Progeria Research Foundation
progeriaresearch.org

MISSION: To discover treatments and the cure for Hutchinson-Gilford Progeria Syndrome and its aging-related disorders, including heart disease.

Timeline Highlights through April 2016: Our History, Our Future…

- 1999: The Beginning…No awareness, no research, no knowledge
- 2003: Progeria Gene Discovery - The doors of science are flung open when *progerin* is found
- 2007: First Clinical Trial – A drug is identified as a potential treatment
- 2012: First Treatment – Trial results show the drug lonafarnib improves the cardiovasculature
- 2014: Longer Life - Time reveals that the treatment lonafarnib increases estimated lifespan
- 2016: New Drug, New Trial – Another drug is added that may help more than lonafarnib alone
- 20--: CURE!

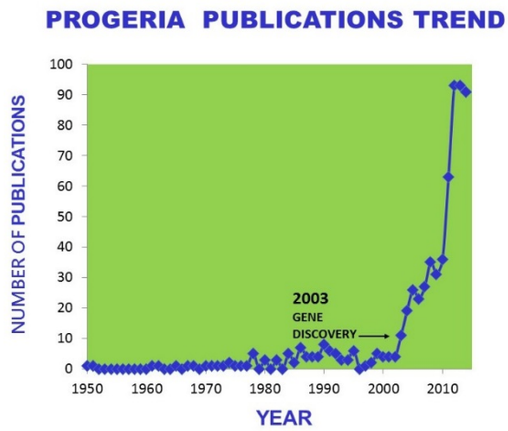
1999…

March 1999: The Progeria Research Foundation (PRF) is incorporated as a Massachusetts non-profit organization. On June 9, 1999, the Board of Directors holds its first meeting.

August 1999: PRF awards its 1st research grant. To date, PRF has awarded 62 grants totaling over $6.7 million. The projects have allowed innovative research in Progeria to thrive, and has helped Progeria scientific publications increase by 2,200% since PRF was founded.

November 1999: PRF launches its web site, a comprehensive resource of information on Progeria for researchers, families of children with Progeria and their physicians, and the general public. Together with social media sites (over 1 million Facebook followers!), millions around the world follow PRF's progress regularly.

www.progeriaresearch.org



…2000

January 2000: PRF launches its International Registry, maintaining centralized information on children and families living with Progeria. This program assures rapid distribution of new information that may benefit the children.

October 17, 2000: PRF is instrumental in securing language in the Children's Health Act 2000 mandating the National Institutes of Health (NIH) to devise a plan to support children with Progeria. As a direct result, NIH has been supporting Progeria research efforts spearheaded by PRF including every PRF scientific workshop, a first-ever natural history study of children with Progeria, and the Progeria Triple Drug Trial.

…2001

November 2001: PRF holds an historic, first-ever workshop in Bethesda, Maryland. PRF has organized 12 successful scientific conferences that have brought together scientists and clinicians from all over the world to share their expertise and cutting edge scientific data, and foster collaboration in the fight against Progeria.

…2002

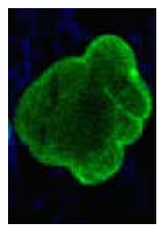
January 2002: The PRF Genetics Consortium is formed. The goal of this group is to find the gene for Progeria, which was accomplished ten months later.

March 2002: PRF launches the Cell & Tissue Bank at Rhode Island Hospital and Rutgers University Cell & DNA Repository, so researchers have the biological tools needed to advance Progeria research. The Bank now holds an impressive 197 lines, including cutting-edge Induced Pluripotent Stem Cells (IPSC's).

The PRF Medical & Research Database is created, to help learn more about the disease and provide treatment recommendations. Four years later, the Database information helped define the primary clinical parameter for Progeria clinical drug trials.

…2003

May 2003: **Progeria Gene Discovered!** Using cells from the PRF Cell & Tissue Bank, PRF Genetics Consortium members Drs. Maria Eriksson and Francis Collins lead the Progeria gene discovery, published in the journal *Nature.*



June 2003: In the wake of the gene discovery, the PRF Diagnostics program is launched. With a definitive genetic test to diagnose children, earlier diagnosis, fewer misdiagnoses and early medical intervention ensure a better quality of life for the children.

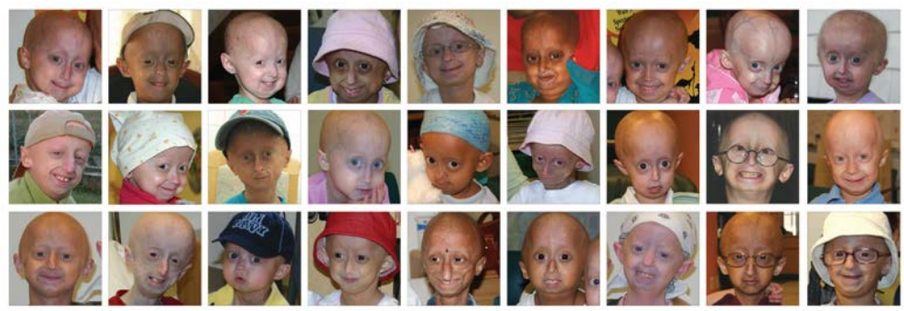
…2004 **August 2004:** PRF awards its 10th research grant, funding the creation of a Progeria mouse. Animal models provide essential preclinical testing for new drugs.

…2005 **November 2005:** A record 96 scientists from 9 countries meet for PRF's fourth scientific workshop in Boston, MA. By 2013, the number of participants and countries nearly doubled. With leading scientists in the fields of heart disease, aging, genetics, and lamins, the depth and breadth of research into Progeria grows stronger with every meeting.

…2006 **June 2006:** PRF launches a public service announcement (PSA) campaign featuring the recognizable voices of long-time PRF supporters Ted Danson and Mary Steenburgen. The PSA received endorsement by the Ad Council and aired in Times Square. PRF has since created many PSAs with other celebrities, including Boston Bruins players and Dave Matthews.

February/July 2006: PRF-funded studies find that farnesyltransferase inhibitors (FTIs) are a potential drug treatment for Progeria, as they prevent some signs of disease in Progeria mice. These and other studies, along with data analyzed from PRF's Medical & Research Database, pave the way for a clinical drug trial using the FTI lonafarnib.

…2007 **April 2007:** Boston Children's Hospital approves the first-ever Progeria Clinical Drug Trial, funded and co-coordinated by PRF. For the first time, we have a possible treatment for children with Progeria. 28 children with Progeria from 16 countries come to Boston to enroll in the 2-year clinical trial. Enrollment took less than 6 months, thanks to PRF's International Registry and the remarkable teamwork with families, their doctors, and trial partners.



…2008… **August 2008:** PRF's 7th chapter opens in Kentucky. Chapters, as well as thousands of other volunteers worldwide, help raise awareness and conduct local fundraising events so that PRF's mission of developing treatments and the cure will be achieved more rapidly to win this race against time.

October 2008: In a stunning display of progress with the FTI drug, and providing further confirmation of how Progeria research may help millions with heart disease, an NIH study finds that FTI's prevent the most devastating effect of Progeria in mice: cardiovascular disease.

…2009… **April 2009:** In a spectacular show of support for children with Progeria, PRF's first annual campaign, called the ONEpossible Campaign, is a success, reaching our $100,000 goal. To date, ONEpossible supporters have raised $1.2 million. They are each ONE who will make a cure POSSIBLE!

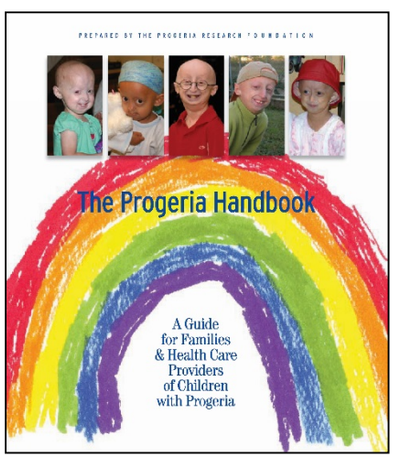
onepossible
The Progeria Research Foundation

August 2009: Clinical Trial #2: Triple Drug Therapy begins. PRF and Boston Children's Hospital begin a three-drug combination trial. Researchers have identified two additional drugs that, when used in combination with the current FTI drug being tested, may provide an even more effective treatment for children with Progeria than FTIs alone. This much larger trial includes 45 children from 24 different countries.

FIND THE OTHER 150
Join PRF's global effort to find all children with Progeria >

October 2009: PRF partners with Spectrum and GLOBALHealthPR to launch "Find the Other 150", a global campaign to find all children with Progeria so they can get the support they need. To date, along with other public awareness efforts, the campaign has helped PRF achieve a 150% increase in the number of known children: 84 children in 5 years. www.findtheother150.org

… 2010 Media coverage reaches new heights with Progeria and PRF's work featured in a variety of top-tier TV, on-line and print media, including The Dr. Oz Show, Barbara Walters' 20/20, CNN, and The Wall Street Journal.



April 2010: PRF publishes the first ever *Progeria Handbook: A Guide for Families & Health Care Providers of Children with Progeria*, providing vital information for families of children with Progeria and their doctors: basic health facts, daily care advice and extensive treatment guidelines to improve the children's quality of life. The book is now available in 5 languages.

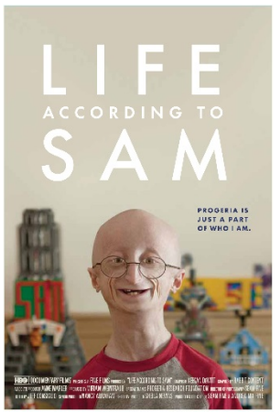
September 2010: A landmark study, led by PRF's medical director in partnership with the National Institutes of Health, demonstrates that the Progeria-causing protein *progerin* exists and increases in everyone as we age. The study suggests a new risk factor for heart disease and confirms that by examining one of the rarest diseases, we gain crucial insight into the heart disease that affects millions, and the aging affecting us all.

… 2011

September 2011: PRF launches Established Investigator, Innovator, and Specialty Awards for basic research. This new grant program structure increases funding towards finding new treatments and cure for Progeria and aging-related diseases, including heart disease.

… 2012

September 24, 2012: First-ever Treatment for Progeria Discovered! PRF makes history with the discovery that lonafarnib is the first-ever treatment for Progeria. Every child in the clinical trial showed improvement in one of more areas, including the vital cardiovascular system. With a definitive finding that drugs can alter the Progeria disease process, the quest for a cure is stronger than ever.



… 2013

January 2013: HBO Film's ***Life According to Sam*** (LATS) debuts at the Sundance Film Festival. Featuring Sam Berns and his parents (PRF co-founders) Drs. Leslie Gordon and Scott Berns, this riveting documentary about love, life and hope for children with Progeria received an Emmy, Peabody and Christopher Awards, and ten festival awards.

Along with LATS, Sam's **October 2013** Katie Couric Show appearance and wildly popular TEDx talk, "How to Live a Happy Life", has vastly raised awareness of Progeria and the importance of PRF's mission in a unique way, resulting in an unprecedented surge in supporters, social media presence and general interest in these remarkable children.

… 2014

May 2014: In a stunning development, a study shows that the drugs tested in the PRF-funded clinical trials increase estimated lifespan of children with Progeria. Lonafarnib appears to be giving them improved cardiovasculature and longer lives. Remarkable!

July 2014: While PRF continues to search for more effective drug candidates**,** the PRF-funded extension and expansion of the clinical trial gives every child with Progeria access to lonafarnib, the drug that could give them longer and healthier lives. 78 children have enrolled to date, including 31 new children, from 31 countries speaking 21 languages.

The drug trial expansion allowed Prachi, age 4 from India, to enroll in December

… 2015

PRF continues its remarkable pace of progress all year, working on a multitude of research-related projects including the lonafarnib expansion trial, planning for a new trial and a new international scientific workshop, and a record-high $1 million investment in research grants.

July 2015: PRF and Merck make the farnesyltransferase inhibitor lonafarnib available to the research community through the PRF Cell & Tissue Bank. This supply will foster preclinical studies to further investigate the effects of lonafarnib on Progeria. As new compounds are identified with the potential to ameliorate Progeria, these compounds can be tested in combination with lonafarnib in vitro and in animal models.

Carly (left) and Zoey enroll in the PRF-funded, 2-drug trial at Boston Children's Hospital

… 2016

April 2016: Children begin to enroll in a new, 2-drug trial that includes the treatment lonafarnib plus everolimus, a form of rapamycin, with the hope that the two drugs together will be even more effective than lonafarnib alone.
New Drug, and New Hope for Children with Progeria.

We've come so far in such a short time.
With your continued support,
Together, we *WILL* find the cure!