Precision cancer therapy effective in both children and adults

February 21 2018

Three quarters of patients, both adults and children, with a variety of advanced cancers occurring in different sites of the body responded to larotrectinib, a novel therapy that targets a specific genetic mutation. Results of this multisite phase 1/2 trial have been published in the *New England Journal of Medicine* on February 22, 2018. Unlike most cancer therapies, this oral treatment is based on the genetic traits of the tumor and not the organ where the cancer originated.

An acquired genetic defect, TRK fusions accelerate cancer cell growth. Larotrectinib is highly selective for inhibiting this process. Fifty-five patients, ranging from 4 months to 76 years of age, with 17 unique tumor types, were treated with larotrectinib. Three quarters of patients enrolled responded to therapy and 86% of responding patients remain on study or have undergone curative surgery. No patients discontinued treatment due to drug-related side effects.

Several pediatric patients that enrolled in the study had infantile fibrosarcoma, a type of cancer that harbors a TRK fusion and is difficult to treat since it responds poorly to chemotherapy. Radiation therapy is also not a good option since it has devastating long-term effects for young patients.

"This is truly a magic bullet for our patients with TRK-positive cancer," said Leo Mascarenhas M.D, M.S, deputy director of the Children's Center for Cancer and Blood Diseases and director of the Names Family Foundation Early Phase Clinical Trials Program in oncology at

Children's Hospital Los Angeles, who helped design the pediatric part of the study. "In some cases, this cancer can be treated surgically - often requiring amputation or another disfiguring surgical procedure. After treating our patient with infantile fibrosarcoma with larotrectinib, the cancer shrunk sufficiently and we were able to surgically remove the tumor while preserving the patient's leg."

This study is part of a noteworthy drug development program. Typically, testing of new therapies in a pediatric population is done after the drug is licensed for adults, if at all. However, larotrectinib was simultaneously studied in children and adults. A special liquid formulation was developed for administering appropriate doses to very young patients. This early pediatric focus helped to accelerate clinical development by aiding in the rapid accrual of appropriate patients.

The FDA granted larotrectinib breakthrough therapy designation that resulted in an expedited review. Drugs may qualify as breakthrough therapies when preliminary clinical data indicate that the new treatment offers substantial advantages over existing options for serious or life-threatening diseases.

Provided by Children's Hospital Los Angeles

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