



Stanford
M E D I C I N E

Stem Cell Transplant Research



This study is being
conducted at:

**Lucile Packard
Children's Hospital**



Stanford
Children's Health

Lucile Packard
Children's Hospital
Stanford

**UCSF Benioff
Children's Hospital**



UCSF Benioff Children's Hospital
San Francisco

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Study of a Monoclonal Antibody to Allow Blood Forming Stem Cell Engraftment in Children with SCID without Chemotherapy

Although bone marrow transplant has saved the lives of children with severe combined immune deficiency (SCID), many of the patients still have abnormal immune systems. Their immune systems may have persistent or acquired T cell abnormalities and/or B cells which are unable to make protective antibodies. As a result, they may still require replacement immunoglobulin or be susceptible to recurrent viral infections. The problem of continued immune abnormalities after transplantation is especially true of children, who received their transplants without prior chemotherapy. In those patients, there was no engraftment of the normal donor blood forming stem cells, which is required for the long-term production of normal T and B cells and normal immunity. Doctors and scientists from the Departments of Medicine and Pediatrics at Stanford School of Medicine have developed a new transplantation approach designed to allow donor blood forming stem cells to engraft without the need for chemotherapy prior to transplantation. Based upon successful studies in animals, patients will receive a single dose of an antibody that binds to a protein (c-Kit, CD117), which is found on blood forming stem cells. The antibody depletes the marrow of the abnormal blood forming stem cells and makes space for normal blood forming stem cells to engraft. Once the antibody is no longer present in the patients, they will receive a second transplant of normal blood forming stem cells from their original donor. The patients will then be followed to determine if the new donor blood forming stem cells engraft and if their immune systems improve.