St. Jude Children's Research Hospital®
Finding cures. Saving children.
ALSAC • DANNY THOMAS, FOUNDER

About X-Linked Severe Combined Immunodeficiency Disease (SCID-X1)

X-linked severe combined immunodeficiency disease, or SCID-X1, is an inherited blood cell disease. Almost all children who have it are boys. It is caused by changes in a gene that help develop the body's infection-fighting system, called the immune system. Genes contain information that tells the body how to develop and work. When children have SCID-X1, their immune system do not work correctly. That makes it hard for them to fight off life-threatening infections.

ABOUT THIS RESEARCH STUDY

In this research study, researchers will give children with SCID-X1 a treatment called "lentiviral gene transfer," also called "gene therapy." This method replaces the damaged gene with a normal one. The research team puts a copy of the normal gene into your child's bone marrow stem cells. Stem cells are young, early cells found in the bone marrow. Bone marrow is the soft, sponge-like material inside bones. Stem cells can grow up to become any type of blood cell.

WHY IS THIS STUDY BEING DONE?

The purpose of this research study is to determine if gene therapy can treat SCID-X1. We want to learn if the procedure is safe, if it can be done the way researchers chose, and if it will give your child a normal immune system. We hope this will be a new way to treat children with SCID-X1 who do not have brothers or sisters to donate stem cells for transplant.

ELIGIBILITY

If all the items below are true, your child might qualify to be in this study:

- A diagnosis of severe combined immunodeficiency disease, X-linked (SCID-X1)
- Age: newborn to 2 years old
- No prior stem cell transplant with cells from a brother or sister
- Does not have HIV (the virus that causes AIDS)

LEARN MORE

Read our "Frequently Asked Questions" or visit *stjude.org/LVXSCID-ND* for more information about this research study and gene therapy. To enroll your child in the study, please contact:

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LVXSCID-ND GENE TRANSFER CLINICAL TRIAL **FOR X-LINKED SCID**

Frequently Asked Questions

Q. Has lentiviral gene transfer been done before?

A. Yes. Recently, St. Jude Children's Research Hospital took part in a research study with the National Institute of Allergy and Infectious Diseases. Five teens and young adults with SCID-X1 did better after treatment with lentiviral gene transfer. The results of this study were published in the medical journal *Science Translational Medicine*. St. Jude developed the process used in this study and made the gene transfer material on the St. Jude campus under strict guidelines. We now want to test this same procedure in infants. Our study team includes researchers experienced in gene therapy and in caring for children with blood disorders. We hope this method will give us a way to treat children who do not have matched donors for stem cell transplants.

Q. What will happen in the study?

A. The research study has the following parts:

1. Collecting bone marrow stem cells from your child in the operating room
2. Giving your child chemotherapy to make room for the healthy bone marrow cells
3. Putting the healthy gene into the collected stem cells, and then putting the cells into your child's vein
4. Treating your child in the hospital until the immune system recovers or the doctors believe your child is well enough to go home
5. Checking on your child after he goes home from the hospital.

Q. Where will the study be done?

A. The research study will take place at St. Jude in Memphis, Tennessee.

Q. My child qualifies to be in the study. What do we do next?

A. Ask your child's doctor or other health care provider to contact St. Jude to schedule an evaluation to see if your child is eligible for the study. If your child is a good candidate for this study, the study team will get you and your child ready to be in it. The team will also answer questions you may have. They can help you arrange transportation to Memphis if you need help.

*www.stjude.org/**LVXSCID-ND***