

Preliminary Study Shows that the Lung Cancer Drug, Crizotinib, Is Effective for Three Childhood Cancers

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In an early study with the targeted therapy drug crizotinib (Xalkori), researchers found that it stopped the growth of neuroblastoma, anaplastic large cell lymphoma (ALCL), and inflammatory myofibroblastic tumors (IMT), and in some instances, removed all signs of the cancer.

Neuroblastoma is a tumor that develops in the nerve cells. ALCL commonly begins in T cells, and rarely B cells, which are types of white blood cells that help the body fight infection. IMT is a rare tumor that often begins in the lungs, soft tissues, and other organs. Targeted therapy is a treatment that targets a cancer's specific genes, proteins, or the tissue environment that contributes to cancer growth and survival. Specifically, crizotinib targets genetic mutations (changes) in the *ALK* gene, which is found in each of these cancers. The drug was also recently approved by the U.S. Food and Drug Administration to treat adult lung cancers that have mutations to the *ALK* gene.

The 70 children who participated in this study had neuroblastoma, ALCL, or IMT that had worsened while receiving the standard treatments. They received one of six different doses of crizotinib twice a day and continued taking the drug as long as they had no side effects. When possible, the cancers were tested for a change to the *ALK* gene to find out if the crizotinib worked better to treat cancer with a changed *ALK* gene.

Researchers found that most (7 out of 8 patients or 88%) of the patients with ALCL had no evidence of the cancer for as long as 18 months, and most of the patients with IMT had the tumor shrink or disappear for up to two years. For neuroblastoma, researchers found that three out of the 27 patients who participated in the study had the disease disappear, and eight had the disease stop growing. For the eight patients who had neuroblastoma with mutations to the *ALK* gene, two had the disease disappear. The findings for children with neuroblastoma are particularly important because the benefit lasted from nine months to more than two years, and generally neuroblastoma that has already been treated with all the standard therapies usually worsens within one to two months.

What this means for patients

It's remarkable that this targeted oral medication provided such a benefit for these children with highly aggressive cancers, most of whom had already received every available therapy, said [Yael Mosse, MD \[1\]](#), Assistant Professor of Pediatrics at the Children's Hospital of Philadelphia and the University of Pennsylvania and recipient of a Conquer Cancer Foundation of ASCO Young Investigator Award in 2003 and a Career Development Award in 2004. "Now that we know more about factors that drive cancer growth in children, we can target those changes and give treatment in a much smarter, and potentially safer, way." Talk with your child's doctor about all treatments available for these cancers, including clinical trials.

Questions to ask the doctor

- What type of cancer does my child have?
- What is the prognosis (chance of recovery)?
- Will testing of the cancer's genes be needed to help determine the best treatment options?
- What are my child's treatment options?
- What are the risks and benefits of this treatment?
- What clinical trials are open to my child?

For More Information

[Cancer in Children \[2\]](#)

[Guide to Neuroblastoma \[3\]](#)

[Guide to Childhood Non-Hodgkin Lymphoma \[4\]](#)

[Understanding Targeted Treatments \[5\]](#)

Links:

[1] <http://www.conquercancerfoundation.org/press-release-pediatric-cancers>

- [2] <http://www.cancer.net/patient/Coping/Age-Specific+Information/Cancer+in+Children>
[3] <http://www.cancer.net/patient/Cancer+Types/Neuroblastoma+-+Childhood>
[4] <http://www.cancer.net/patient/Cancer+Types/Lymphoma+-+Non-Hodgkin+-+Childhood>
[5] <http://www.cancer.net/patient/All+About+Cancer/Cancer.Net+Feature+Articles/Treatments%2C+Tests%2C+and+Procedures/Understanding+Targeted+Treatments>