. But for Chloe's 9th birthday, it was an opportunity for her to do something for her friend, Bennett. Instead of gifts, Chloe asked people to make donations to PRF's PA West Chapter. Thank you Chloe and friends for raising $250 for The Progeria Research Foundation!



DMB Fans Unite for PRF

Back in 2014, a group of Dave Matthews Band (DMB) fans designed and sold hundreds of Kit Kat Sam shirts after seeing Sam Berns' special relationship with band members in the HBO documentary ***"Life According to Sam."*** All t-shirt sale proceeds were donated to PRF, and to this day, you can see the blue Kit Kat Sam shirts in the crowd at many DMB shows! This year, to further honor Sam and celebrate the shows taking place at Sam's and one of the band's favorite venues – the Gorge – they designed another t-shirt to benefit PRF. Many thanks to organizer Nathaniel Shoshan and all who have bought, and proudly wear, these t-shirts. #KitKatSam #CureProgeria #LiveLikeSam

A group of Kit Kat Sam fans at the Gorge in Washington, and this year's t-shirt.

PRF *ON THE MOVE*

Board of Directors Holds Strategy Summit in Boston

This dedicated group met in the fall of 2017 to drive PRF's strategic planning. The Board developed a comprehensive plan that will be the blueprint for future organizational and program growth, all to propel us towards fulfilling our mission to cure Progeria. The day was made extra special by a visit with Sammy Basso and his parents from Italy, who were in Boston for Sammy to participate in the two-drug clinical trial.

(Top row l-r) PRF Executive Director Meryl Fink; Paula Kelly, Karen Ballack, PRF supporter Miss Skoom, John Marozzi; Kim Paratore, Liza Morris, PRF Development Director Audrey Gordon, Tom O'Brien. (Bottom row l-r) Amerigo Basso, Laura Basso, Sammy Basso, PRF Board Chair Scott Berns, PRF Medical Director Leslie Gordon.

Meryl Fink next to the beautiful portrait of Meghan Waldron, PRF Youth's Ambassador, on display at the Broad Institute.

Executive Director Presents at Broad Institute's 2018 Rare Disease Day Seminar

Rare Disease Day takes place each year in February and events are held around the world, providing a forum where the community of children and families affected by rare diseases join together to raise awareness and garner support for their research efforts. PRF Executive Director Meryl Fink spoke in Cambridge, Massachusetts at the Broad Institute's Rare Disease Day seminar, which also featured the traveling art exhibit "Beyond the Diagnosis" showcasing portraits of children with rare diseases.

Highest 4-Star Rating Achieved For a Fifth Consecutive Year!

We are thrilled and honored to be among the top 9% of evaluated charities to reach this 5-year distinction. This highest possible rating from the leading charity evaluator in America verifies that PRF exceeds industry standards and executes our mission in a financially efficient way. Thank you all for helping us achieve this impressive milestone!

CHARITY NAVIGATOR ★★★★ Four Star Charity

The Second Annual Progeria Research Foundation's

CURE CUP CLASSIC

2019 CureCup CLASSIC

SAVE THE DATE

Golf Tournament
Ipswich Country Club
May 13, 2019

Progeria Research Foundation

Progeria Research Foundation®
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

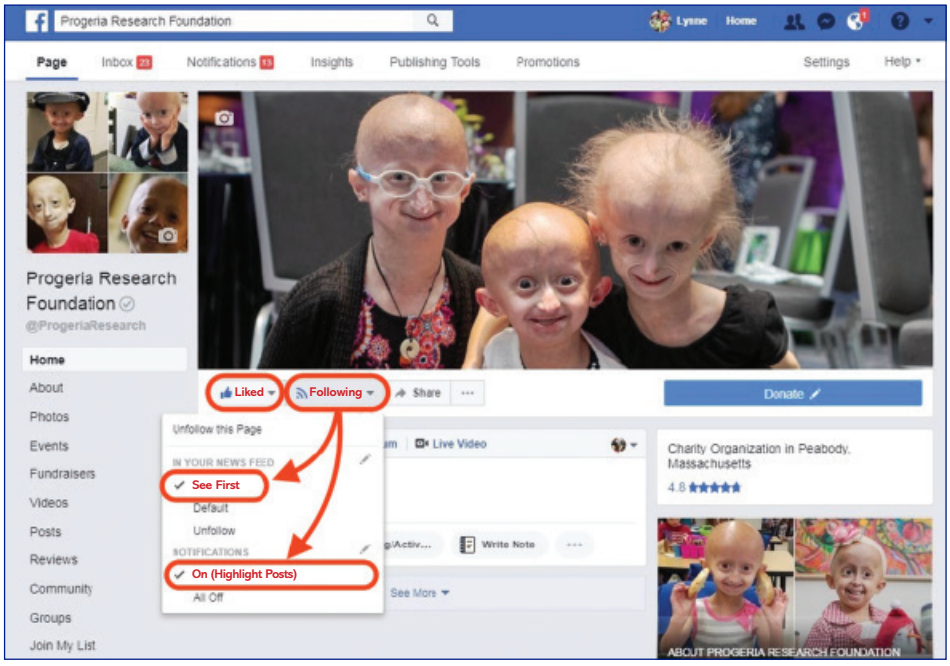
Enzo hopes everyone has a great school year!

Don't Miss an Update!

Enjoy reading news from PRF?
Get the latest updates by connecting with us on Facebook, Twitter, and Instagram.

Already "Like" us on Facebook?
Be sure to "Follow" us too!

Facebook recently changed the way your news feed is created, so many of you aren't seeing all the fantastic posts about the children, fun events, and our progress towards the cure. To see PRF's posts, be sure to "LIKE" AND "FOLLOW" us from the main PRF page.



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Other ways to connect with us:

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