

Progeria Research Foundation



P.O. Box 3453
Peabody, MA 01961-3453
(978) 535-2594
www.progeriaresearch.org



Mission

The Progeria Research Foundation (PRF) was created to raise awareness about Hutchinson-Gilford Progeria Syndrome and to educate and help families, their doctors, researchers, and the general public. The foundation's mission is to discover treatments and the cure for progeria and its aging-related disorders. PRF funds medical research and runs research-related programs specifically aimed at finding the cause, treatments, and cure for this syndrome.

Strategy/Approach

Before PRF was formed in 1999, progeria was an obscure disease that few people knew about. A large part of PRF's strategy has been disseminating information and raising awareness about progeria through various communications efforts. In order to facilitate cures for progeria, PRF runs an international patient registry, a cell and tissue bank, a diagnostics testing program, and a medical research database. The foundation also organizes scientific workshops, gives medical research grants, and funds and coordinates clinical trials.

Key PRF programs include:

- **Progeria Clinical Trial Drugs and Treatment** bring children from around the world promising treatments that may help to improve disease and even extend the lives of children with progeria.
- The **International Progeria Registry** maintains centralized information on children and families living with progeria. This assures rapid distribution of any new information that may benefit the children.
- The **Cell & Tissue Bank** provides researchers with genetic and biological material from progeria patients and their families to facilitate progeria research.
- The **Translation Program** eliminates barriers to communication for non-English-speaking patients and their families around the world by translating PRF program materials into 20 languages.
- Through the **FindTheOther150** campaign, PRF and its partner, GlobalHealthPR, work to identify every child suffering from progeria worldwide.

Research Portfolio

Types of grants include:

- **Innovator Awards** – For investigators embarking on new lines of investigation and to produce enough

preliminary data to be competitive for longer-term funding by other agencies

- *\$75,000 over two years*
- **Established Investigator Awards** – for senior investigators established either in the field of progeria or a field that can be directly applied to progeria
 - *\$100,000 over three years*
- **Specialty Awards** – For smaller, technology-driven projects
 - *Flexible funding*

Partnership Practices

PRF partners with biotech and pharmaceutical companies.

For the first-ever progeria clinical drug trial in 2007, PRF partnered with Schering-Plough Pharmaceuticals. Also, PRF is funding a progeria trial that repurposes the cancer drug lonafarnib, which is donated by Merck. PRF has also received sponsorship from pharmaceutical companies.

Financials

PRF is a 501(c)(3) charitable organization. The most recent financial information available is from 2012.¹

Year ending 12/31/2012:

- Revenue: \$1,806,700
- Medical Research Grants: \$509,445
- Gifts received (individuals and private foundations): \$758,814
- Expenditures: \$1,575,560

Key Accomplishments

- PRF has awarded 54 grants totaling more than \$5 million.
- Since 1999, PRF has raised more than \$12.67 million toward gene discovery and clinical trials.
- In 2012, through a PRF-sponsored clinical trial, history was made with the discovery that a farnesyltransferase inhibitor, or FTI, is the first-ever treatment for Progeria – a remarkable step forward in the pursuit of a cure.
- Since PRF launched its FindTheOther150 campaign, the foundation has increased the number of children identified as having progeria by 60 percent.
- PRF grants have allowed innovative research in progeria to thrive and have helped progeria scientific

¹ Financial Profile
<http://www.progeriaresearch.org/assets/files/pdf/Income%20and%20Expense%20Analysis%202012.pdf> July 2013

- publications increase by more than 1,500 percent since the discovery of the progeria gene.
- PRF launched the Cell & Tissue Bank at Rhode Island Hospital and Rutgers University Cell & DNA Repository in March 2002. Using cells from this bank, PRF Genetics Consortium members Dr. Maria Eriksson and Dr. Francis Collins (now director of the National Institutes of Health) led the progeria gene discovery.
 - PRF developed a diagnostic testing program for progeria in the wake of the gene discovery so that children, their families, and medical caretakers can get a definitive, scientific diagnosis.
 - PRF has sponsored three progeria clinical drug trials and has another planned for 2012.
 - In 2011, media coverage of progeria reached more than 206 million people worldwide through nearly 400 pieces. Also, the Boston Bruins partnered with PRF to record a public service announcement in order to find more children living with progeria in Europe and Canada.
 - For its cell and tissue bank, PRF has collected 161 cell lines and has 110 children as part of the Medical & Research Database.

Leadership

PRF is governed by a **Board of Directors and Board of Advisors**.

- **President & Executive Director:** Audrey Gordon, agordon@progeriaresearch.org
- **Medical Director:** Leslie B. Gordon, PhD, lgordon@progeriaresearch.org
- **Donor and Communications Specialist:** Lynne MacKenzie, lamackenzie@progeriaresearch.org
- **Director of Volunteer Activities:** Michelle Fino, mfino@progeriaresearch.org