

Gene therapy gives new hope to curing 'bubble boy' disease

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David Vetter, the famous "bubble boy" of the 1970s, had to live in a plastic pod, eating sterilized food and wearing sterilized clothes, to avoid the everyday germs that can be fatal to those with X-linked severe combined immunodeficiency (SCID), a rare immune disorder that affects an estimated 1 in 100,000 people, primarily boys.

But if he were born today, Vetter would likely have been the perfect candidate for a new [gene therapy](#) technique that seems to get the immune systems of SCID patients back on track. And while previous genetic treatments had been performed on infants, this new approach works in teenagers and young adults, according to a small but promising [study](#).

The [earliest gene therapy for SCID](#) kept patients alive, but it also had the nasty side effect of sometimes causing leukemia. Even when researchers tweaked the virus they were using as a vehicle for the genetic change, making it safer, the therapy only restored the function of certain kinds of immune cells. That meant patients were still sick, and needed regular injections of pathogen-fighting immunoglobulins to make sure that their bodies could recognize dangerous microbes.

To overcome this problem, researchers turned to a newer viral vehicle, a lab-grown version of HIV with the dangerous innards torn out. They used this to insert healthy genes into stem cells extracted from each patient's bone marrow.

Read full, original post: [New therapy offers gene fix for 'bubble boy' disease](#)