Progeria
Progress toward the Cure

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

Features
- Growth failure
- Loss of body fat and hair
- Premature atherosclerosis
- Joint stiffness
- Normal intelligence
- Heart failure & strokes
- Premature aging

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 estimates that there are 400 children and young adults with Progeria worldwide.
PRF knows of fewer than half of those living with Progeria in 50 countries.

Together, we WILL find the cure!

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In 2020, after 13 years of PRF-funded clinical trials, we celebrated the first-ever FDA approval of lonafarnib. With long-term use, this treatment is shown to extend lifespan by an average of 4.5 years, or 30%. The drug is also now approved in Europe and Japan.

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 lead to even better treatments and a future cure.

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