Progeria:
Progress toward the Cure

What is Progeria?

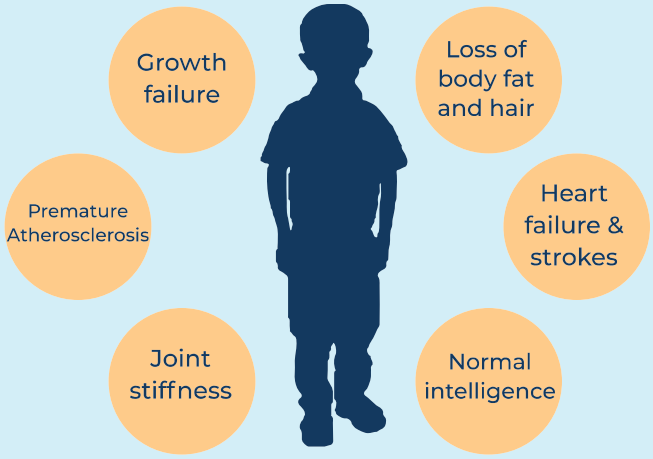
(HGPS)

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

Without treatment, children with Progeria die of heart disease at an average age of 14.5 years.

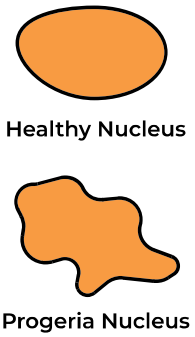
While there is currently no cure, there is a life-extending treatment.

Features



What causes Progeria?

A mutation in the *LMNA* gene creates a protein, called **progerin,** which makes the nucleus of a cell unstable and causes premature aging.



1 in 18 million
people have Progeria.

PRF knows of more than 130 children living with Progeria in 50 countries.

PRF estimates that there are 400 children and young adults with Progeria worldwide.

Together, we *WILL* find the cure.

Progeria Research Foundation
FOR THE CHILDREN ♥ FOR THE CURE

To learn more, visit www.ProgeriaResearch.org
Email info@progeriaresearch.org
Call (978) 535-2594

ABOUT THE PROGERIA RESEARCH FOUNDATION (PRF)

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

PRF'S Programs and Services

Clinical Trials

PRF works with Boston Children's Hospital to test the most promising treatments.

Diagnostic Testing

PRF offers free genetic testing for children suspected of having Progeria.

Research Funding

PRF has funded nearly $9M in novel research pathways toward treatments and the cure.

Cell & Tissue Bank

PRF has distributed over 1,200 samples to advance Progeria research.

International Scientific Workshops

PRF hosts conferences for world-class researchers to collaborate & share breakthrough findings.

International Patient Registry

PRF's patient registry assures children with Progeria and their families stay connected to all PRF activities.

Treatment Guidelines Handbook

PRF has created clinical guidelines to help caretakers and doctors care for children and young adults with Progeria.

Public Awareness

PRF has reached tens of millions through its newsletters, website, media and events.

Treatment Discovery & FDA Approval

In **2020**, in partnership with Eiger BioPharmaceuticals, PRF achieved FDA approval of lonafarnib (Zokinvy), the **first-ever FDA-approved treatment** for Progeria. This historic milestone is the result of 13 years of PRF-funded clinical trials.

Zokinvy extends lifespan by an average of at least **2.5 years.**

Progress toward the CURE

PRF has made **tremendous progress** funding **breakthrough research** as we charge ahead toward the cure for Progeria.

Mouse studies in **gene editing, RNA therapeutics,** and **small molecule therapies** have shown dramatic results that we hope will soon lead to clinical trials.

Together, we *WILL* find the cure.

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