PRF By The Numbers

PROGERIA RESEARCH FOUNDATION™





Please Do Not Reproduce Children's Photographs Without Express Permission From PRF

Produced by Leslie B. Gordon, MD. PhD; Medical Director

© 2026 The Progeria Research Foundation. All Rights Reserved.

September 30, 2026

Table of Contents

- Introduction and Collaborations 3 -10
- Overview Data 11 -20
- International Progeria Registry 21 - 24
- PRF Diagnostics Program 25 - 28
- PRF Cell & Tissue Bank 29 - 35
- PRF Medical & Research Database 36 - 40
- Weighing - In Program 41 - 44
- Clinical Trials 45 - 55
- Progerin Biomarker 56 - 58
- PRF Grants Program 59 - 60
- Scientific Meetings and Workshops 61 - 64
- Publications 65 - 68

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF By The Numbers: A Data Sharing Tool

- PRF By The Numbers is a **data sharing tool** originating from The Progeria Research Foundation's programs and services.

- We translate information collected within our programs and services, and develop charts and graphs which track our progress from year to year.

- This allows you to assess where we've been, and the improvements we've made for children with Progeria.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Why Sharing Data Is Essential

- According to the National Institutes of Health:

  "data sharing is essential for expedited translation of research results into knowledge, products, and procedures to improve human health."

  http://grants.nih.gov/grants/guide/notice-files/NOT-OD-03-032.html

- In other words, everyone benefits by knowing and learning as much as possible about Progeria - the scientific and medical communities, the public, and the children.

NIH National Institutes of Health
Turning Discovery Into Health

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF By The Numbers…Here's How It Works

➢We take raw data collected through our programs and services, remove any personal information to protect the participant, and present it to you in a format that is engaging and informative.

➢ PRF programs and services include:



- The PRF International Registry
- The PRF Diagnostics Program
- The PRF Cell & Tissue Bank
- The PRF Medical & Research Database
- PRF Research Grants
- Scientific Workshops
- Clinical Trial Funding and Participation
- Progeria Connect

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Our Target Audience

- PRF By The Numbers is intended for a broad array of users
  - Families and children with Progeria
  - The general public and nonscientists of all ages
  - Scientists
  - Physicians
  - The media
- This means that different types of slides will be of interest depending on who is looking at the information. We have designed this slide set so that you can pull out what is most important to you.
- We love suggestions - if you don't see some facts and figures here that you think would be informative, please let us know at

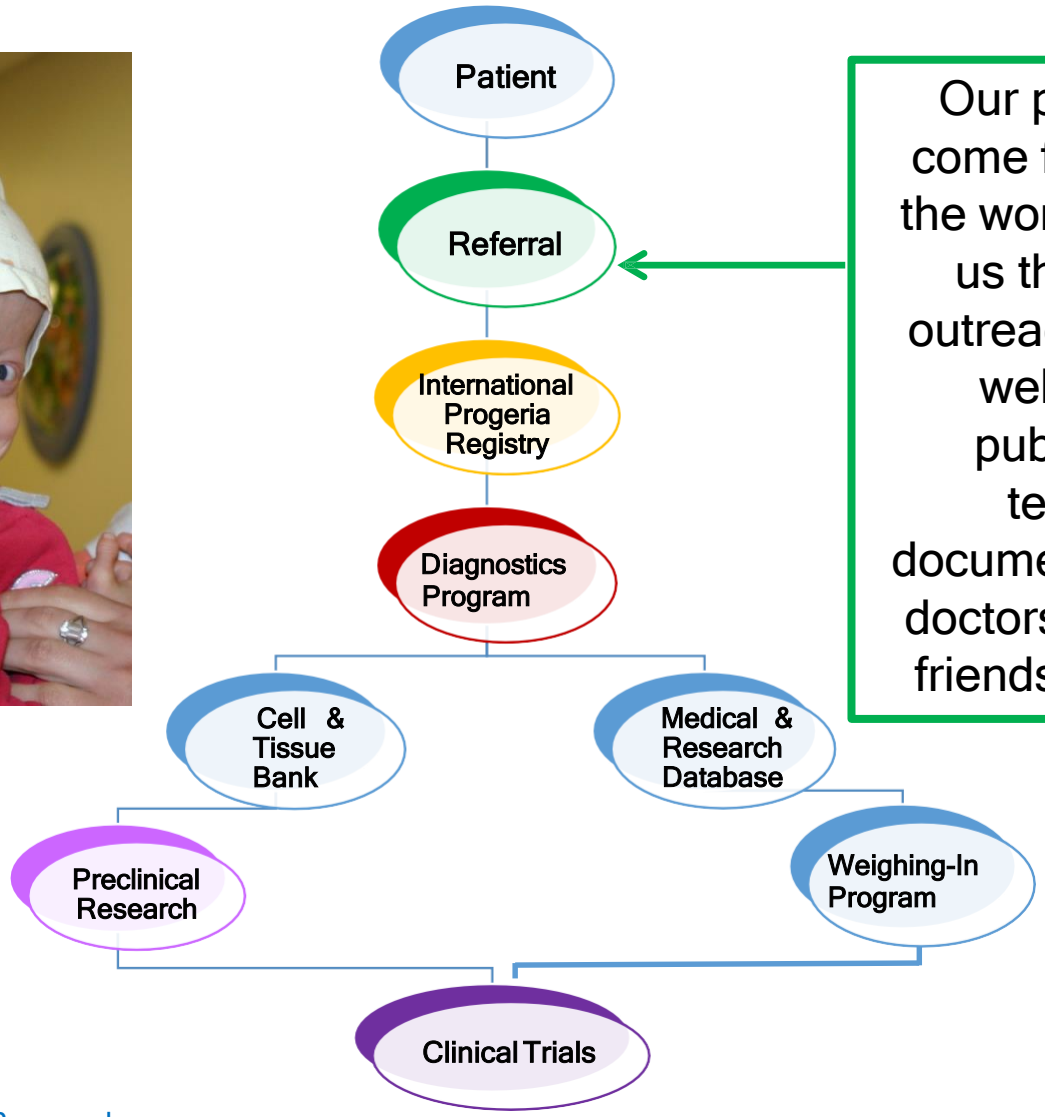
info@progeriaresearch.org

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Programs: It All Starts With The Children



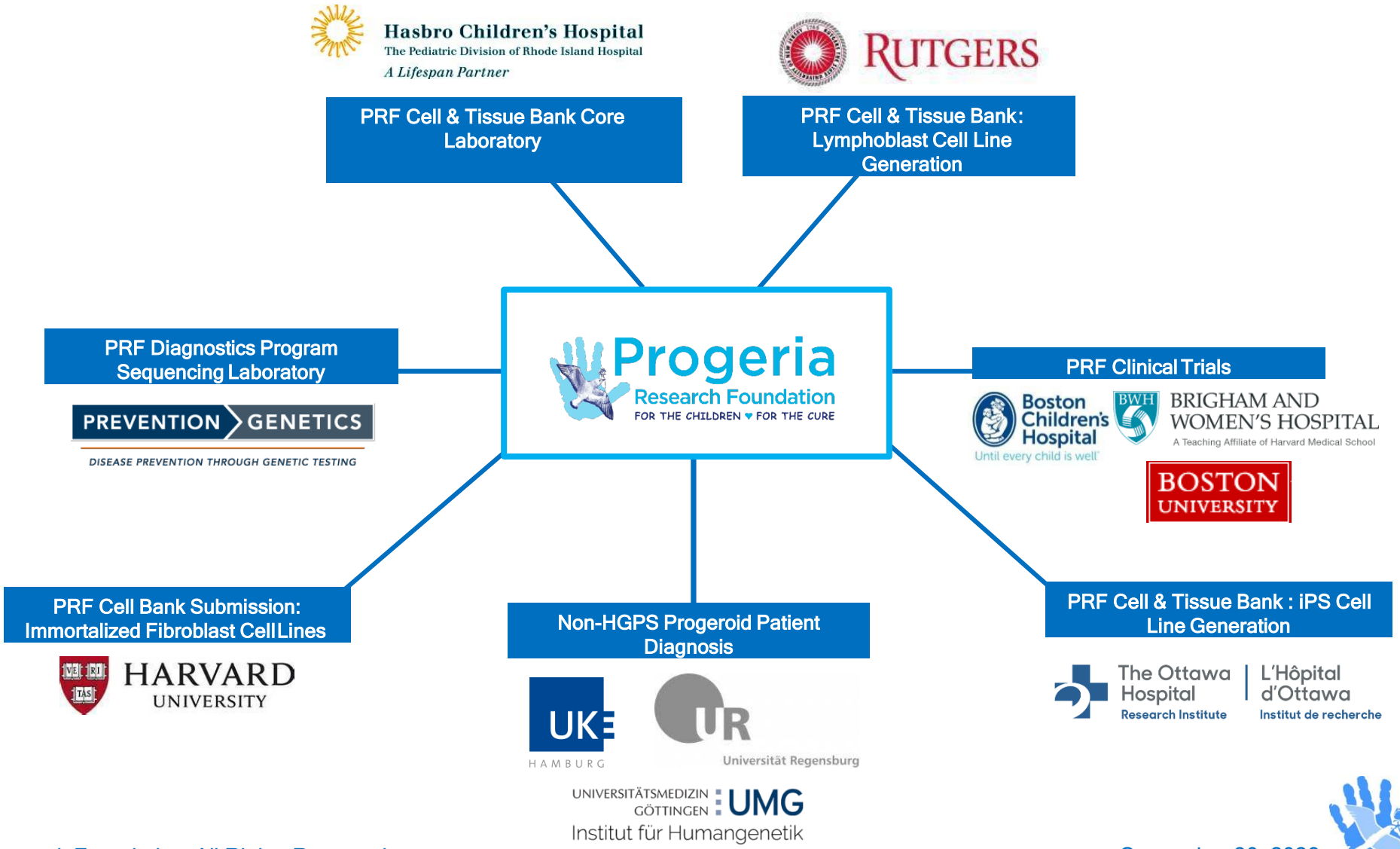


Our participants come from all over the world. They find us through our outreach – the PRF website, our publications, television documentaries, their doctors, neighbors, friends and family.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Program Collaborations for Success



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Our Program Collaborators

Our collaborating institutions are crucial to our ability to help children with Progeria. We are extremely grateful for these ongoing partnerships:

- Hasbro Children's Hospital
  - Location of The PRF Cell & Tissue Bank
  - Program IRB approval

- PreventionGenetics
  - CLIA*-approved genetic sequence testing

- Ottawa Hospital Research Institute
  - Induced Pluripotent Stem Cell (iPSC)
  - CLIA*-approved generation and distribution

© 2026 The Progeria Research Foundation. All Rights Reserved.

Our Clinical Trial Collaborators

Our collaborating institutions are crucial to our ability to help children with Progeria

- Harvard University – Associated Hospitals:
  - Boston Children's Hospital
  - Brigham and Women's Hospital
  - Dana Farber Cancer Institute

Boston Children's Hospital
Until every child is well™

PRG Science & Technology

BWH BRIGHAM AND WOMEN'S HOSPITAL
A Teaching Affiliate of Harvard Medical School

- NIH – funded Clinical and Translational Study Unit at Boston Children's Hospital
- Sentynl Therapeutics, Inc.

NIH National Institutes of Health
Turning Discovery Into Health

SENTYNL THERAPEUTICS, INC.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Number of Living PRF-Identified Cases

September 30, 2026

Total Number of Children with HGPS* and PLs** Worldwide: 227

HGPS* Worldwide: 163

HGPS* in the United States: 20

Progeroid Laminopathies** Worldwide: 62

Progeroid Laminopathies** in the United States: 16

*Children in the HGPS category have a progerin-producing mutation in the LMNA gene

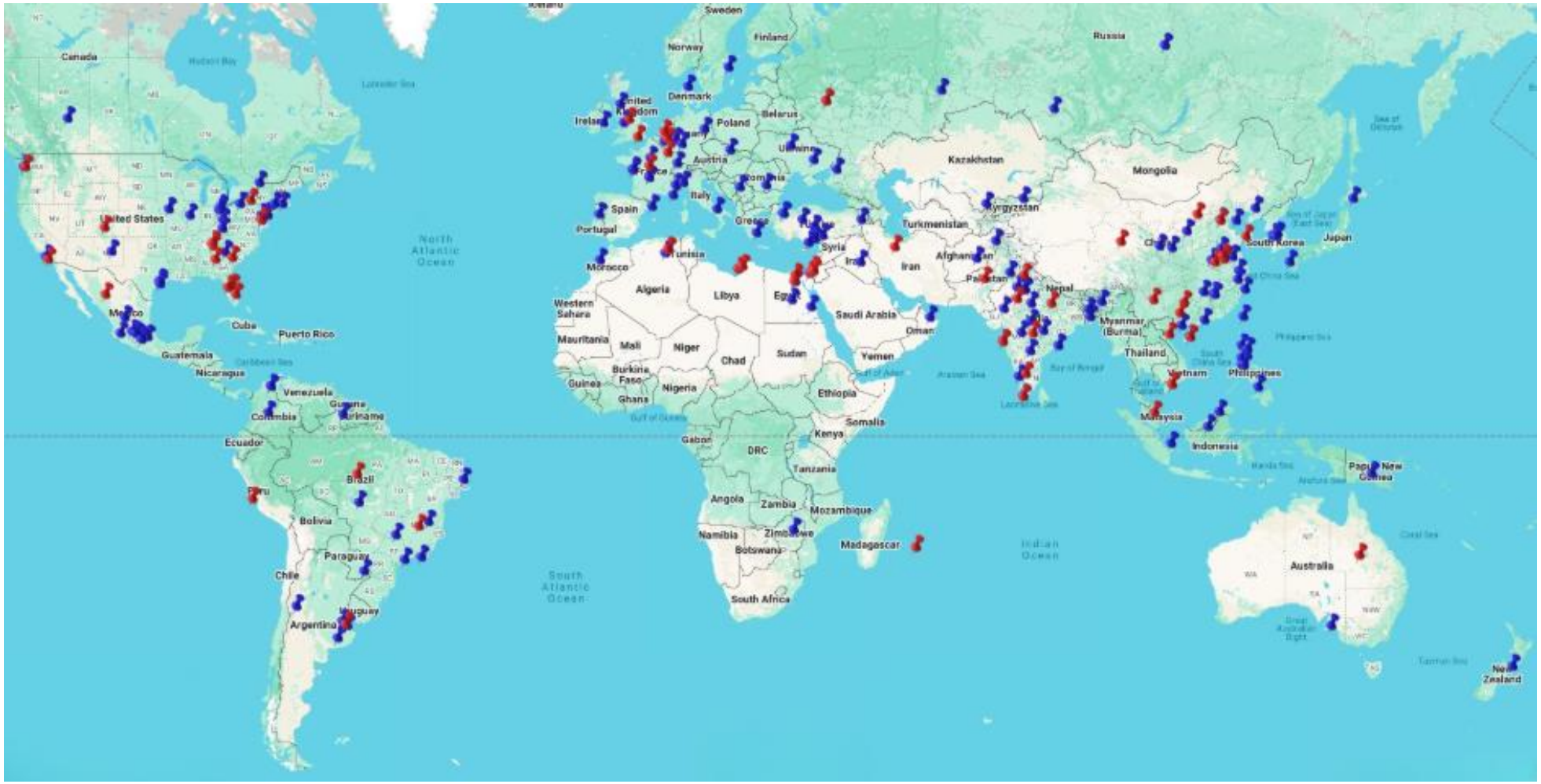
**Those in the Progeroid Laminopathy category have a mutation in the lamin pathway but don't produce progerin
**Cases of PL do not include those identified solely from published scientific journal articles

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Living PRF-Identified Cases Reside In 56 Countries Today

Afghanistan
Algeria
Argentina
Armenia
Australia
Bangladesh
Belgium
Brazil
Canada
China
Colombia
Czech Republic
Denmark
Egypt
Ethiopia
France
Germany
Greece
Guyana
India
Indonesia
Iran
Iraq
Ireland
Israel
Italy
Japan



Children and young adults with HGPS

People with PLs

163 Known Children and Young Adults with HGPS and 59 People with PLs Living Around the World as of September 30, 2026.

Kazakhstan
Libya
Luxembourg
Malaysia
Mexico
Morocco
Netherlands
New Zealand
Oman
Pakistan
Palestine-Gaza
Papua New Guinea
Peru
Philippines
Portugal
Romania
Russia
Serbia
South Korea
Spain
Sweden
Taiwan
Tajikistan
Turkey
Ukraine
United Kingdom
USA
Vietnam
Zimbabwe

© 2026 The Progeria Research Foundation. All Rights Reserved.

September 30, 2026

...and Speak 42 Languages

Amharic	Cebuano	English	Indonesian	Makay	Portuguese	Shona	Telugu	Vietnamese
Arabic	Chinese	French	Italian	Malayalam	Punjabi	Sindhi	Tok Pisin	Yiddish
Armenia	Czech	German	Japanese	Marathi	Romanian	Spanish	Turkish	
Assamese	Danish	Hebrew	Kannada	Nepali	Russian	Tagalog	Ukrainian	
Bengali	Dutch	Hindi	Korean	Pashto	Serbian	Tamil	Urdu	

прогерии исследовательский фонд

خيشلا ثاحبأ ةسسؤم



조로증 연구 재단

早衰症研究基金會

Progeria Araştırma Vakfı

Progeria रिसिच फाउंडेशन

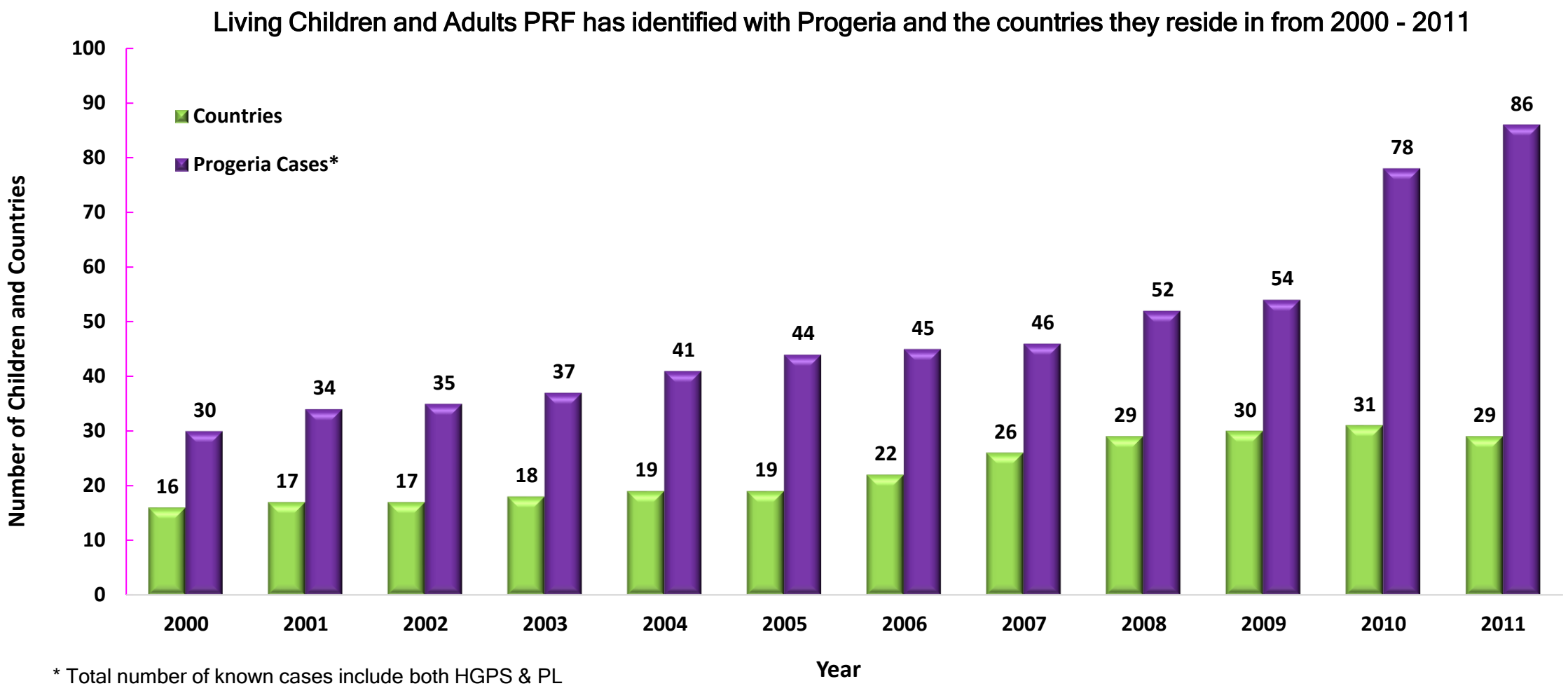
早老症研究財団

బాలుడ బాలికవయస్సుముదరుకండానే వృద్ాాప్యరూప్ంలోనికిమచ్చటరోయుచ్చు ండేసున్

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Every Year Our Numbers Grow

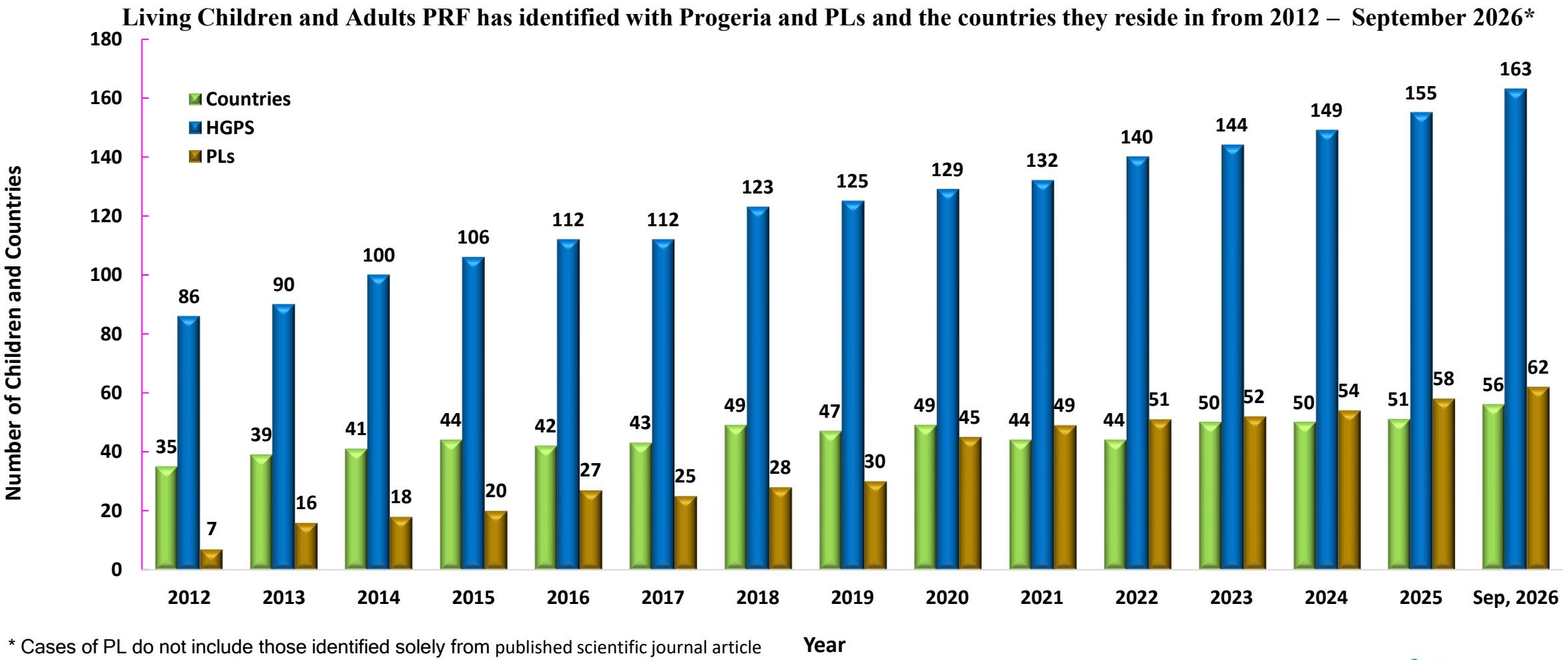


* Total number of known cases include both HGPS & PL
* When a child passes away, numbers are decreased

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Every Year Our Numbers Grow



* Cases of PL do not include those identified solely from published scientific journal article
* When a child passes away, numbers are decreased

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Tracking Children with Progeria Through Prevalence

- How does PRF estimate how many children we are searching for, and in what countries? We use ***population prevalence***.

- Prevalence is the proportion of children with Progeria per total population.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

How Prevalence Is Estimated

- At PRF, we use a formula based on the number of children we've identified in the US. We then expand that out to the world population.
- We do this because we have the most complete reporting for the US and since Progeria has no gender, ethnic, or other biases, we assume that the prevalence in the US is the same prevalence in other countries.
- PRF calculates prevalence for the US based on *Worldometer* population estimates.

© 2026 The Progeria Research Foundation. All Rights Reserved.

USA Prevalence of Progeria

September 30, 2026 population statistics:

- The US population is:

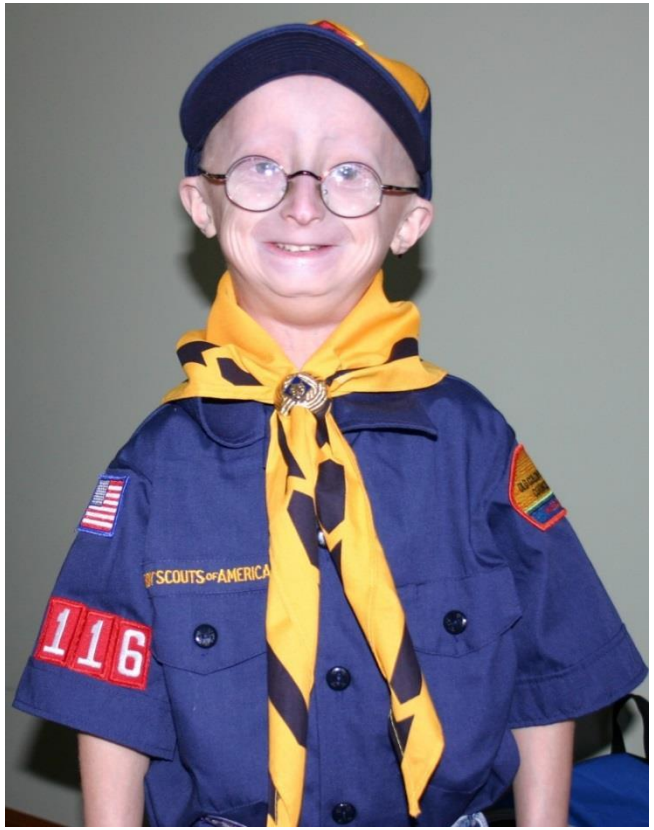
  349,600,219 people

- Number of PRF-identified children with HGPS in the US (3 year average)

  17

- Average prevalence of HGPS in the US:
  17 in 349 million is about

  1 in 20 million people



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Prevalence and World Population of Progeria

Given the world population on September 30, 2026

·Given the world population on September 30, 2026 There are approximately 416 children living with Progeria worldwide



PRF strives to find every child with Progeria because in order to help every child, we must find every child

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Using Prevalence To Find Children

We can now use the total population estimates for any given country, in order to understand whether we have found most or all children in a particular country.

➢ For example, as of September 30, 2026:





- Brazil's population was estimated as

213,833,652 people

- Using Prevalence, the number of children living with Progeria in Brazil is 213,833,652/20,000,000 = **11**

- PRF has identified 7 of these 11 children, and is searching for the 4 others

* Data based on the latest *Worldometers* estimates

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

International Progeria Registry*

Program Goals:

- Patient identification
- Outreach to patient families and their physicians
- A springboard for program enrollment

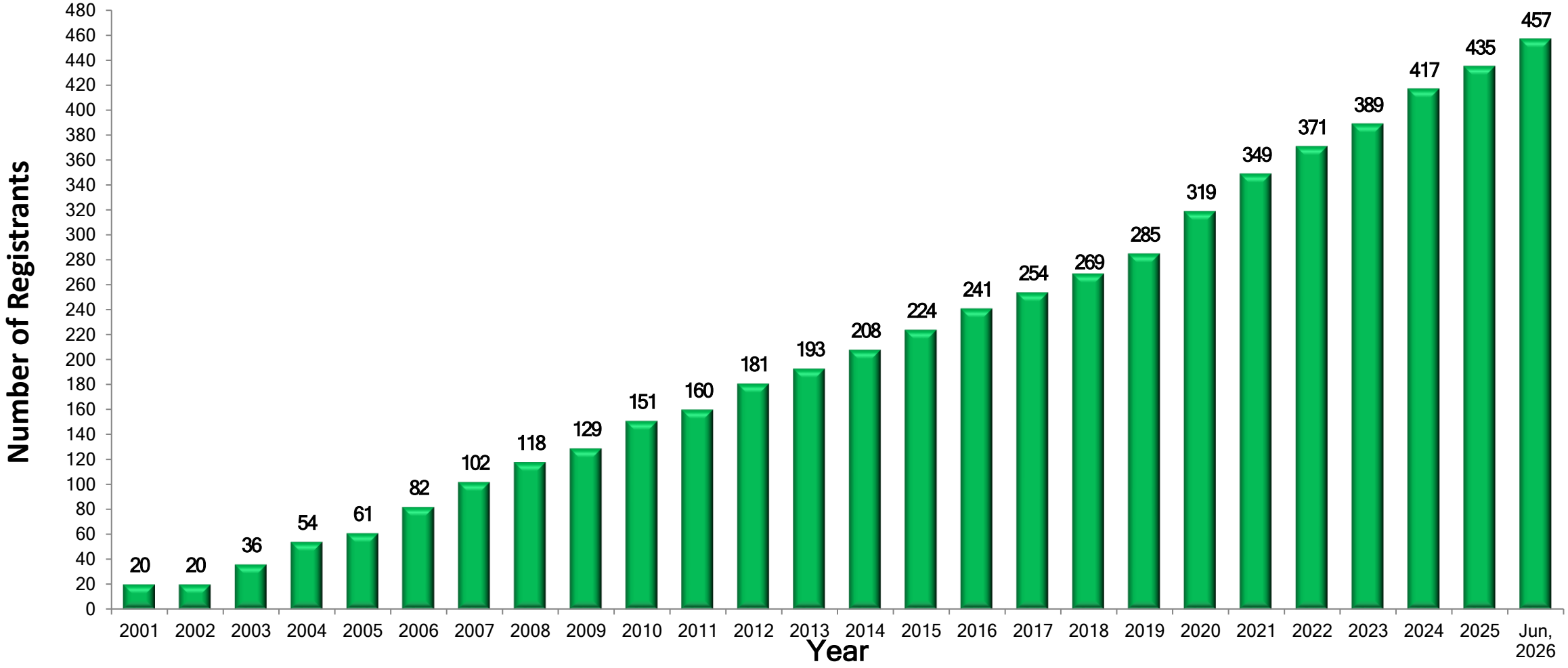
Registry forms available at
https://www.progeriaresearch.org/international-registry-2/

*PRF International Registry includes those with genetically confirmed or clinically suspected Progeria, as well as those with ZMPSTE24 and other possible progeroid syndromes

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

457 Children and Adults Have Been Registered With PRF



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

From 78 Countries and 1 Territory

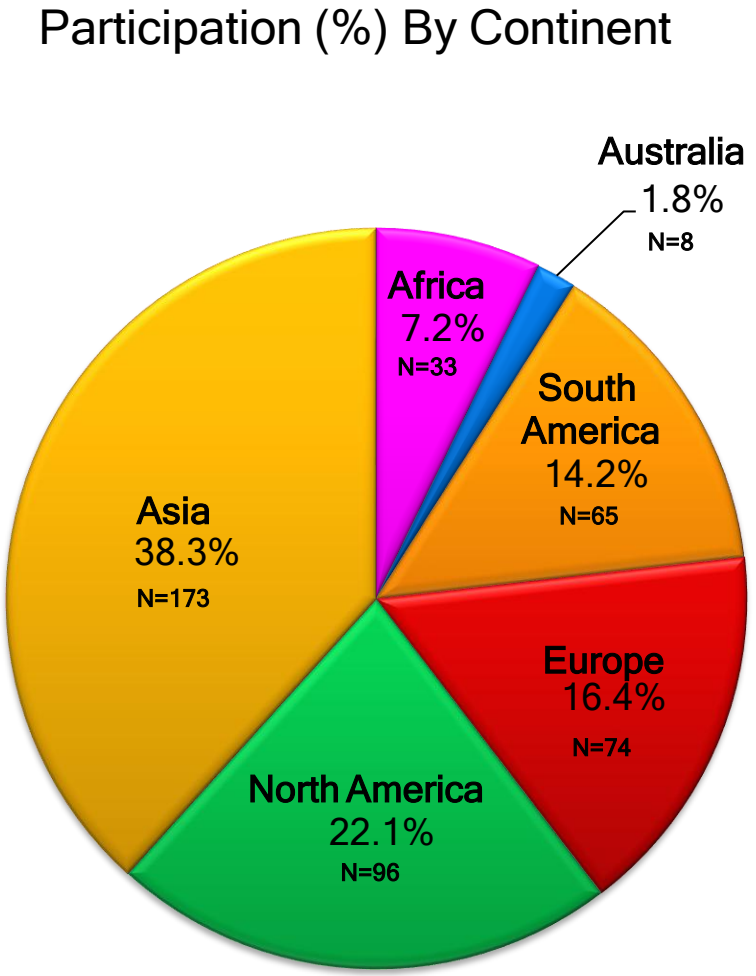
Algeria, Argentina
Armenia, Australia,
Bangladesh
Belgium, Bolivia
Brazil, Bulgaria
Canada, Chile
China, Colombia
Czech Republic
Denmark
Dominican Republic
Ecuador, Egypt
England, Ethiopia
Finland, France
Germany, Greece,
Guatemala Guyana,
Honduras
Hong Kong, India
Indonesia, Iran, Iraq
Ireland, Israel
Italy, Japan
Kazakhstan
Kyrgyzstan, Libya
Luxembourg, Malaysia
Mexico, Morocco
Nepal, Netherlands



New Zealand, Nigeria
Oman, Pakistan
Palestine
Papua New Guinea
Panama
Peru, Philippines
Poland, Portugal
Puerto Rico
Romania, Russia
Saudi Arabia
Serbia,
South Africa
South Korea
Spain, Sri Lanka
Suriname, Sweden
Switzerland, Taiwan,
Tanzania
Thailand, Togo
Turkey, Ukraine
USA, Uzbekistan,
Venezuela
Vietnam
Zimbabwe

© 2026 The Progeria Research Foundation. All Rights Reserved.

...And All Continents



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Diagnostics Program

Program Goal:

- Genetic Sequence Testing for Progeria-causing mutations



Pre-requisites for Testing:

- Registration with PRF International Registry
- One or more of the following
  - Family history – proband, prenatal
  - Phenotypic presentation – proband, postnatal
  - Relative of positive proband

Testing information available at:

https://www.progeriaresearch.org/the-prf-diagnostic-testing-program/

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Diagnostics Testing Summary

September 30, 2026

Total Number of Proband Tests Performed: 173

Exon 11 (HGPS) Mutations: 118

Other Progeroid Laminopathies (Exons 1 – 12): 17

ZMPSTE24 Mutations : 4

Average Number of Patients Tested Per Year : 9

All tests are performed in a Clinical Laboratory Improvement Amendments (CLIA) certified facility.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Mutations Identified Through PRF Diagnostics Program

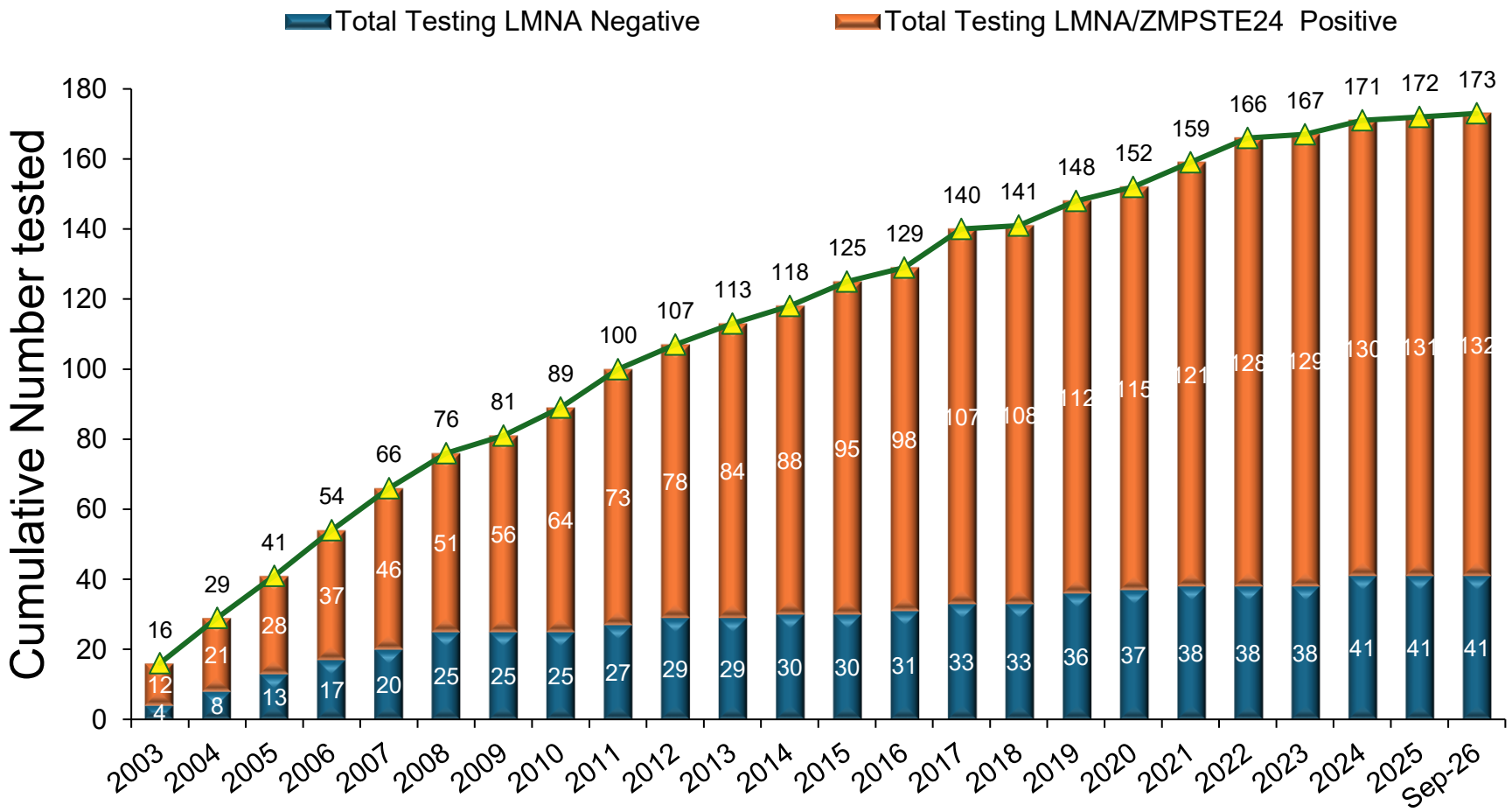
DNA Mutation	Amino Acid Effect	Zygosity	Progerin Producing?	Number Diagnosed
Classic HGPS – LMNA Mutation				
1824 C>T, exon 11	G608G	heterozygous	Yes	104
Non-Classic HGPS– LMNA Mutation				
1821 G>A, exon 11	V607V	heterozygous	Yes	2
1822 G>A, exon 11	G608S	heterozygous	Yes	3
1968+1 G>A, intron 11	None	heterozygous	Yes	1
1968+1 G>C, intron 11	None	heterozygous	Yes	3
1968+2 T>A, intron 11	None	heterozygous	Yes	1
1968+5 G>C, intron 11	None	heterozygous	Yes	2
Progeroid Laminopathy– LMNA Mutation				
331 G>A, exon 1	E111K	heterozygous	No	1
1579 C>T, exon 9	R527C	heterozygous	No	3
1579 C>T, exon 9	R527C	homozygous	No	6
1580 G>T, exon 9	R527L	homozygous	No	2
1619 T>C, exon 10	M540T	Heterozygous	No	2
1619 T>C, exon 10	M540T	homozygous	No	3
1868 C>G, exon 11	T623S	heterozygous	Yes	1
Progeroid Laminopathy– ZMPSTE24 Mutation				
1274 T>C, exon 10	L425P	heterozygous	No	2
1274 T>C, exon 10	L425P	homozygous	No	2

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Longitudinal Testing Data for PRF Diagnostics Program

Number of Affected Children/Adults Tested and the Number Testing Positive for *LMNA* Gene Mutation



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Cell & Tissue Bank

Program Goals:

- Provide a resource for researchers worldwide
- Ensure the sufficient availability of genetic and biological materials essential for research aimed at understanding the pathophysiology of disease and the links between Progeria, aging and heart disease
- Obtain long-term clinical data



Resource information available at: https://www.progeriaresearch.org/cell-and-tissue-bank/

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Cell and Tissue Bank Holdings

As of September 30, 2026

Total Number of Participants: **420***

71 Dermal Fibroblast Lines from **47** affected and **24** parents

6 Immortalized Fibroblast Cell Lines from **1** affected and **5** parents

125 Lymphoblast Lines from **71** affected, **46** parents and **8** siblings

9 Induced Pluripotent Stem Cell Lines from **5** affected and **4** parents

* Participants may have donated multiple times
* Additional sample types are available for special projects upon request

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Mutations Available in PRF Cell & Tissue Bank

DNA Mutation	Amino Acid Effect	Zygosity	Progerin Producing?	Cell Type DFN=Dermal Fibroblast LBV= Lymphoblast
Classic HGPS – LMNA Mutation				
c.1824 C>T, exon 11	p.G608G	heterozygous	Yes	DFN, LBV, iPSC
Non-Classic HGPS– LMNA Mutation				
c.1821 G>A, exon 11	p.V607V	heterozygous	Yes	LBV
c.1822 G>A, exon 11	p.G608S	heterozygous	Yes	DFN, LBV
c.1824 C>T, exon 11 & SMC3 c.562 A>G	p.G608G & p.K188E	heterozygous	Yes	DFN
c.1968+1 G>A, intron 11	---------	heterozygous	Yes	DFN, LBV
c.1968+2 T>C, exon 11	---------	heterozygous	Yes	DFN
c.1968+2 T>C, exon 11 & c.1968+2 T>A, exon 11	---------	heterozygous	Yes	DFN
c.1968+5 G>A, intron 11	---------	heterozygous	Yes	DFN
c.1968+5 G>C, intron 11	---------	heterozygous	Yes	DFN
Progeroid Laminopathy– LMNA Mutation				
c.331 G>A, exon 1 & c.1158-44 C>T, intron 6	p.E111K	heterozygous	No	DFN, LBV
c.412 G>A	p.E138K	heterozygous	No	DFN
c.973 G>A, exon 6	p.D325N	heterozygous	No	DFN
c.1303 C>T, exon 7	p.R435C	homozygous	No	DFN
c.1579 C>T, exon 9	p.R527C	heterozygous	No	LBV
c.1579 C>T, exon 9	p.R527C	homozygous	No	LBV
c.1580 G>T, exon 9	p.R527L	homozygous	No	LBV
c.1619 T>C, exon 10	p.M540T	homozygous	No	DFN
c.1762 T>C, exon 11	p.C588R	heterozygous	No	DFN
c.1868 C>G, exon 11	p.T623S	heterozygous	Yes	LBV
c.1930 C>T, exon 11	p.R644C	heterozygous	No	DFN
Progeroid Laminopathy– ZMPSTE24 Mutation				
c.743 C>T, exon 6 & c.1349 G>A, exon 10	p.P248L & p.W450Stop	heterozygous	No	DFN
c.1274 T>C, exon 10	p.L425P	homozygous	No	DFN, LBV

PRF Cell & Tissue Bank Distribution

As of September 30, 2026:

267 Research Teams From **29** Countries Have Received

1737 Cell Lines

207 DNA Samples

604 Tissue, plasma, serum and other biological samples

38 Lonafarnib Samples

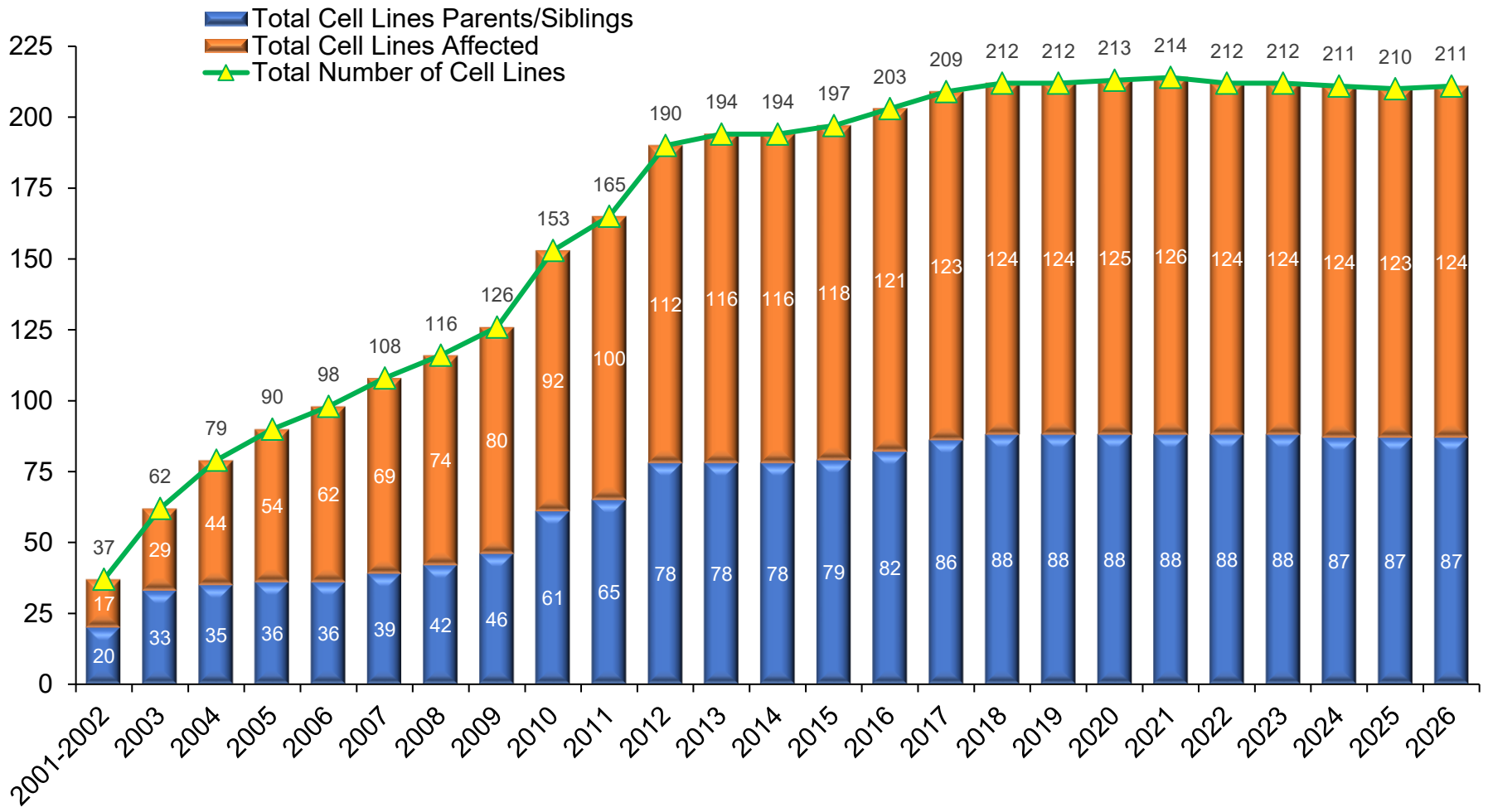


Senescent Progeria Fibroblasts in Culture

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

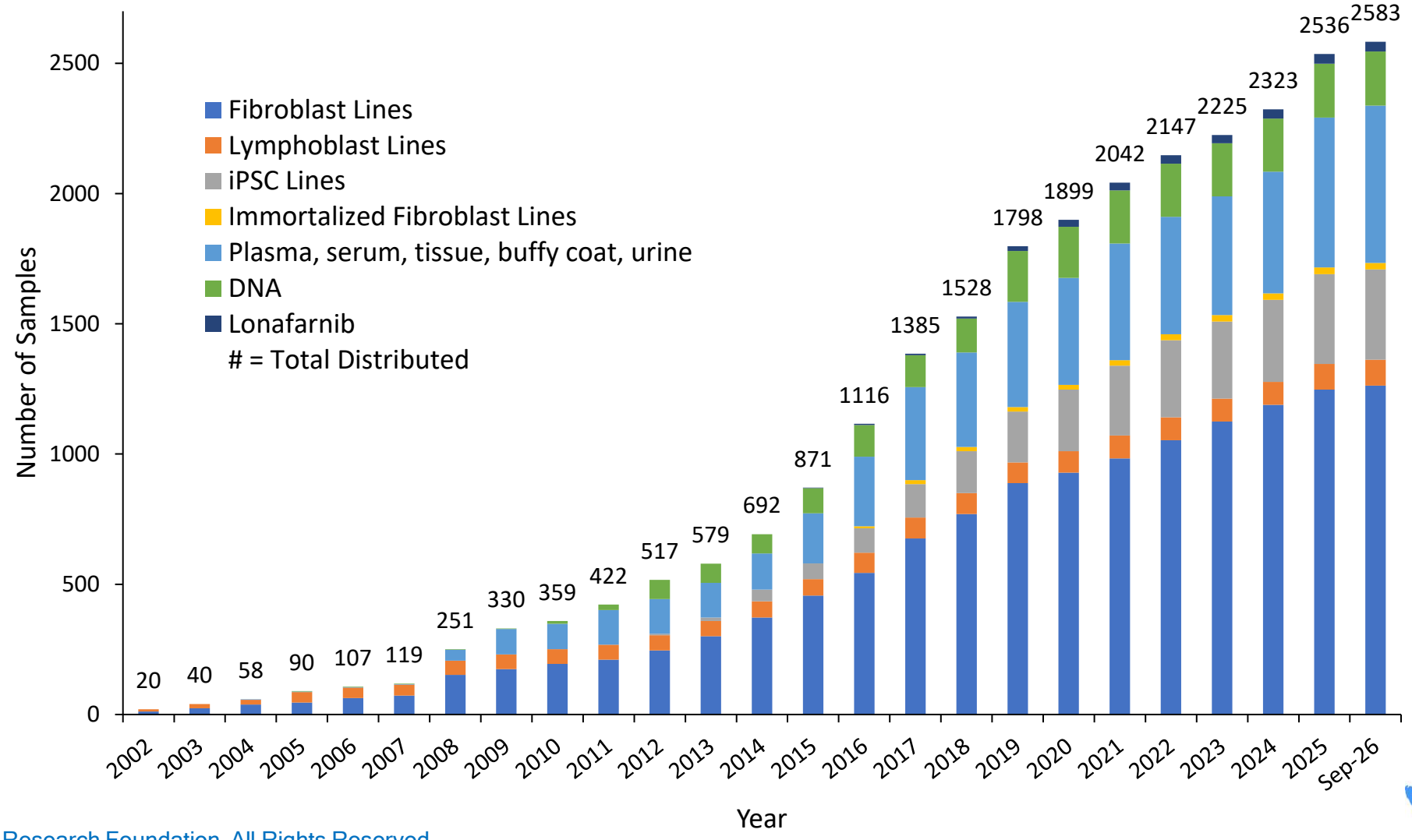
Number Of Cell Lines By Year



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Cumulative Number of Biological Samples Distributed



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Cell and Tissue Bank Recipients

Cells and biological material have been distributed to
267 laboratories in **29** countries
Complete list of researchers available at: https://www.progeriaresearch.org/cell-and-tissue-bank-recipients

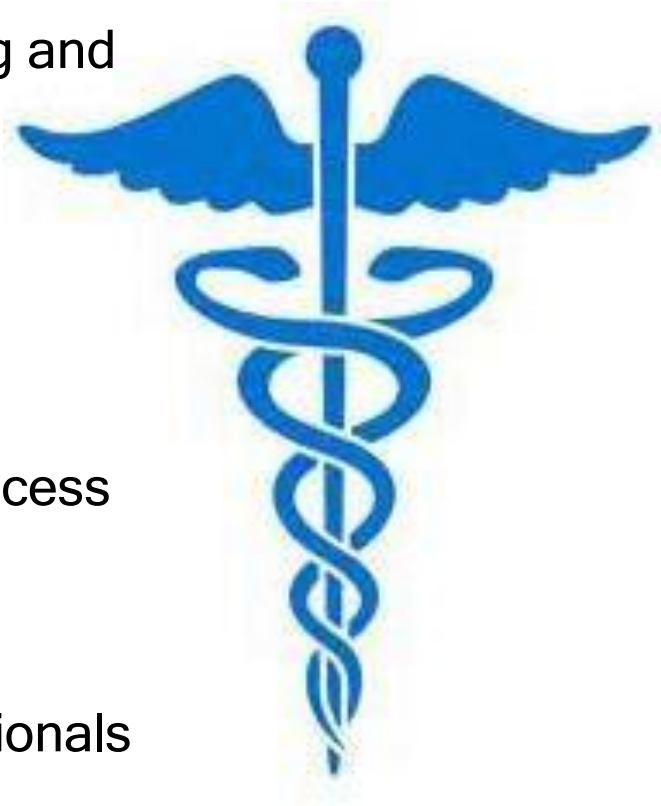


© 2025 The Progeria Research Foundation. All Rights Reserved.

PRF Medical & Research Database

Program Goals:

- Collect the patient health records for living and deceased children with Progeria
- Obtain long-term clinical data
- Abstract data for longitudinal and cross-sectional analyses
- Better understand the clinical disease process in Progeria and aging related diseases
- Develop treatment strategies and recommendations for health care professionals and families



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

How The PRF Medical & Research Database Works

- Project staff obtain the patient's medical records and film studies from birth throughout the participant's lifespan.
- Medical records include visits to: primary care physicians, specialty physicians, hospital emergency rooms, hospital admissions, dentists, physical therapy, occupational therapy and school health records.
- Retrospective data abstraction protocol allows for specifically targeted or broad spectrum of data.

Enrollment information available at: https://www.progeriaresearch.org/medical-database/

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Medical & Research Database Participation

225 Participants are enrolled from 57 countries and 1 US territory

Algeria
Argentina
Australia
Bangladesh
Belgium Brazil
Canada, Chile
China, Columbia
Denmark
Dominica Republic
Egypt, England,
Ethiopia
France, Germany
Guatemala,
Guyana
Honduras, India
Indonesia, Ireland
Israel, Italy
Japan, Kazakhstan

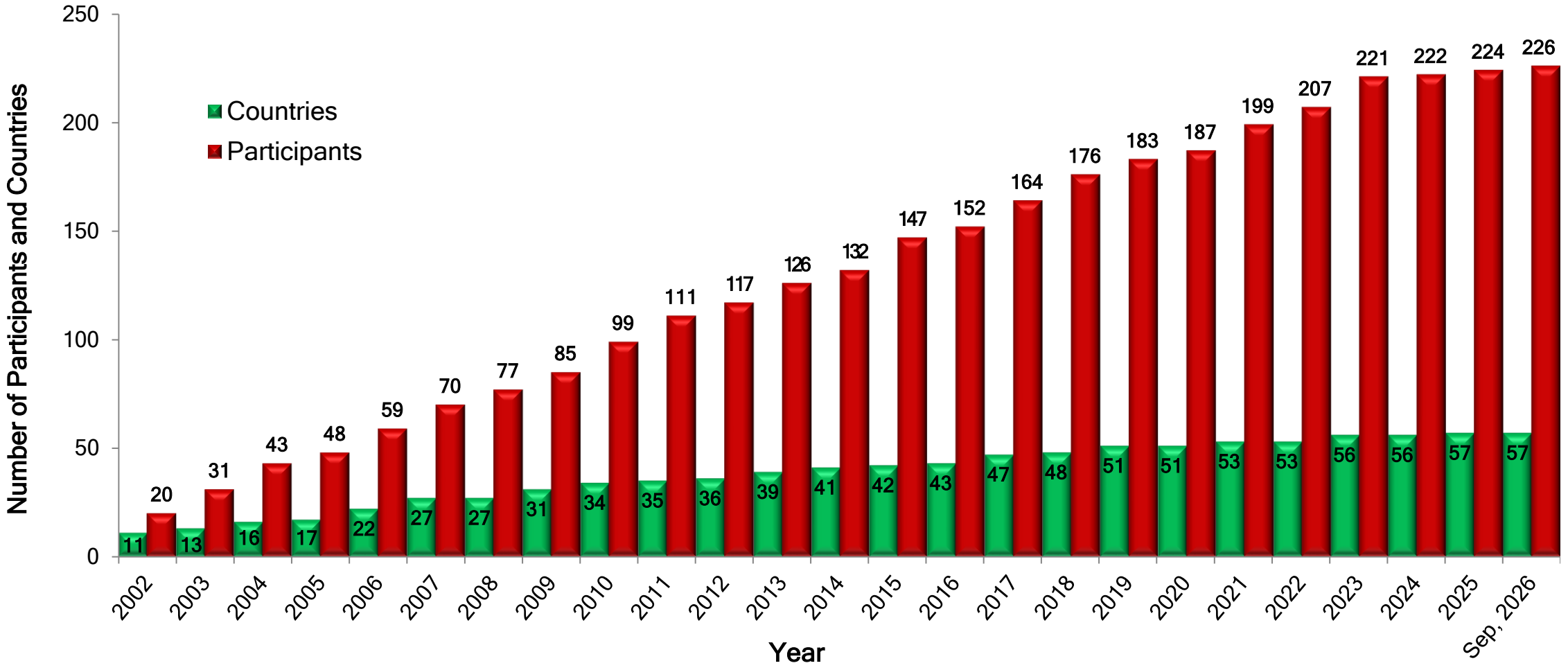


Libya, Mexico
Morocco, Nepal
Netherlands
Oman, Pakistan
Papua New Guinea
Peru, Philippines
Poland, Portugal
Puerto Rico
Romania, Russia
Senegal, Serbia
South Africa
South Korea
Spain, Sri Lanka
Suriname, Sweden
Tanzania, Togo
Turkey, Ukraine
USA, Venezuela
Vietnam

PROGERIA RESEARCH FOUNDATION

© 2026 The Progeria Research Foundation. All Rights Reserved.

Medical & Research Database Longitudinal Enrollment

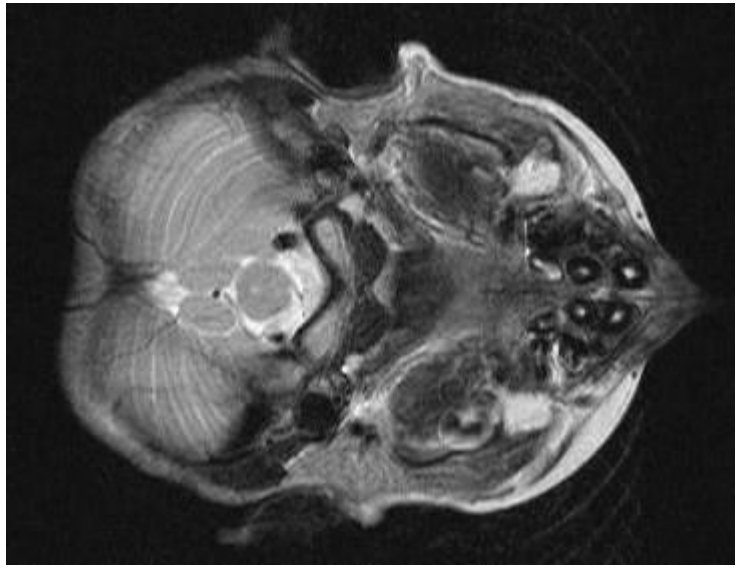


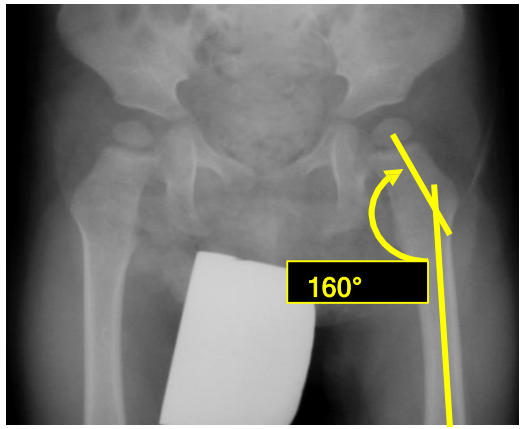
© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Types Of Data Collected

- Participants with Medical Records Reports: **166**
- Participants with Radiology Studies: **65**





© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Weighing-In Program

- A sub-program of The PRF Medical & Research Database
- Collects weight-for-age data prospectively:
  - Home scale provided by PRF
  - Parents weigh child weekly or monthly
  - Report weights electronically



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Weighing-In Program Participation

122 Participants are enrolled from 42 countries and 1 US territory

Argentina, Australia
Bangladesh
Belgium, Brazil
Canada, China
Colombia, Denmark
Dominican Republic
England, Germany
Guatemala
Honduras, India
Indonesia, Ireland
Israel, Italy, Japan
Mexico, Morocco

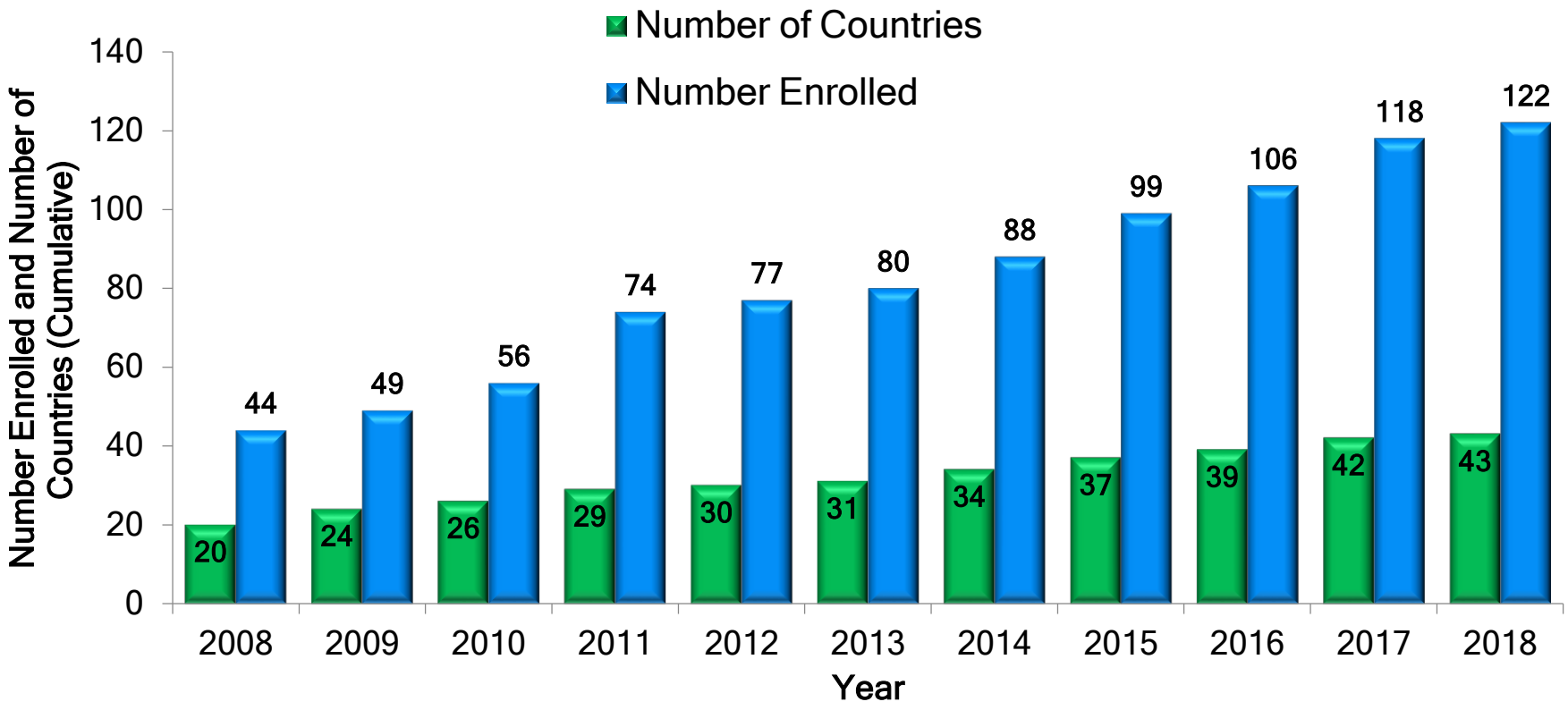


Nepal, Pakistan
Peru, Philippines
Poland, Portugal
Puerto Rico
Romania, Russia
Senegal
South Africa
South Korea
Spain, Sri Lanka
Togo, Tanzania
Turkey, Ukraine
USA, Venezuela
Vietnam

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Participants Enrolled In The PRF Weighing-In Program and Countries of Residence



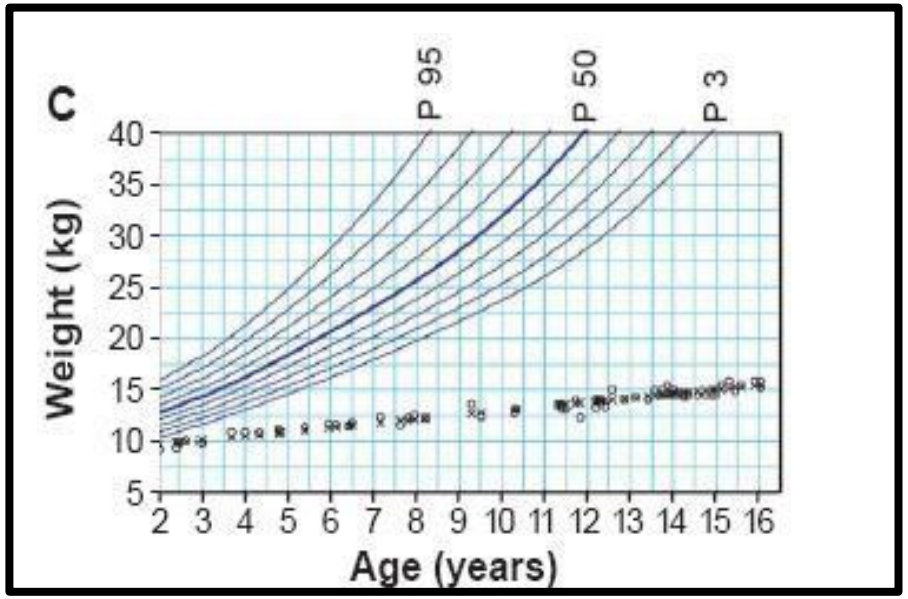
© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Clinical Trials And The Weighing-In Program

- Data from this program were key in the development of primary outcome measure for the first drug treatment trial for Progeria.

- As of December 1, 2018, 90 children from The PRF Weighing-In Program have entered clinical treatment trials using this data.



Failure to Thrive Starts Towards End of Year One

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF-Funded Clinical Treatment Trials



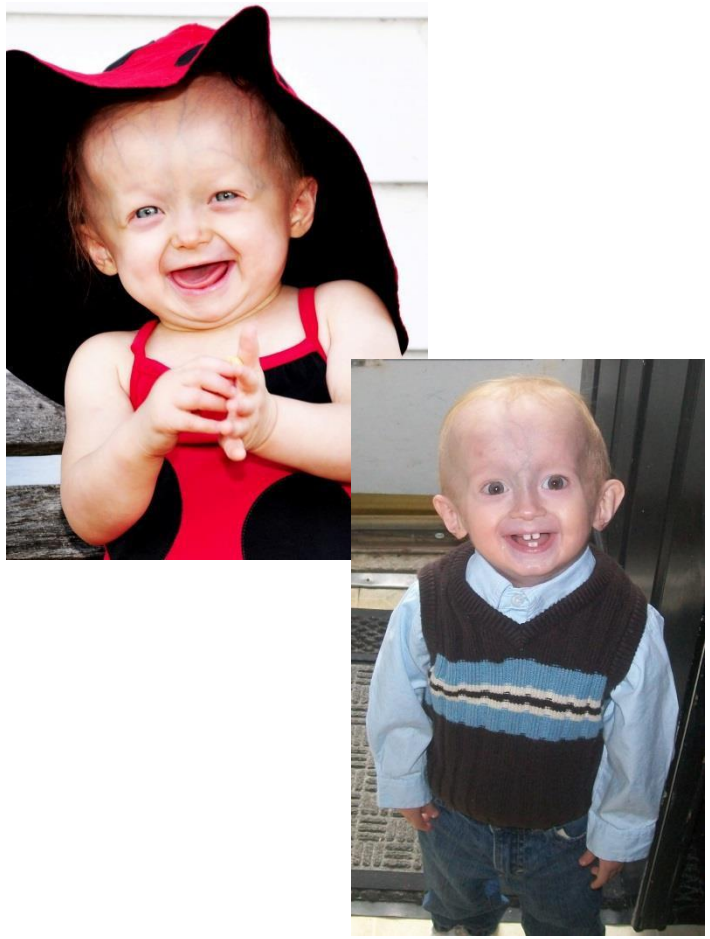
© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Clinical Drug Treatment Trials

Goals:

- To define the natural history of HGPS in quantifiable terms that will expand our ability to measure treatment outcome
- To assess the safety of new treatments for HGPS
- To measure effects of treatments for children with HGPS on disease status, changes in health, and survival



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Participation in PRF Clinical Trials

109 Children have participated in PRF Clinical Trials from **42** countries

Argentina
Australia
Bangladesh
Belgium, Brazil
Canada, China
Colombia
Czech Republic
Denmark
Dominican Republic
Egypt, England
France
Germany
Greece
Guyana
Honduras
India, Israel
Italy, Japan
Kazakhstan
Libya, Malaysia
Mexico

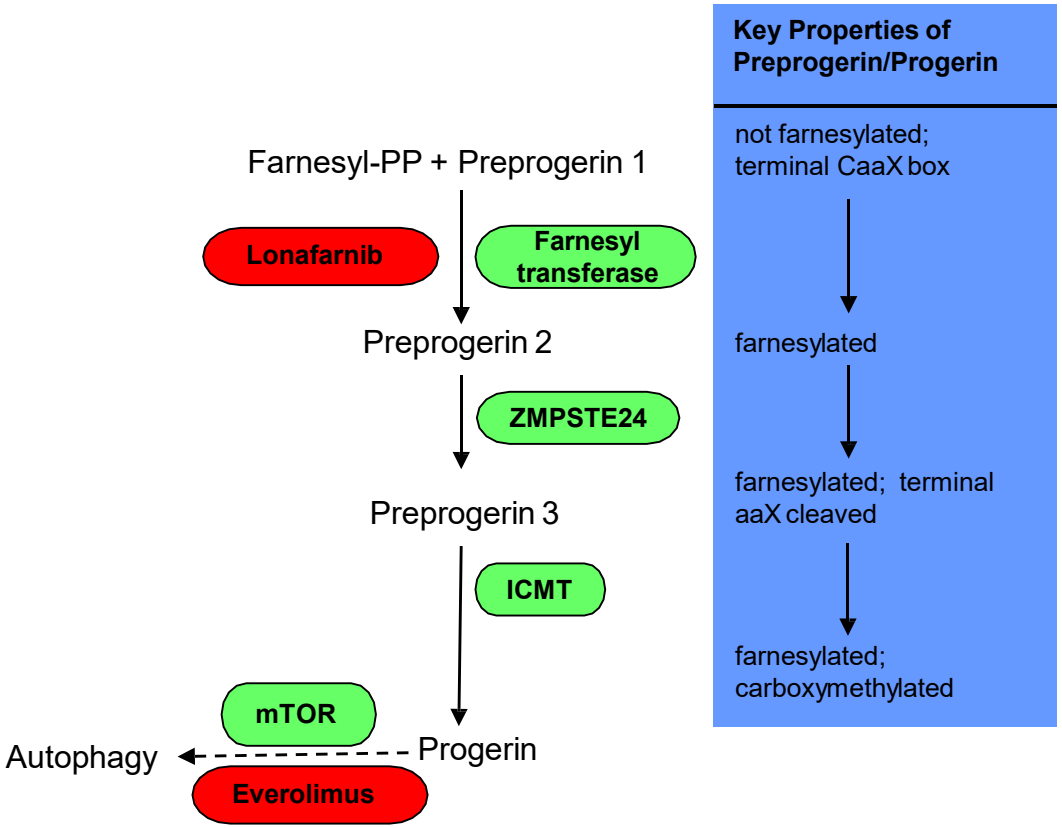


Morocco, Oman
Pakistan
Peru, Philippines
Poland, Portugal
Romania
Russia, Serbia
South Africa
South Korea
Spain, Sri Lanka
Suriname
Sweden
Tanzania
Togo
Turkey
Ukraine
USA
Venezuela

PROGERIA RESEARCH FOUNDATION

© 2026 The Progeria Research Foundation. All Rights Reserved.

Current Therapeutic Intervention Strategies



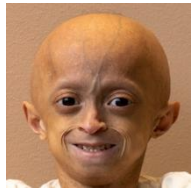
Post-translational processing and medications currently under investigation in clinical treatment trials for Progeria. Items in green = enzymes. Items in red = clinical trial medications that inhibit corresponding enzymes. Lonafarnib is a farnesyltransferase inhibitor. Everolimus is a rapamycin analogue that inhibits mTOR and promotes cellular autophagy. FT=farnesyltransferase.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF-Funded Clinical Treatment Trials

Children & young adults have flown from all over the world to participate in clinical treatment trials 1-5 at the Boston Children's Hospital

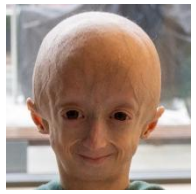













Trial #	Year	Drug(s)	Phase	# Enrolled	Countries Represented
1	2007-2010	Lonafarnib	2	29	16
2	2009	Lonafarnib, Pravastatin, Zoledronate	Feasibility	5	2
3	2009-2013	Lonafarnib, Pravastatin, Zoledronate	2	45	24
	2014-2021	Lonafarnib	2 (Extension)	71	32
4	2016-2023	Lonafarnib, Everolimus	1/2	60	27
	2018-Present	Lonafarnib	2 (Extension)	98*	33 *
5	2025-Present	Progerinin, Lonafarnib	2	10 *	7 *

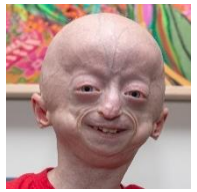


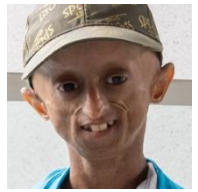











© 2026 The Progeria Research Foundation. All Rights Reserved.

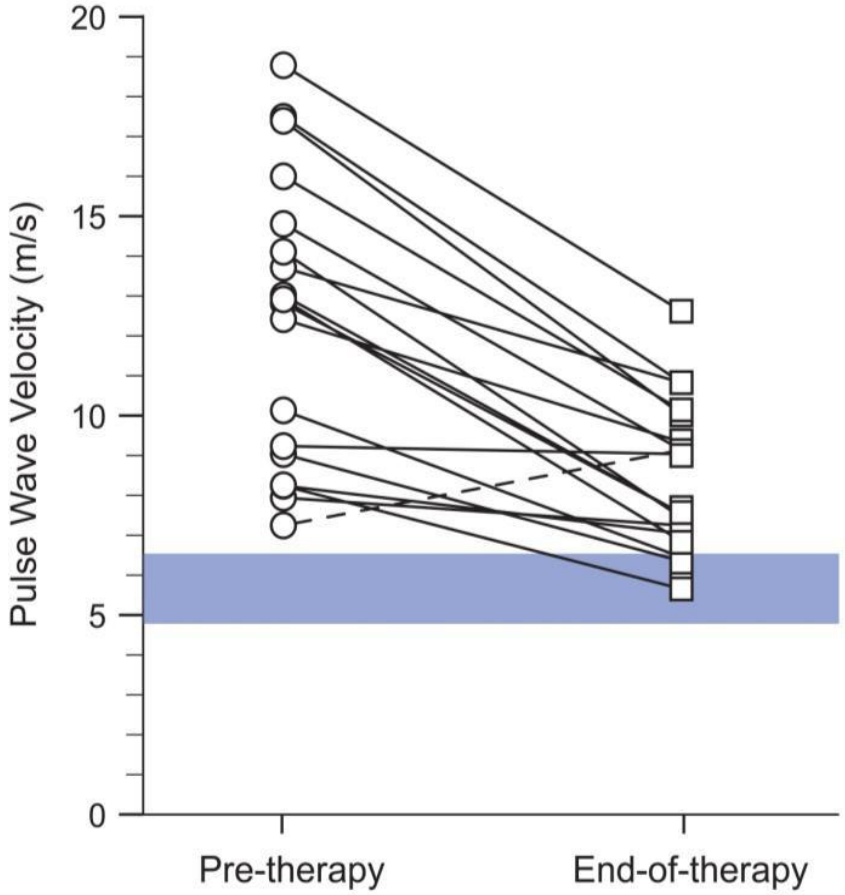
* As of September 30, 2026

Clinical Treatment Trial Efficacy Results

Lonafarnib, a type of farnesyltransferase inhibitor (FTI) is our first treatment for Progeria

- Results showed improvement in:
  - Rate of weight gain
  - Increased vascular distensibility
  - Improved bone structure
  - Better neurosensory hearing
  - Increased Lifespan

Gordon et al, PNAS, 2011

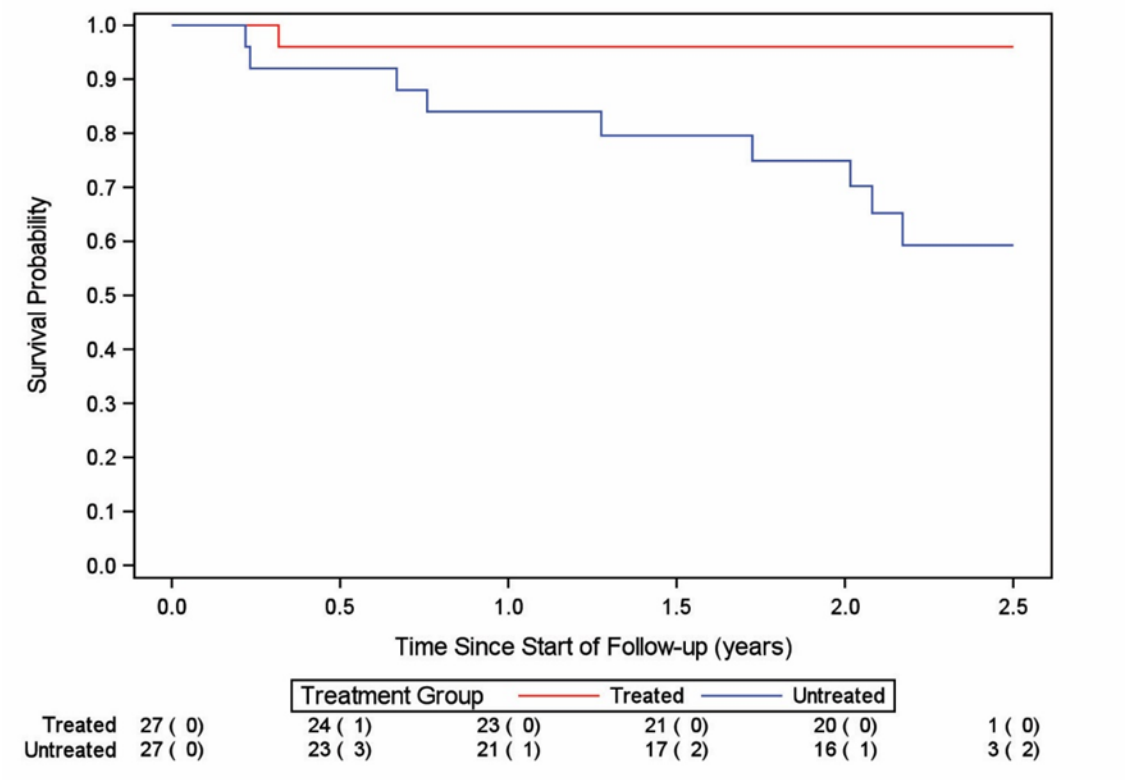


© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Positive Effects of Lonafarnib (Zokinvy) on Progeria: Results of PRF's Clinical Treatment Trials

- Average increased lifespan of 4.3 years
- Increased vascular distensibility
- Improved bone structure
- Better neurosensory hearing
- Modest increase in rate of weight gain



Gordon et al., PNAS, 2011 and
Gordon et al., JAMA, 2018
Gordon et al., Circulation 2023

JAMA | Preliminary Communication

Association of Lonafarnib Treatment vs No Treatment With Mortality Rate in Patients With Hutchinson-Gilford Progeria Syndrome

Leslie B. Gordon, MD, PhD; Heather Shappell, PhD; Joe Massaro, PhD; Ralph B. D'Agostino Sr, PhD; Joan Brazier, MS; Susan E. Campbell, MA; Monica E. Kleinman, MD; Mark W. Kieran, MD, PhD

© 2026 The Progeria Research Foundation. All Rights Reserved.

3 Ways to Access Lonafarnib (Zokinvy)

Zokinvy (lonafarnib) is an FDA approved drug in the US. It is the current standard of care for those with Progeria. It is available through

1. Prescription in the US and in select non-US countries
2. PRF's clinical treatment trials
3. The manufacturer's (Sentynl) Managed Access Program in select non-US countries

We are far from finished!
We must forge ahead with increased intensity and collaborative efforts to find additional treatments, and the cure!



Please contact PRF at info@progeriaresearch.org for more information on how to access Zokinvy (lonafarnib) for your child or patient with Progeria

PROGERIA RESEARCH FOUNDATION

© 2026 The Progeria Research Foundation. All Rights Reserved.

Treatment Trial Collaborations For Success

- **The children are seen by physicians from:**
  - Boston Children's Hospital
  - Dana-Farber Cancer Institute
  - Brigham and Women's Hospital
- **Data were also generated by scientists from:**
  - Alpert Medical School at Brown University
  - Brown University School of Public Health
  - University of California Los Angeles
  - National Human Genome Research Institute
  - Schering-Plough Research Institute
- **Lonafarnib generously provided by Sentynl**
- **Everolimus generously provided by Novartis**

© 2026 The Progeria Research Foundation. All Rights Reserved.

Clinical Trial Publications

Drug Effect:

Plasma Progerin in Patients With Hutchinson-Gilford Progeria Syndrome: Immunoassay Development and Clinical Evaluation. Gordon et al., *Circulation*, 2023;147(23):1734-1744

FDA approval summary for lonafarnib (Zokinvy) for the treatment of Hutchinson-Gilford progeria syndrome and processing-deficient progeroid laminopathies. Suzuki et al., *Genetics in medicine*, 2023;25(2):100335

Association of Lonafarnib Treatment vs No Treatment With Mortality Rate in Patients With Hutchinson-Gilford Progeria Syndrome. Gordon et al., *JAMA*, 2018, 319(16):1687-1695.

Survey of Plasma Proteins in Children with Progeria Pre-therapy and On-Therapy with Lonafarnib. Gordon et al., *Pediatric Research*, 2018;83(5):982-992

Clinical Trial of the Protein Farnesylation Inhibitors Lonafarnib, Pravastatin, and Zoledronic Acid in Children With Hutchinson-Gilford Progeria Syndrome. Gordon et al., *Circulation*, 2016 Jul 12;134(2):114-25.

Seeking a Cure for One of the Rarest Diseases: Progeria. Collins, *Circulation,* 2016 Jul 12;134(2):126-9.

Impact of Farnesylation Inhibitors on Survival in Hutchinson-Gilford Progeria Syndrome. Gordon et al., *Circulation*, 2014 Jul 1;130(1):27-34.

Moving from Gene Discovery to Clinical Trials in Hutchinson-Gilford Progeria Syndrome. King et al., *Neurology*, 2013 Jul 30;81(5):408-9.

Neurologic Features of Hutchinson-Gilford Progeria Syndrome after Lonafarnib Treatment. Ullrich et al., *Neurology*, 2013, 81:427-430.

Clinical Trial of a Farnesyltransferase Inhibitor in Children with Hutchinson-Gilford Progeria Syndrome. Gordon et al., *Proceedings of the National Academy of Sciences*, 2012 Sep 24.

General:

Hutchinson-Gilford Progeria Syndrome. Gordon et al., 2003 Dec 12 [Updated 2025 Mar 13]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025.

Clonal hematopoiesis is not prevalent in Hutchinson-Gilford progeria syndrome. Díez-Díez, et al., *GeroScience*. 2023;45(2):1231-1236

Pubertal Progression in Adolescent Females with Progeria. Greer et al., *Journal of Pediatric and Adolescent Gynecology*, 2018;31(3):238-241

Phenotype and Course of Hutchinson-Gilford Progeria Syndrome. Meredith et al., *New England Journal of Medicine*, 2008, 358(6): 592- 604.

Dermatology:

Initial Cutaneous Manifestations of Hutchinson-Gilford Progeria Syndrome. Rork et al., *Pediatric Dermatology*, 2014,1-7.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Clinical Trial Publications Continued

Dental:

Microbiome at Sites of Gingival Recession in Children with Hutchinson-Gilford Progeria Syndrome. Bassir et al., *Journal of Periodontology*. 2018, 89(6): 635-644.

Hutchinson-Gilford Progeria Syndrome: Oral and Craniofacial Phenotypes. Domingo et al., *Oral Diseases*, 2009, 15(3): 187-195.

Cerebrovascular:

Imaging Characteristics of Cerebrovascular Arteriopathy and Stroke in Hutchinson-Gilford Progeria Syndrome. Silvera et al., *American Journal of Neuroradiology*, 2013 May;34(5):1091-7.

Cardiology:

Longitudinal Changes in Myocardial Deformation in Hutchinson-Gilford Progeria Syndrome. Olsen et al., *Circ Cardiovasc Imaging*. 2025;18(2)

Abnormal Myocardial Deformation Despite Normal Ejection Fraction in Hutchinson-Gilford Progeria Syndrome. Olsen, et al. *J Am Heart Assoc*. 2024;13(3)

Progression of Cardiac Abnormalities in Hutchinson-Gilford Progeria Syndrome: A Prospective Longitudinal Study. Olsen FJ, et al. *Circulation*. 2023;147(23):1782-1784.

Cardiac Abnormalities in Patients With Hutchinson-Gilford Progeria Syndrome. Prakask, et al., *JAMA Cardiology*, 2018, Apr 17;115(16):4206-4211.

Mechanisms of Premature Vascular Aging in Children with Hutchinson-Gilford Progeria Syndrome. Gerhard-Herman M, et al., *Hypertension*. 2012;59(1):92-97.

Skeletal:

Lonafarnib Clinical Trials Demonstrate Uncoupling of the Muscle-Bone Unit in Hutchinson-Gilford Progeria Syndrome. Kreienkamp et al., *J Bone Miner Res.* Published online December 4, 2025.

Baseline Range of Motion, Strength, Motor Function, and Participation in Youth with Hutchinson-Gilford Progeria Syndrome. Malloy et al., *Phys Occup Ther Pediatr*. 2023 Jan 10:1-20.

Skeletal maturation and long-bone growth patterns of patients with Progeria: a retrospective study. Tsai et al., *The Lancet. Child and Adolescent Health*. 2020 Apr;4(4):281-289.

Extraskeletal Calcifications in Hutchinson-Gilford Progeria Syndrome. Gordon et al., *Bone.* 2019 Aug;125:103-111.

Craniofacial Abnormalities in Hutchinson-Gilford Progeria Syndrome. Ullrich et al., *American Journal of Neuroradiology*. 2012 Sep;33(8):1512-8.

A Prospective Study of Radiographic Manifestations in Hutchinson-Gilford Progeria Syndrome. Cleveland et al., *Pediatric Radiology,* 2012 Sep;42(9):1089- 98. Epub 2012 Jul 1.

Hutchinson-Gilford progeria is a skeletal dysplasia. Gordon, et al., *Journal of Bone and Mineral Research*. 2011 Jul;26(7):1670-9.

Ophthalmology:

Ophthalmologic Features of Progeria. Mantagos et al., *American Journal of Ophthalmology,* 2017 Oct;182:126-132.

Audiology:

Otologic and Audiologic Manifestations of Hutchinson-Gilford Progeria Syndrome. Guardiani et al., *The Laryngscope*, 2011, 121(10): 2250-2255.

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Progerin as a Biomarker for Progeria

- Progerin is the toxic protein produced by cells with progeria in place of the normal lamin A protein
- Normal lamin A plays a role in cell division, but the toxic progerin gets stuck to the nuclear membrane of cells, and its accumulation causes progeria
- A biomarker is “a biological molecule found in blood, other body fluids, or tissues that is a sign of a normal or abnormal process, or of a condition or disease” (National Cancer Institute)
- Treatments for progeria aim to decrease the amount of progerin produced by the body.



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Publication of the Progerin Biomarker

research-article

Circulation

ORIGINAL RESEARCH ARTICLE

Plasma Progerin in Patients With Hutchinson-Gilford Progeria Syndrome: Immunoassay Development and Clinical Evaluation

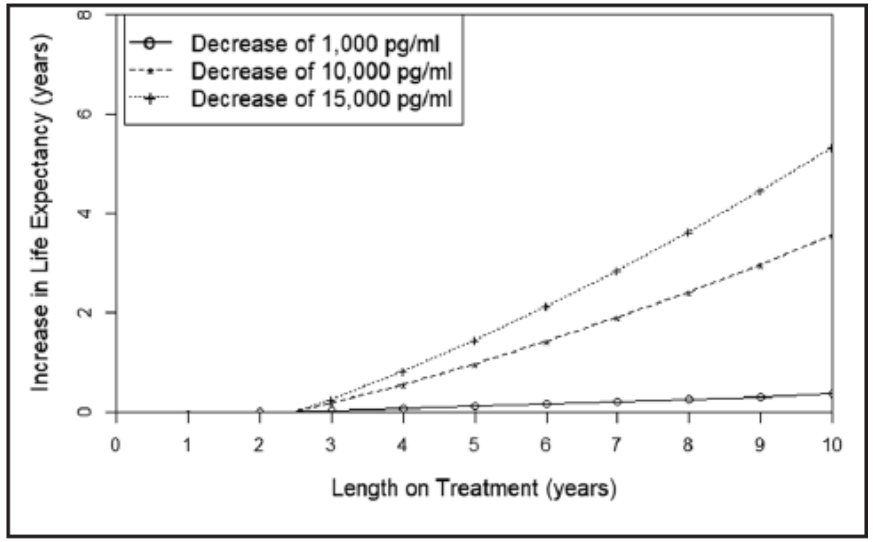
Leslie B. Gordon, MD, PhD; Wendy Norris, MS; Sarah Hamren, BS; Robert Goodson, BS; Jessica LeClair, PhD; Joseph Massaro, PhD; Asya Lyass, PhD; Ralph B. D'AgostinoSr, PhD; Kelsey Tuminelli, MS; Mark W. Kieran, MD, PhD; Monica E. Kleinman, MD

Dr. Gordon *et al.* 2023 developed an assay to measure progerin in blood plasma

© 2026 The Progeria Research Foundation. All Rights Reserved.

Further Findings of the Progerin Biomarker Study

- Plasma progerin levels were 95x higher in those with progeria than in the average healthy human
- Treatment with lonafarnib decreased progerin levels by 35-62% on average
- Lifespan increase was shown to be linked to decreased progerin levels and longer time on treatment
- Long-term treatment with lonafarnib (10+ years) resulted in a lifespan increase of about 35%



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Grant Funding

As of September 30, 2026:

- Since inception 281 grant application received and 85 funded
- PRF has funded 69 principal investigators from 55 institutions in 14 countries



Lamina A, progerin, Lamin B in HGPS and aging

Genetics and nuclear function

Preclinical Drug Therapy

Molecular Abnormalities and Therapies

Vascular Pathology

Mouse Models

Stem Cell Investigations and Therapy

Clinical Trials

© 2026 The Progeria Research Foundation. All Rights Reserved.

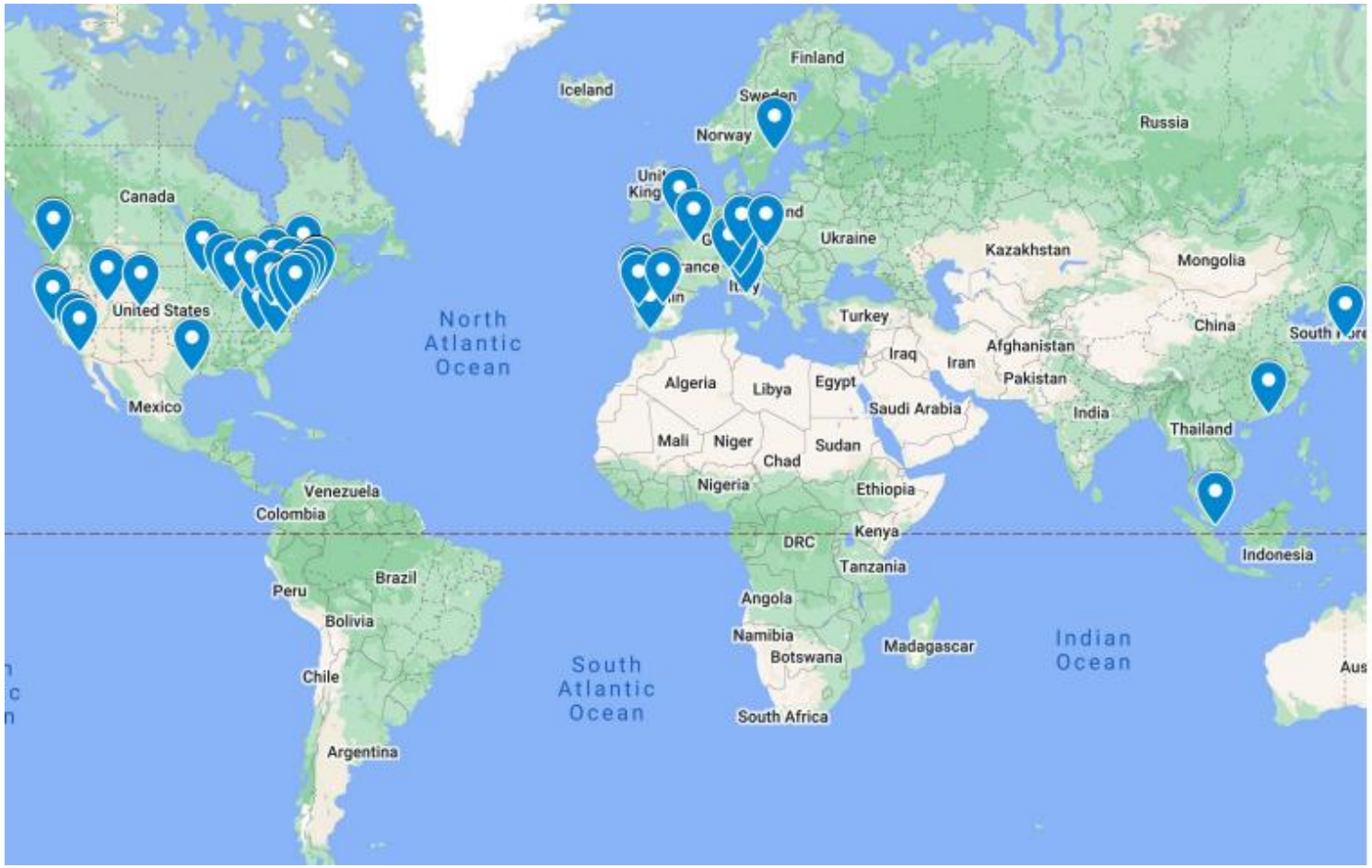
* Submissions include Letters of Intent and Full Grants

PROGERIA RESEARCH FOUNDATION

PRF Grantees

PRF has funded **69** principal investigators from **55** institutions in **14** countries

Complete list of Grantees available at: https://www.progeriaresearch.org/grants-funded/



© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

PRF Scientific Meetings

Meeting Goals:

- October 2025, The Progeria Research Foundation (PRF) held its 12th International Scientific Workshop in Boston.
- To share groundbreaking discoveries, strengthen collaborations, and discuss the next phase of research. Including a focus on developing new treatments, and ultimately, the cure for Progeria.



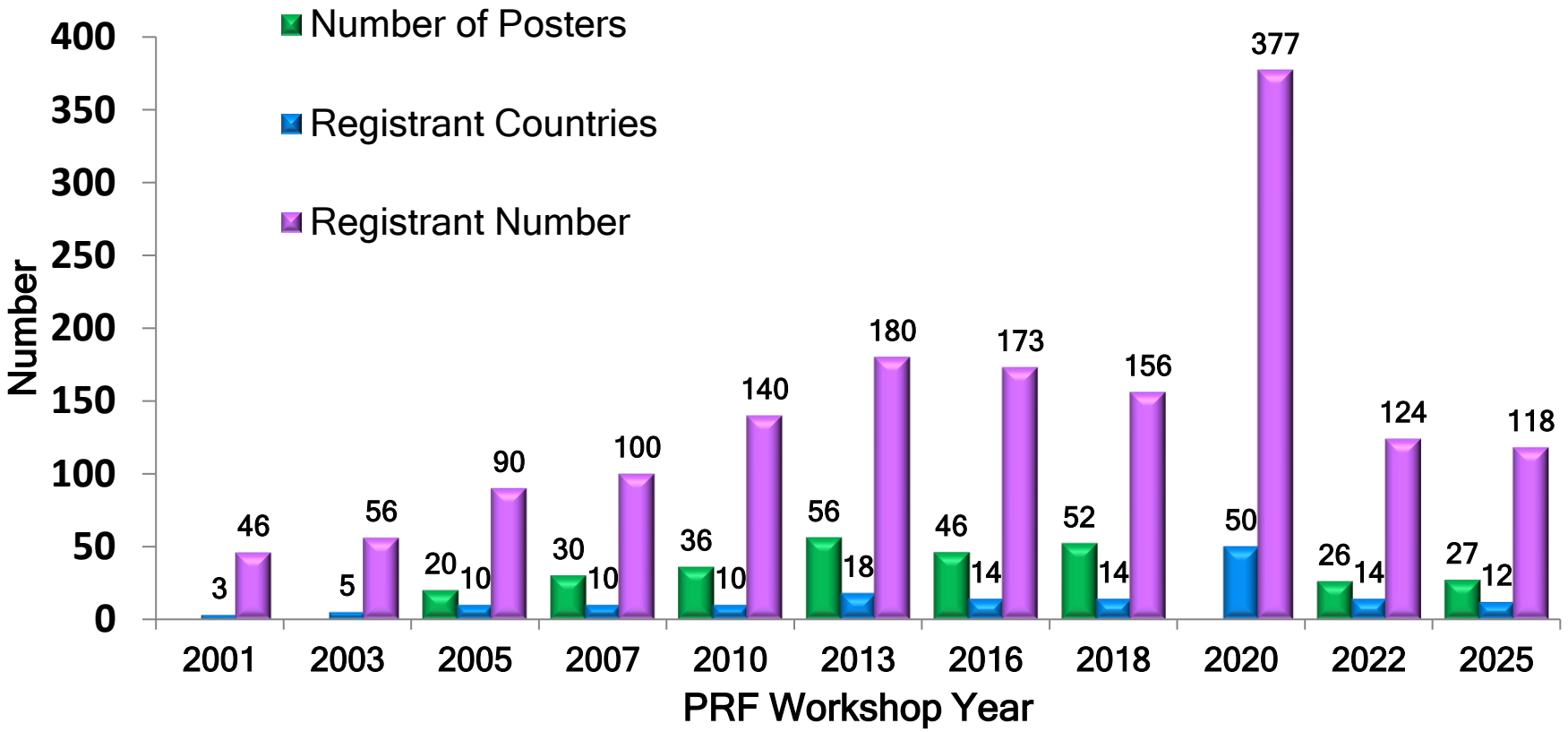
© 2026 The Progeria Research Foundation. All Rights Reserved.

International Workshops Promoting Global Interest In Progeria, Cardiovascular Disease And Aging

- These are large multi-day workshops open to all scientists. Clinical and basic researchers spend intense days sharing data and planning new collaborations for progress towards treatments and cure.
- Various NIH Institutes have funded all international workshops through R13 and other granting mechanisms
- Other organizations have also generously sponsored workshops

© 2026 The Progeria Research Foundation. All Rights Reserved.

Growth of Global Interest In PRF Workshops



* 2020 was a webinar. Posters N/A

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Subspecialty Scientific Meetings

Small, focused meetings designed to promote and support work in areas of high interest for Progeria

First Genetics Consortium Meeting – "Searching for the Progeria Gene", August 23, 2002, Brown University, Providence, RI

Second Genetics Consortium Meeting – "Post- gene Discovery", July 30, 2003, Bethesda, MD

Bone Marrow Transplant Meeting – "Forging Ahead by Exploring Potential Treatments", April 25-26, 2004, National Institutes of Health, Bethesda, MD

New Frontiers in Progeria Research (2012), Boston, MA

The first "Progeria Aortic Stenosis Intervention Summit", May 2, 2023, Virtual by Zoom



NIH National Heart, Lung, and Blood Institute

NIH National Human Genome Research Institute

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Scientific Publications

As of September 30, 2026:

199 Scientific articles have been published citing The Progeria Research Foundation Grants Funding Program

164 Scientific articles have been published citing PRF Cell & Tissue Bank resources:

- Publication list at https://www.progeriaresearch.org/prf-cell-and-tissue-bank-publications/

36 Scientific articles have been published citing The PRF Medical & Research Database:

- Publication list at www.progeriaresearch.org/medical-database/

32 Scientific articles have been published from clinical trial data

- See slide #54 and #55

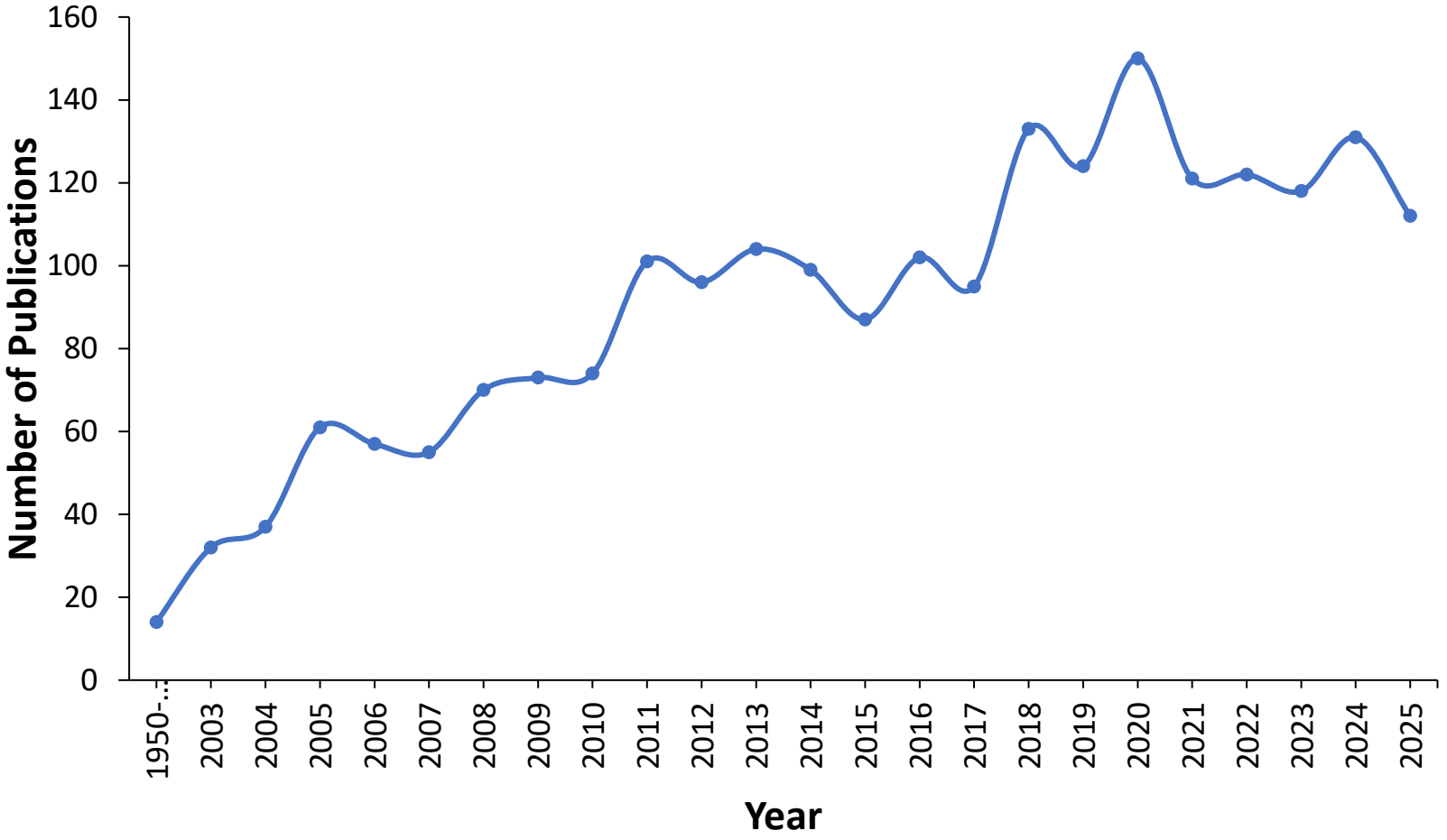
5 Scientific articles have been published concerning PRF Scientific Workshops

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Progeria Related Publications

Today over 100 publications on Progeria per year are published in well known and peer-reviewed scientific journals read by researchers worldwide.

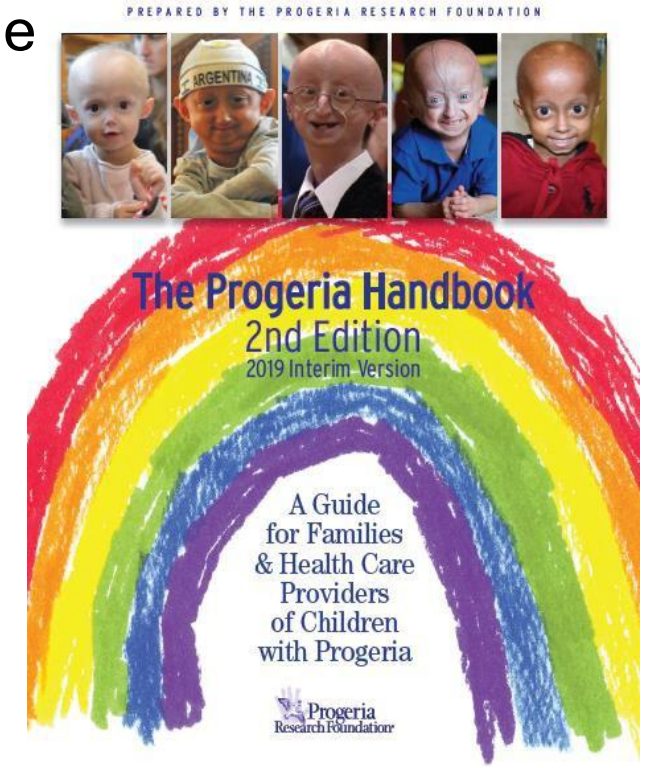


© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

Progeria Clinical Care Handbook

The Progeria Handbook 2nd Edition. A Guide for Families & Health Care Providers of Children with Progeria. *The Progeria Research Foundation.* Leslie B. Gordon MD, PhD; Medical Director (editor) 2019.



- Provided in Chinese, English, Italian, Japanese, Korean, Portuguese and Spanish
- Expert contributors from Boston Children's Hospital
- Number of Progeria Care Handbooks distributed to families of those with Progeria and their care givers: **1011**

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION

The Progeria Research Foundation

Finding…
Diagnosing…
Studying…
Treating…
CURING



Progeria Research Foundation
FOR THE CHILDREN ♥ FOR THE CURE

Together We *WILL* Find The Cure!

www.progeriaresearch.org

© 2026 The Progeria Research Foundation. All Rights Reserved.

PROGERIA RESEARCH FOUNDATION