**The Progeria Research Foundation**
www.progeriaresearch.org
August, 2007

# news

SPECIAL EDITION

**More Exciting News Inside:**



## Incredible News:

# First-Ever Progeria Clinical Drug Trial Has Begun!

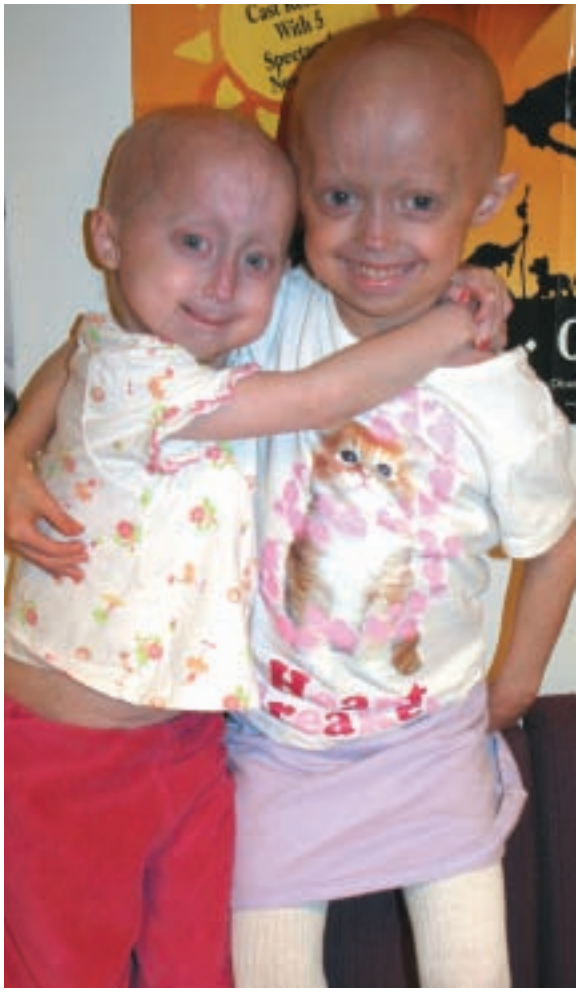
**History is officially in the making, with the start of the clinical drug trial at Children's Hospital Boston.**

We're thrilled to announce that the first-ever clinical drug trial for Progeria has begun. On May 7th, 2007, two children arrived in Boston, MA for their first of seven research visits over a two-year period, where tests were administered and they received their first doses of an experimental drug.

Families are coming from all over the world to participate in the trial, with hope that this drug will effectively treat Progeria in their children.

In this special edition PRF newsletter, we will introduce you to the research team, tell you what this trial is all about, and you will hear what parents have to say about it.

**For the first time since the disease was identified 120 years ago, there is a possible treatment for children with Progeria.**



*"The 2 Megans", both 6 years old, in Boston for the clinical trial.*

## PRF's Mission:

# To discover the cure and effective treatments for Progeria and its aging-related disorders.



**Be the first to hear the latest PRF news!**
Send us your email address to learn of research breakthroughs and other events as the news happens.
*info@progeriaresearch.org*

## Message from the President & Executive Director

Hello everyone,

How is it possible that we have begun a first-ever clinical drug trial that may treat children with Progeria? We've only been in existence for 8 years! If you read PRF's past newsletters and browse our web site, the answer becomes clear:

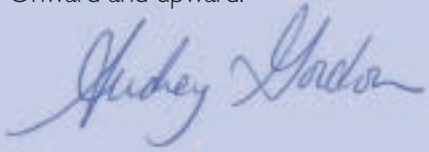
- Families of children with Progeria are mobilizing their communities around the world, raising money and awareness from a wonderful variety of events.
- Our steadfast volunteers – those with us from the beginning and those who have joined us along the way – remain committed to help.
- Miracle Makers from all over the world – many with no personal connection to Progeria but who saw or read about these special children and just had to help – are supporting PRF's efforts in record numbers.
- PRF's Night of Wonder gala and Race for Research get bigger every year, as past participants continue their support and new ones join in.
- Researchers continue to collaborate and work tirelessly to produce the breakthroughs such as those that have led us to the drug trial.

The list goes on and on.

And I suppose I have to attribute some of our successes to sheer luck. As my sister Leslie would say, "The stars have aligned for us in so many ways." So, with that luck, and thousands of the most dedicated people I could ever imagine, we find ourselves in the midst of an historical moment in time.

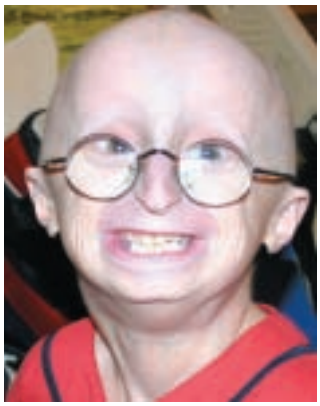
When we first began the $2 million dollar campaign to fund the trial, I was so excited! Yes, we had to raise about 3 1/2 times as much as our annual budget – on top of the money we need for our other activities – but I felt confident that we could do it. Because it's the best ask to make: donate to something that may help children with a fatal disease live longer, healthier lives. And I was right; many people have contributed, saying "This trial has to happen." As we press on to raise the remaining $600,000, I thank all of you who have helped bring us to this point, and invite you to continue to help us reach our goals – and realize our dreams.

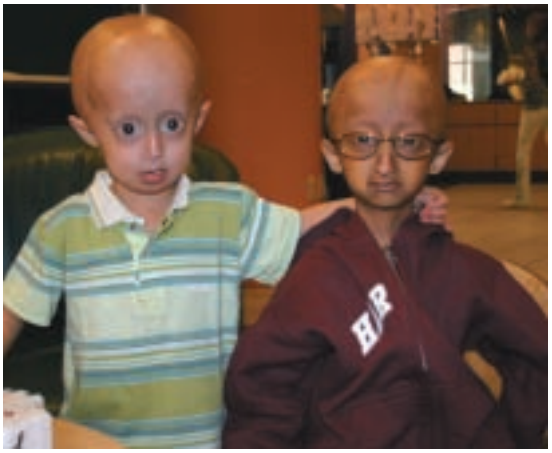
Onward and upward!

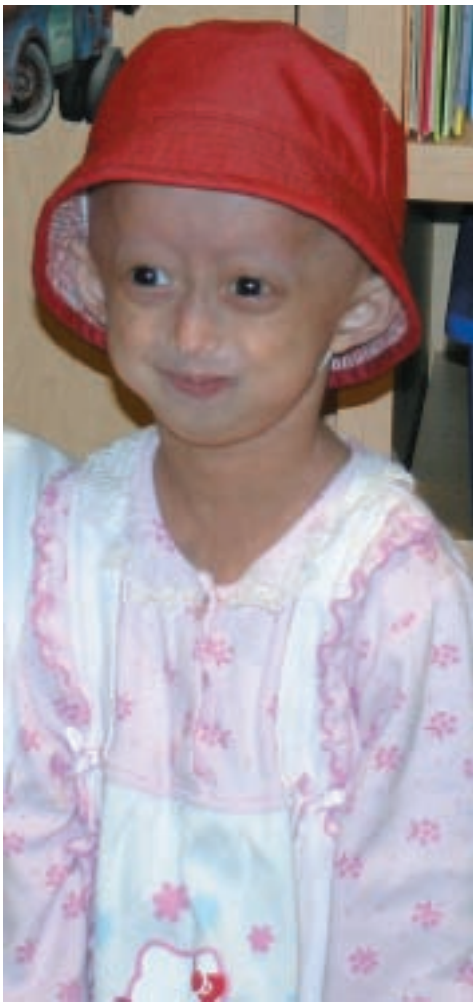


# These are the faces of Progeria.

This is the trial that may offer these children and their families hope for the future.







## The Progeria Clinical Drug Trial:

# Making History

THE CLINICAL DRUG TRIAL IS a collaborative effort - the children are being seen by physicians at Children's Hospital Boston, Dana-Farber Cancer Institute, and Brigham and Women's Hospital, all Harvard University institutions. In addition, physicians and scientists from Warren J. Alpert Medical School of Brown University, UCLA, the National Institutes of Health and Schering-Plough Research Institute are helping to make this trial a success.

The trial began on May 7, 2007, with two children. Through September, 1-2 children will travel to Boston to begin participating in the research. They return every four months for a period of two years, for testing and to receive new drug supply. They will stay in Boston for approximately 3-7 days at a time. While at home, their doctors will keep a close watch over the children and submit periodic health reports to the Boston research team.

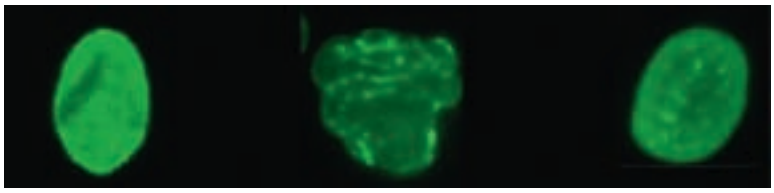
**How did we get to this point?** In 2002, The Progeria Research Foundation's collaborative research team made history with the discovery of the Progeria gene. This discovery not only led to further understanding of Progeria, but scientists now know that studying Progeria can help us learn more about heart disease and the normal aging process that affects us all.

Since the ground-breaking gene discovery, the dedication and support of researchers, clinicians, families of children with Progeria and people like YOU has brought us to another exciting crossroads in the search for treatments and a cure: Researchers have identified a potential drug treatment for children with Progeria, called farnesyltransferase inhibitors (FTIs), and have conducted studies in the lab that support a human trial with the drug.

Schering-Plough is supplying the FTI drug and pharmacokinetics (periodic measurements of drug levels to assess absorption of drug into the body).

**FTIs and the science behind the clinical drug trial:** The protein that we believe is responsible for Progeria is called progerin. To block normal cell function and cause Progeria, a molecule called a "farnesyl group" is attached to the progerin protein. FTIs act by blocking (inhibiting) the attachment of the farnesyl group onto progerin. If the FTI drug can block this farnesyl group attachment in the cells of children with Progeria, then progerin may be "paralyzed" and Progeria improved.

*Progeria cells become normalized when FTIs are applied. (Capell et al., PNAS, 2005)*



*Normal Skin Cell* | *Progeria Skin Cell* | *Progeria Skin Cell Treated with FTI*

For more information on FTIs and the science behind the trial, go to:
*http://www.progeriaresearch.org/the_fti_drug.html*

**How much will the trial cost PRF?**
We estimate the trial will cost PRF $2 million over a two and a half year period. This will pay for clinical testing, flights, food and lodging for families coming from fourteen different countries. We have raised $1.4 million to date.

**Principal Investigator Mark Kieran MD, PhD,** is Director, Pediatric Medical Neuro-Oncology, Dana-Farber Cancer Institute and Children's Hospital Boston; and Assistant Professor, Departments of Pediatrics and Hematology/Oncology, Harvard Medical School. Dr. Kieran is a pediatric oncologist. He has extensive experience with FTI treatment in children.

# Hear from some of the families involved in the clinical drug trial

**It's an exciting time for patients and families, and we wanted to share some of their thoughts about the trial.**

### What will this clinical trial mean for your child, who is living with Progeria?

I am extremely excited about the trial. When Kaylee was first diagnosed with Progeria, we were told that it was 100% fatal. This is news that no parent wants to hear, and was the toughest thing I have ever had to deal with. We were told to enjoy the time we would have with her. Thanks to The Progeria Research Foundation and the hard work of the researchers involved, there is now hope for Kaylee and all children with Progeria. We are optimistic about the future for Kaylee and excited to be a part of the trial.

*- Kaylee's parents, Ohio, United States*

### How has your experience been so far with the clinical trial?

*The Nighbors practice how to dispense the drug.*

We are so excited the treatment has begun!
This treatment has the potential to open up a WORLD of possibilities for Megan and the other children with Progeria! This MAY be the miracle everyone has worked so hard for. We went into this trial with mixed emotions, but after meeting the WONDERFUL staff at Children's Hospital Boston, our fears were put to rest. EVERYONE had such compassion for our family and of course our little Megan, it did NOT feel like being in a hospital, this is EXACTLY what children need. We cannot say THANK YOU enough for their patience and loving care. We hope we will be making history with this treatment, and we are so very grateful for the extraordinary team at Children's Hospital Boston, we KNOW our beautiful Megan is in GOOD hands!

*- The Nighbor Family, Wisconsin, United States*

### What are your feelings about the Progeria clinical trial?

We both feel positive but also a bit apprehensive. It's a very exciting time but very scary too. Our emotions are like a roller coaster. We both feel that there is hope for Hayley. Hayley also feels a bit scared but is being brave and strong.

*- Hayley's parents, United Kingdom*

"We are happy to participate to this trial and so to give a possibility to Sammy. We realize we have a difficult way before us but we are hopeful in its success."

*- The Basso Family, Italy*

# Meet the Trial Team

**In addition to Dr. Kieran, researchers from many clinical specialties are involved in the clinical aspect of the trial.**

**Co-Investigator** Leslie Gordon, MD, PhD is Assistant Professor of Pediatrics Research, Warren J. Alpert Medical School of Brown University and Rhode Island Hospital, Providence, RI, and Staff Scientist at Children's Hospital Boston. She is the co-founder and Medical Director for PRF, and serves as the Principal Investigator overseeing the PRF Diagnostics Testing Program, Cell & Tissue Bank, and Medical & Research Database. Dr. Gordon has published in the field of Progeria for 7 years, including authorship on the Progeria gene finding.

*Devin with Bill Fletcher*

**Co-Investigator** Monica Kleinman, MD is Assistant Professor of Anesthesia (pediatrics) at Harvard Medical School, a member of the executive committee of the Section on Transport Medicine, American Academy of Pediatrics, and a member of the Pediatric Subcommittee of the American Heart Association's Emergency Cardiovascular Care Program. She is trained in neonatal and pediatric critical care and currently serves as the clinical director of the Medical-Surgical Intensive Care Unit at Children's Hospital Boston. Dr. Kleinman is also an original member of PRF's Board of Directors and Medical Research Committee.

*Sam with Marie Gerhard-Herman (r) and assistant Nicole Wake, RVT.*

## From Children's Hospital Boston (CHB)

**Geneticist** David Miller, MD, PhD specializes in DNA diagnostics, congenital malformations and dysmorphology. He is also board certified in clinical genetics, clinical molecular genetics and pediatrics.

**Radiologist** Robert Cleveland, MD is Chief of the Division of Radiology, and Professor of Radiology. His research is directed at pediatric pulmonary disorders.

**Occupational Therapist** Annette Correia, OTR/L is the Clinical Manager of the Occupational Therapy Service at CHB. Her specialties include Hand Therapy, Power Mobility and Sensory Integration.

**Occupational Therapist** Jennie Dapice, MA, OTR/L works with research patients through the Department of Physical and Occupational Therapy.

**Audiologist** Brian Fligor, ScD, CCC-A is director of the Diagnostic Audiology Program and Instructor of Otology and Larnygology at Harvard Medical School.

**Endocrinologist** Catherine Gordon, MD, MSc is Assistant Professor and the Director of the Bone Health Program, Department of Adolescent/Young Adult Medicine.

**Dermatologist** Marilyn Liang, MD is Assistant in Medicine and Instructor at Harvard Medical School, and Board Certified in Dermatology.

**Dentist** Amy Regen, DDS is a clinical fellow in developmental biology at Harvard School of Dental Medicine and works at CHB's dentistry department.

Physical Therapist Susan Riley, PT, MS, DPT, PCS specializes in neuromuscular disorders at CHB.

Cardiologist Leslie B. Smoot, MD is Instructor in Pediatrics and the Assistant Director of the Heart Failure/Cardiac Transplant Program. Dr. Smoot's research focuses on the genetics of Congenital Cardiovascular Disease.

*Lindsay and Sarah play in a CHB waiting room.*

Neurologist Nicole Ullrich, MD, PhD is Director, Neuro-Oncology and Assistant in Neurology at CHB, Assistant Professor in Neurology at Harvard Medical School, and Board Certified in ABPN (Child Neurology).

## CHB's General Clinical Research Center (GCRC) Administrators and Nurses, including:

Administrative Director Kristine Jordan
Nutritionist and Research Dietitian Nicolle Quinn, MS, RD, LDN

*The GCRC serves as the central focus of clinical research at Children's Hospital Boston. Its mission is to provide an optimal setting for multidisciplinary clinical research, therefore ensuring the timely translation of scientific knowledge into effective patient care.*

## CHB's Center for Families Staff, including:

Social Worker Maura Ammon
Project Manager Jennifer Leach
Director Cynthia Levin

*The Center for Families helps parents become active members of their child's health care team, providing information on questions they may have about community or hospital resources.*

Manager Dorys Alarcon, CHB Interpreter Services

## From Brigham & Women's Hospital (BWH):

Cardiologist Marie Gerhard-Herman, MD is Director of the Non-Invasive Vascular Laboratory at BWH.

## From Dana-Farber Cancer Institute:

Trial Coordinator William Fletcher
Patient Coordinator Shannon Hubbs
Patient Coordinator Rebecca Wark

## The Biological Assay Team

***These researchers will assess the effects of the FTI drug using patient cells and blood samples***

W. Robert Bishop, Executive Director, Biological Research, Oncology & Virology, Schering-Plough Research Institute.

Francis S. Collins, MD, PhD, Director of the National Institute of Health (NIH) National Human Genome Research Institute.

Loren Fong, PhD, Assistant Adjunct Professor at University of California at Los Angeles (UCLA).

Stephen G. Young, MD, Cardiologist and Professor of Medicine at UCLA.

## *Also part of the trial team from NIH*

Cardiologist Elizabeth G. Nabel, MD, Director of NIH's National Heart, Lung and Blood Institute.

*Hayley works with physical therapist Christine Ploski.*

## From The Progeria Research Foundation:

Susan Campbell, PRF Coordinator for Medical Database, Diagnostics and Cell and Tissue Bank
Brown University Center for Gerontology

Lorraine Fast, PRF Cell and Tissue Bank Technician
Rhode Island Hospital.

Audrey Gordon, Esq., PRF Executive Director
Susan Rosenblatt, PRF Executive Assistant
Kyra Johnson, PRF Trial Coordinator

Children's Hospital Boston · Schering-Plough · DANA-FARBER CANCER INSTITUTE · UCLA · National Institutes of Health · BWH

# General Principles of Clinical Drug Trials and Specifics About the Progeria Clinical Drug Trial

## What is a Clinical Drug Trial?

A clinical drug trial is a type of research study designed to determine if a new drug is safe and effective in humans. Before human patients are involved, usually the drug is tested in cells or tissue grown in the lab, and then in animals, to determine its effects and provide the best possible assurance that a drug will be safe for people to take. There have been PRF-funded studies of FTIs applied to Progeria cells and given to Progeria mouse models. Moreover, FTIs have been administered to both adults and children in clinical trials for several types of cancers. Experiences with laboratory studies and cancer clinical drug trials are helping researchers to conduct the clinical trial for Progeria.

## Who conducts a clinical trial?

The clinical trial team includes doctors, nurses, physical and occupational therapists, and other health care professionals. They check the health of the participant at the beginning of the trial, give specific instructions for participating in the trial, test and monitor the participant carefully during the trial, and stay in contact after the trial is completed.

## Who can participate in a clinical trial?

Due to requirements for producing reliable data and accounting for risks, patients may have to be of a certain age, gender, stage of disease, or medical condition or status, to participate in many clinical trials. For this Progeria trial, children will need to have, in particular, positive genetic testing for Progeria, be medically cleared to travel to the trial site in the USA every four months for two years, and provide the researchers with specific weight data and clinical information prior to entry. We anticipate that children will be coming from 14 different countries, and will range from about 3 to 17 years of age.

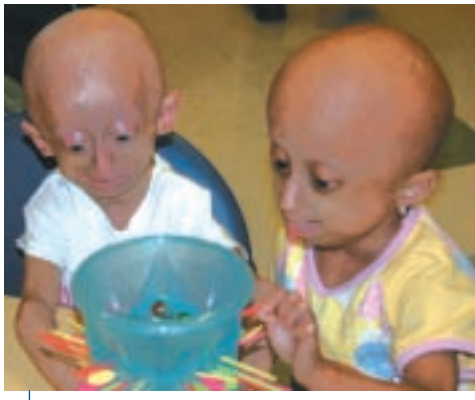
*Josiah in the CHB waiting room*

## How do clinical trials proceed?

Clinical trials can be Phase 1, 2 or 3, or a combination of these. Phase 1 trials measure safety and side effects in a small number of individuals (about 25 people). These trials vary drug dosage to look at safety and side effects at higher and lower doses, and measure something called "pharmacokinetics", where levels of drug are measured at various time points after drug is taken in order to assess how much drug is getting into the tissues. Phase 2 trials measure not only drug safety and side effects, but also whether the drug is effective in treating disease, in a small number of individuals (usually around 50 people). Phase 3 trials usually involve large numbers of people (often thousands) to assess not only safety and side effects, but also whether the drug will treat disease well enough to be recommended to the general population.

The trial for Progeria is a Phase 2 study: measuring side effects, measuring pharmacokinetics, and measuring the effects of the drug on disease. The Progeria trial has an open label design, wherein all participants receive the drug.

## What are the risks of participating in a clinical trial?



*Milagros and Maria play a game*

Risks to patients include side effects from the treatment, some of which may yet be unknown in nature, frequency or severity. This is why drugs are tested extensively in animals before use in people, but sometimes effects on patients are not revealed until the clinical trial stage or a clinical trial in a particular clinical condition. This is the very purpose for having participants monitored closely. To date, the FTI administered in this trial has been given to over 1,300 people – adults and children – for other trials.

## What improvements are expected in a drug trial?

As with any experimental treatment, researchers will not know with certainty if and how a drug will affect disease treatment when implemented. We at PRF are extremely hopeful about this clinical trial with FTIs, and researchers have designed the trial to recognize and objectively measure the many Progeria disease characteristics that may change with treatment. These include assessments of joints, bones, skin, heart, blood vessels, teeth, hearing, nutrition, and certain molecules in the blood. To this end, researchers will perform careful analysis on baseline clinical status (measurements taken before treatment starts) of children with Progeria. Researchers will compare these baseline measurements to measurements made during FTI treatment so that the impact of the treatment on each and every participant, as well as on the overall population enrolled, can be assessed.

### What if the drug is not effective?

Even if a candidate drug fails to be effective, the results of any clinical trial give new insight and direction to those working to cure the condition. The key is to design a trial that carefully measures the disease status, so that researchers have the best possible chance of knowing whether the drug has had an effect on the disease process.

### Where are clinical drug trials carried out and who pays for them?

Many clinical trials are conducted at medical centers of teaching hospitals linked to a medical school. Different organizations may support a clinical trial. These can include drug or biotech companies, federal agencies like the National Institutes of Health, or private charitable organizations such as The Progeria Research Foundation. The Progeria Clinical Drug Trial is being conducted as a collaborative effort between Children's Hospital Boston, Dana-Farber Cancer Institute (DFCI), and Brigham and Women's Hospital, all Harvard Medical School institutions in Boston, MA. Other institutions involved include Rhode Island Hospital, the National Institutes of Health, and UCLA. Pharmacokinetics and drug are being provided by Schering-Plough Research Institute (SPRI). It is being funded primarily by PRF, at an estimated cost of two million dollars.

*After only 8 years in existence, we are scientifically ready for this trial with a drug that may treat children with Progeria, and in the process we may help millions of older people who suffer from heart disease and other, aging-related conditions.*

### Will the families pay for any costs?

Thanks to the generosity of supporters like you, costs for travel to Boston, lodging, food, and clinical testing in Boston for the trial DO NOT fall on the families enrolled in the study.

### What are the steps leading up to the trial that ensure it is the safest and most effective plan to follow?

A variety of regulatory entities and review boards must review and approve the trial protocol. A protocol is a plan which details the various steps of the trial, including who may participate; the schedule of tests, procedures, medications, and dosages; and the length of the study. Every clinical trial protocol in the U.S. must be approved and monitored by an Institutional Review Board (IRB). An IRB is an independent committee of physicians, statisticians, community advocates, and others that ensures that a clinical trial is ethical and the rights of study participants are protected. The Progeria drug trial protocol was reviewed by the IRB and the General Clinical Research Center at Boston Children's Hospital, the US Food and Drug Administration (FDA), SPRI, and PRF's Medical Research Committee.

Prospective participants undergo written informed consent, the process of learning the key facts about a clinical trial – such as its purpose, duration, required procedures, key contacts, risks and potential benefits – before deciding whether or not to participate. Consenting is a continuing process throughout the study. Clinical trials are entirely voluntary and patients can withdraw at any point. Moreover, participants are informed of what is happening throughout the trial, including if known risks and side effects change.

**For more information about the Progeria Clinical Drug Trial, visit *www.progeriaresearch.org***


*Hayley with younger brother Louis*

# Thank you!

**MANY OF YOU HAVE DONATED TO PRF OVER THE YEARS,** and we are forever grateful for your support. Whether you attended a Night of Wonder, ran in our International Race for Research, participated in other special events that have taken place worldwide, or otherwise donated, you have all helped advance the field of Progeria research at a pace virtually unheard of in the scientific community. THANK YOU ALL for your support!

**On this page we have listed those that have made a donation in any amount directed to the Progeria Clinical Drug Trial, and those that have joined our Circle of Hope (which now includes the clinical drug trial – see opposite page) with a donation of $250 or more since the trial campaign was launched in June 2006.**

Did we miss anyone?

We try our best to include everyone, and apologize in advance if we inadvertently left you out.

Please email us at *info@progeriaresearch.org* so we can correct our records and list you in the next trial campaign mailing.

**Everyone who participated in the following special events:**

*Led by the CALIFORNIA Chapter:*
Back to the 50's Show, St. Paul, MN
BobCo Auto's F-100 Super Tour, Lake Elisnore, CA
Sioux Falls Ford Event, Sioux Falls, SD
UniquePerformance Fest, Farmers Branch, TX
Wheels 'n Waves car show, Santa Barbara, CA
YearOne Braselton Bash, Braselton, GA

*Led by the MICHIGAN Chapter:*
Lindsay's Miles for Miracles Fun Run/Walk, Flat Rock, MI

*Led by the OHIO Chapter (all events took place in Ohio):*
Cub Scout Pack 205 Father/Son Cake Bake, Maumee
Halko Garage & Bake Sale, St. Mary's
Kaylee's Course Run/Walk for Progeria, Montclova
Lial Elementary School's Hats-On Day, Whitehouse
Mercy Hospital and Defiance Clinic Bake Sale/Raffle, Defiance
Park Street Intermediate School events, Grove City
St. Joan of Arc School's Rock-a-thon, Toledo
Saturn of Toledo/Radio K100 event, Toledo
Whitehouse Christmas Tree Farm tree sales event, Whitehouse

*Other Events:*
Bud Boys events, Hamilton, Ontario, Canada
CIBC World Markets, Miracle Day USA
IDEAL Day, Evansville, IN
Chris Kempf's Golf Tournament, Evansville, IN
Aunt Luciana's many events, Argentina
Annual Megan Nighbor Benefit in Markesan, WI
Ory's Race for Research, MI
Rock 'Til You Drop car show, Ft. Meyers, FL
Shop Around 3 Hutchins Drive, Foxboro, MA
Walk in Honor of Kristian McGuiness, Middleboro, MA
Over forty events that took place all over the world, run by our Miracle Maker volunteers!

**Foundation, Corporate and Individual Support:**

**$100,000 and over:**
Anonymous
Italian Association for Progeria – Sammy Basso (A.I.Pro.Sa.B.)
Gretchen and Robert K. Morrison
Yawkey Foundation

**$50,000 to $99,999:**
Joe Crookham, Musco Lighting
Stephen Nixon
Zuckerman Family Foundation

**$10,000 to $49,999:**
Alum-A-Pole Foundation
Anonymous
Auen Foundation
Carl C. Anderson & Marie Jo Anderson Charitable Foundation
Charles Conaway
Tom B. Holman III, Dallas Flat Glass Distributors Inc.
Foose Design/Lynne and Chip Foose
Leslie Gordon and Scott Berns
Harvey Industries
Charles Henry Leach, II Foundation
Newman's Own Foundation
Santa Barbara Foundation
Mary Steenburgen and Ted Danson

**$5,000 to $9,999:**
Amelia Peabody Charitable Fund
CVS Pharmacy
Krauss Foundation
Kim and Joe Paratore
Monica Peacocke
Diane Pysniak
Michael Scotto Memorial Foundation

**$1,000 to $4,999**
Aberdeen Group, LLC
Angela Anderson
Aranon Corporation
Norman J. Arnold Foundation
John D. Baker
Gail and Tom Beamer
Karen and Dave Betournay
Rita and Harvin Betournay
Sandra Bresnick
Stephen Burlone
Sarah and Randall Cail
Community Investment at TransCanada
Thomas Lee Cooper
Joe DiGeronimo Charitable Fund
Alexander Falk
Patricia and Robert Flynn
Terry and Sam Foose
Honey and Harvey Freeman
Robyn Glazer and Thomas Milbury
Global Impact Combined Federal Campaign
Audrey Gordon and Richard Reed
Barbara and Burt Gordon
Seth Hammond, Specialty Crane and Rigging
Margaret Hicks
J J Eldred Limited Partnership
Monica Kleinman
Carole Krusch, Carole B. Design Associates, Inc.
John and Lori Ledoux
Florence and Edgar Leslie Charitable Trust
Agnes M. Lindsay Trust
Mark Allen Plumbing
Mass Mutual
Sherrie and Hershel McGriff
MCJ Foundation
Andrea Millioni
Jessica and Charles Myers
Joanne and Mathew Naimie
North Shore Bank
Jeffrey Osterman and Susan J. Matula
Gregory Pomeroy, T C G of Texas
Brett Ponn
George A. Ramlose Foundation
RC2 Corporation
Ritzow Family Foundation
Jill Rosenthal
Nancy Saikhon
Tammy Lee Singleton
Fred E. and Terry R. Slocum Charitable Fund
Phyllis and Edward Sorkin
Lisa and Bruce Spaulding
Spectrum Science Communications/ Christine and John Seng
Rupesh Vadrev
Veterinary Surgical Care, Inc.
Henry & Beate Voremberg Donor Advised Fund
Wal-Mart Foundation
Quintin Yao

**$250 to $999:**
Jennifer Adamo
Marjorie and Milton Adler
Alto Dairy Cooperative
Patricia M. Baker
Paul Battaglino
Kara and Dan Berns
Emilia Black
Katie and Rory Bloom
Karin and Jeff Bouvier
BP Foundation
Thomas Brooks
Rachel Liba Cardozo Children's Foundation
Elke Chrapko
Eric Chasen
Cherry Street Mission Ministries
James Clark
Combined Federal Campaign of South Hampton Roads
Ellen Conlin and Michael Jackowski
Jennifer and Curtis Cotton
Mina Davenport
Del Commune Enterprises, Inc.
Susan Duguid
John Dyett/Salem Partners LLC
Charles Edwards
Linda and Howard Eisenberg
Eugene Tucker Revocable Trust
Karla and James Fazzio
Carol and Stuart Feen
Karen and Curtis Franke
Sheila and Sanford Fitlin
Jack & Pauline Freeman Foundation, Inc.
Jeanie and Antonio Furlan
Alisa Galperin
GK Building Supply Corp.
Graivier Family Trust
Happy Dance/Carol Oehler
Harborage/Palisades Medical Center
Ariel and Isaac Haber
HCC Global Financial Products
Herbert Heflich
Heritage Wholesalers, Inc.
Sherre and Jeff Hirsch
Sally and Charles Hobbs
Sherri Hughes and AlanKauffman
Madelyn and James Jansma
Kewill Trade & Logistics
Kathleen Kinat
Kathleen M. King
Lisa Laird and Barbara Corso
Levy Family Giving Fund
Rebecca Li
Logical Health Solutions
Nancy and Joseph Luchetta
Lugo Caravan 113 / Order of Alhambra
Ken Major
Michael Maroldi
Carolyn Matula
Kevin McFadden
Sharon Mehlman
Marie and Nick Migliaccio
Sofia Mitchell
Joseph & Marion L. Mitola Family Fund
Elizabeth and Robert Morrison
Motorists Insurance Group
Kerry and Kathie Mulholland
Kelley and Steven Myers
North Texas Mustang Club

A. Kim O'Brien
Maureen and Patrick O'Toole
Joanne and Kenneth Owens
Pacific Coast Corvettes
Debra J. Pearlstein
Perkins Drug, Inc.
Tory Peterson
PrairieFire Foundation
Michael Ricker
Frank Rose, Dent Surgeon Inc.
Sam's Club Foundation
Semmer Group
Anne M. Sereno
JT Sherry
Sixthman
Roy Sonenshein
Spiller L'Chaim Fund
Thelma Sullivan
C.J. Svatos
Melanie and Jim Sweeney
Tracey Lynn Tanaka
United Way
Nancy and David Tocci
Janine Todd and Yvonne Duchene
Toledo Police Patrolmen's Association
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Rebecca Vasquez
Rahim Hassanali Walji
Linda White
Betsey Zbyszynski

## We need your support for this Progeria Clinical Drug Trial

# This is the time for *you* to be a part of history.

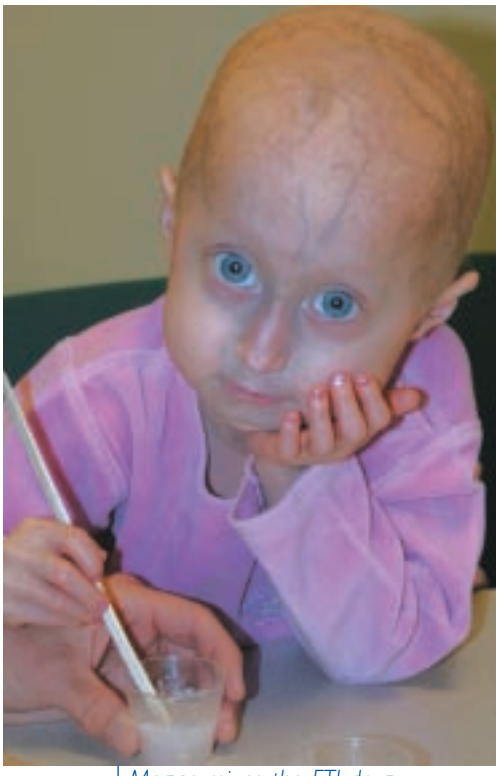
### This clinical trial is now part of our Circle of Hope…

PRF's Circle of Hope consists of all the research-related programs needed to advance the field of Progeria research and continue our record-breaking pace of progress: Our Cell & Tissue Bank, Diagnostics Testing, Scientific Workshops, Medical & Research Database, and Research Grant Funding are all running full speed ahead thanks to your support. We now welcome this historic clinical drug trial into our Circle of Hope.

Your financial support is what keeps the Circle of Hope intact, making this drug trial happen.

### To donate online, please visit:

*http://www.progeriaresearch.org/ways_to_donate.htm* and designate your donation to the clinical trial.



*Megan mixes the FTI drug with a sweetener*


## $600,000 left to raise…

PRF must raise approximately $2 million dollars to fund this drug trial, and we are excited to announce that we have raised $1.4 million so far – Amazing! Let's keep going!!

# thank you

**to our in-kind donors, who have generously given their time, talents and treasure to the clinical trial in many different ways.**

Delta Airlines: Airline tickets
Devon Nicole House at Children's Hospital Boston: lodging
The Family Inn: lodging
(also made possible through a grant from the Yawkey Foundation)
Tahlia Fischer and Eric Neilson: Cuddly Bears
Hasbro: toys and games
March of Dimes: Airline tickets
Regina Printing: design and printing
Schering-Plough: FTI drug and pharmacokinetics

Our lawyers: Karen Ballack, Jeff Osterman, and Jeannie Karl of Weil, Gotshal & Manges, LLP, NY, NY and Silicon Valley, CA

Over 50 volunteer translators representing 11 languages

**And thank you to our volunteer ambassadors, who pick up the families from the airport and get them settled in for the week of testing:**

Rachel Ammerman
Rich Cummings
Barbara Gordon
Audrey Lampert
Luciana Maffeo
Kim Paratore
Debbie Ponn
Jen Queally
Vickie Robbin
Hideshiro Saito-Benz
Hyun Soo Shim
Susan Sussman

DELTA · Devon Nicole House · THE FAMILY INN · Hasbro · March of Dimes · Schering-Plough


The Progeria Research Foundation

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




## Together we WILL find the cure!

To learn more about how you can help, contact us:
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