Promising cancer treatment targets rare genetic flaw

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An experimental cancer medicine called larotrectinib has shown promise treating a diverse range of cancers in people young and old, researchers said at a major cancer conference in the United States.

The treatment targets a genetic abnormality which is often found in rare cancers—including salivary gland cancer, juvenile breast cancer, and a soft tissue cancer known as infantile fibrosarcoma—which are particularly difficult to treat.

This abnormality also occurs in about 0.5 percent to one percent of many common cancers.

In the study released at the American Society of Clinical Oncology conference, 76 percent of cancer patients—both children and adults with 17 different kinds of cancer—responded well to the medicine.

A total of 79 percent were alive after one year. The study is ongoing.

Twelve percent went into complete remission from their cancer.

The clinical trial included 55 patients—43 adults and 12 children. All had advanced cancers in various organs, including the colon, pancreas and lung, as well as melanoma.

"These findings embody the original promise of precision oncology: treating a patient based on the type of mutation, regardless of where the cancer originated," said lead study author David Hyman, chief of early drug development at Memorial Sloan Kettering Cancer Center in New York.

"We believe that the dramatic response of tumors with TRK fusions to larotrectinib supports widespread genetic testing in patients with advanced cancer to see if they have this abnormality."

Made by Loxo Oncology Inc., larotrectinib is a selective inhibitor of tropomyosin receptor kinase (TRK) fusion proteins.

TRK proteins are a product of a genetic abnormality when a TRK gene in a cancer cell fuses with one of many other genes, researchers said.

The US Food and Drug Administration has not yet approved the treatment for widespread use.

The treatment was well tolerated by patients, and the most common side effects were fatigue and mild dizziness.

"If approved, larotrectinib could become the first therapy of any kind to be developed and approved simultaneously in adults and children, and the first targeted therapy to be indicated for a molecular definition of cancer that spans all traditionally-defined types of tumors." said Hyman.