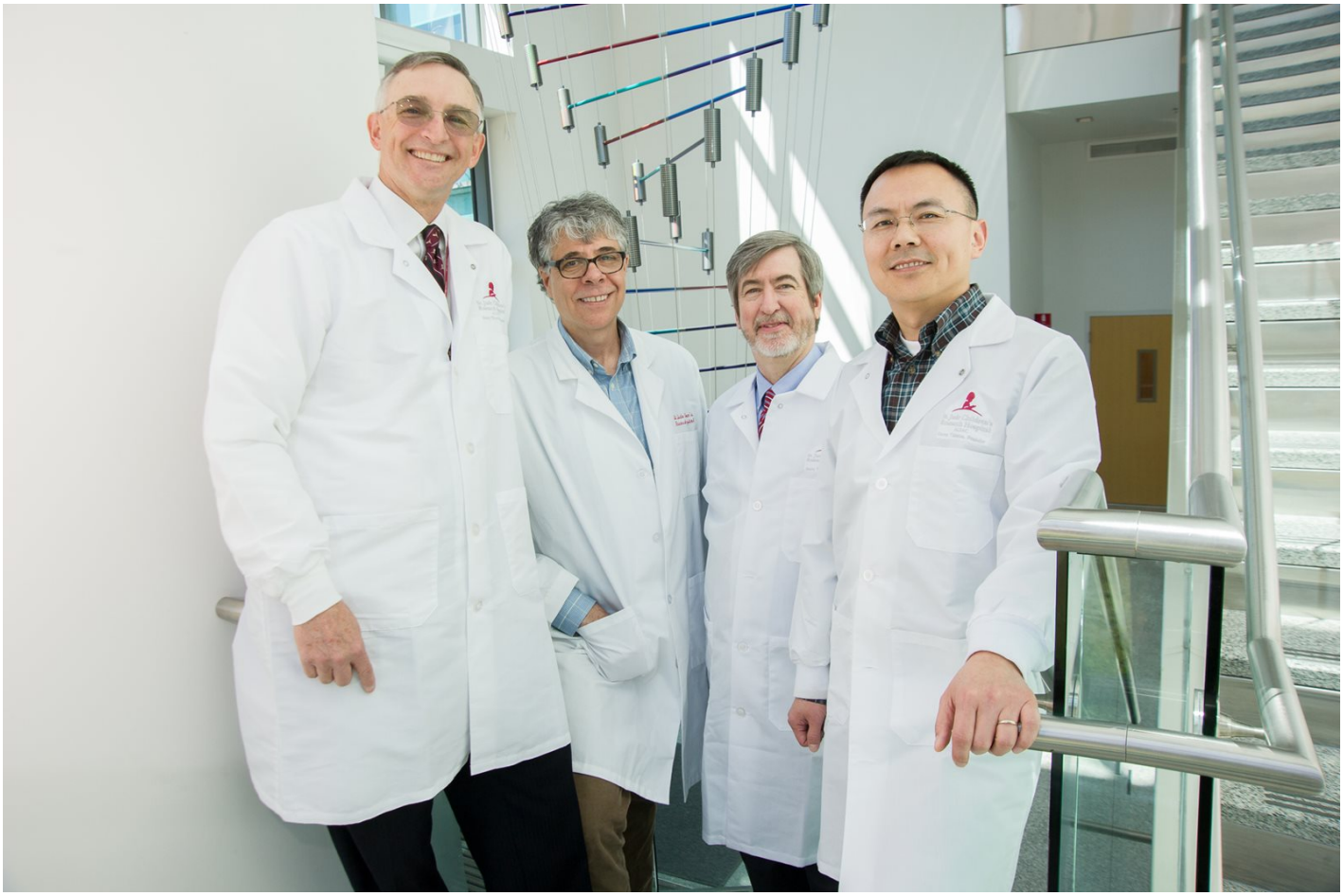
Groundbreaking Gene Therapy For Rare Genetic Disorder

St. Jude Children's Research Hospital



You may have heard of the "bubble boy" disease, but research licensed from St. Jude Children's Research Hospital may have made this disease a thing of the past. A lentiviral (LV) vector made at St. Jude is being investigated in multicenter clinical trials in conjunction with reduced-exposure busulfan conditioning. The therapy uses the bone marrow of the patients and a lentivirus that "installs" a copy of the gene to activate the immune system in the cells. The therapy was developed by a team led by Brian Sorrentino, M.D., a faculty member who died in late 2018.

X-linked severe combined immunodeficiency (XSCID) is a rare genetic disorder that occurs in 1 to 2 births per 100,000. The children are born lacking the ability to produce T cells or natural killer (NK) cells; and although they have a normal number of B cells, they are not functional. As a result, patients often suffer from potentially deadly bacterial, viral, or fungal infections very early in life.

Previously, patients on gene therapy trials gained T cells but still required lifelong gamma globulin therapy. More than two years after the first clinical trial, initial patients are producing a greater percentage of immune cells.

Both trials continue to add patients, with those under the age of 2 being treated at St. Jude, UCSF Benioff Children's Hospital San Francisco, and Seattle Children's Hospital; and older patients being treated at the NIH.

In 2018, the successful trials generated commercial interest which ultimately resulted in an exclusive worldwide license agreement between St. Jude and Mustang Bio to further develop and commercialize this therapy. St. Jude's Office of Technology Licensing helped market the invention and vetted interested companies for a potential commercial license. These efforts ended in successful licensing to Mustang Bio.

The St. Jude XSCID clinical trials provide insights for treating other disorders, which include Wiskott-Aldrich Syndrome, a disorder that causes infections and reduces the ability to form blood clots, and sickle cell disease, which affects about 100,000 Americans. St. Jude cares for around 1,000 pediatric sickle cell patients, and the gene therapy platform could potentially be curative for these patients as well as for many other devastating immune disorders in the future.

The treatment was honored with a Smithsonian magazine American Ingenuity Award for Life Sciences in 2019.

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