

Stem cell cure for 'bubble boy' disease breakthrough in treating genetic disorders

Alysia Padilla-Vaccaro and Christian Vaccaro owe their daughter's life to stem cells. Evangelina, now two, is alive today because she saved herself with her own bone marrow cells.

Evangelina, a twin, was born with a severe immune disorder caused by a genetic aberration that makes her vulnerable to any and all bacteria and viruses; even a simple cold could be fatal. But doctors at University of California Los Angeles (UCLA) Broad Stem Cell Research Center gave her a new treatment, using her own stem cells, that has essentially cured her disease. She's one of 18 children who have been treated with the cutting-edge therapy, and the study's leader, Dr. Donald Kohn, says that the strategy could also be used to treat other gene-based disorders such as sickle cell anemia.

Known to doctors as adenosine deaminase (ADA)-deficient severe combined immunodeficiency (SCID), it's better known as "bubble boy" disease, since children born with the genetic disorder have immune systems so weak that they need to stay in relatively clean and germ-free environments. Until Evangelina and her sister Annabella were 11 months old, "We were gowned and masked and did not go outside," says their mother Alysia Padilla-Vaccaro. "Our children did not physically see our mouths until then because we were masked all the time. We couldn't take them outside to take a breath of fresh air, because there is fungus in the air, and that could kill her."

Read full, original article: ['Bubble Boy' Disease Cured With Stem Cells](#)