

Hutchinson-Gilford Progeria Syndrome (HGPS)

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Hutchinson-Gilford Progeria Syndrome (HGPS) Latest News

U.S.-based Sentyln Therapeutics (part of Zydus Lifesciences) recently signed a deal with South Korea's PRG S&T to develop Progerinin, a pill aimed at treating Hutchinson-Gilford progeria syndrome (HGPS).

About Hutchinson-Gilford Progeria Syndrome (HGPS)

- Progeria, also known as HGPS, is an **extremely rare genetic disease** that causes **rapid aging in children**.
- Progeria is extremely rare. It occurs in **1 in every 4 million live births** worldwide.
- **What Causes Progeria?**
 - A **genetic mutation in the LMNA gene** causes progeria. The LMNA gene is **responsible for making** a protein called **lamin A**.
 - **Lamin A** is an important part of the structural scaffolding that **holds the nucleus of each cell in your body together**.

- A **tiny mutation** in the LMNA gene **causes** it to create an **irregular form** of the **lamin A protein** called **progerin**.
- **Progerin** takes the place of the lamin A and **makes the nuclei** of your cells **unstable, slowly damaging** them.
- This leads to the **early death of every cell** in your body, which causes the process of **premature aging**.
- **Almost all cases** of progeria occur as a **new, spontaneous mutation** in the LMNA gene. This means there's **no biological family history** of the disease.
- **Newborns** with the disorder appear to be healthy at birth but usually start to show **signs of premature aging** during their **first one to two years** of life.
- Their **growth rate slows** and they **don't gain weight** as expected.
- This condition **does not affect intellectual development** or the **development of motor skills** such as sitting, standing, and walking.
- However, their rapid aging **causes distinct physical characteristics**, including:
 - Hair loss (**baldness**).
 - **Prominent eyes**.
 - Aged, **wrinkled skin**.
 - A thin, beaked nose.
 - Disproportionately small face compared to head size.
 - Loss of fat under the skin.
- Progeria is **always fatal**. The **average age of death is 14.5 years**, although some adults with progeria will live into their early 20s.
- Death most often **occurs as a result** of complications of **severe atherosclerosis**.
 - **Atherosclerosis** occurs when **plaque builds up within** the **walls of your arteries**. This makes them less elastic and, therefore, stiffer.
 - This condition greatly increases the chances of having a **heart attack or stroke at a young age**.

Source: NB

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