# 400 children worldwide are born each year with ‘fast-aging disease’ – Hutchinson-Gilford progeria syndrome. Now CRISPR offers hope

[R]esults from a new study have inspired hope for treating children born with progeria, a rare, fatal, genetic disease that causes symptoms much like early aging. In mice with a progeria-causing mutation, a cousin of the celebrated genome editor known as CRISPR corrected the DNA mistake, preventing the heart damage typical of the disease, a research team reports [January 6] in Nature. Treated mice lived about 500 days, more than twice as long as untreated animals.
“The outcome is incredible,” says gene-therapy researcher Guangping Gao of the University of Massachusetts, who was not involved with the study.

Although the developers of the progeria therapy aim to improve it, they are also taking steps toward testing the current version in affected children, and some other scientists endorse a rush. The mouse results are “beyond anyone’s wildest expectations,” says Fyodor Urnov, a gene-editing researcher at the University of California, Berkeley. “The new data are an imperative to treat a child with progeria … and do so in the next 3 years.”



About 400 people in the world are estimated to have Hutchinson-Gilford progeria syndrome… Toddlers soon become bald and have stunted growth, body fat loss, stiff joints, wrinkled skin, osteoporosis, and atherosclerosis. People with progeria die on average around age 14 from a heart attack or stroke.

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