FDA Approves Vitrakvi for All Cancers With Specific Gene Mutation

TRK inhibitor larotrectinib led to durable response in adults and children with 17 cancer types.

November 26, 2018 By Liz Highleyman

On November 26, the Food and Drug Administration (FDA) granted accelerated approved of Vitrakvi (larotrectinib), the first drug developed to treat cancer with a specific genetic characteristic regardless of its location in the body.

Vitrakvi was approved for adults and children with metastatic solid tumors (not blood cancers) that cannot be surgically removed without serious harm and that have no satisfactory existing therapies. It is available as a capsule and a liquid oral solution for children.

Cancer treatment has traditionally been developed, tested and prescribed for tumors in specific parts of the body, such as breast or lung cancer. But so-called site-agnostic or tumor-agnostic therapies, which attack cancer with specific characteristics no matter where it occurs, could reduce the need for separate clinical trials and would be beneficial for people with uncommon cancers.

Vitrakvi (formerly known as LOXO-101), developed by Loxo Oncology and Bayer, is a selective tropomyosin receptor kinase (TRK) inhibitor. TRK proteins are encoded by three neurotrophic receptor tyrosine kinase (NTRK) genes. When one of these genes in a cancer cell fuses with another gene, it acts as an ignition switch to accelerate tumor growth. Interfering with the resulting TRK proteins can stop disease progression. These fusion mutations are rare overall—occurring in only around 1 percent of all cancers—but they're more common in certain cancers, including some rare types.

At the 2017 American Society of Clinical Oncology annual meeting, researchers reported combined results from the NAVIGATE and SCOUT trials and a Phase I study. According to a full report in the New England Journal of Medicine, among 55 adults and children with 17 cancer types, the overall response rate—meaning complete or partial tumor shrinkage—was 75 percent. After one year, 71 percent of responses were ongoing and 55 percent of study participants were still alive without disease progression.

At the recent European Society for Medical Oncology Congress, researchers presented follow-up data showing an overall response rate of 80 percent, including 18 percent with complete

responses. Several people who had partial responses in the initial report became compete responders. The latest data showed that 88 percent of responses were ongoing after six months on treatment and 75 percent were ongoing after 12 months.

The median duration of response, median progression-free survival and median overall survival cannot yet be determined because a majority of study participants are still alive and doing well.

Vitrakvi is generally safe and well tolerated.Few study participants stopped taking the drug or reduced their dose due to adverse events. The most common side effects include fatigue, nausea, vomiting, dizziness, constipation, diarrhea and elevated liver enzymes. More serious side effects may include neurotoxicity and liver problems. Vitrakvi may cause birth defects if used during pregnancy.

Vitrakvi and other site-agnostic therapies represent a new cncer treatment paradigm, underscoring the importance of genetic testing to help guide the selection of appropriate targeted therapies.

Last year, the FDA approved a new indication for the immune checkpoint inhibitor Keytruda (pembrolizumab) for tumors at any site with genetic mutations known as high microsatellite instability or mismatch repair deficiency. Genentech's entrectinib (RXDX-101) targets both NTRK gene fusions and ROS1 fusions, which play a role in lung cancer. Bluprint's BLU-667 targets RET gene mutations and fusions. Loxo is also working on a drug that target RET mutations, LOXO-292, as well as a next-generation TRK inhibitor.

"Today's approval marks another step in an important shift toward treating cancers based on their tumor genetics rather than their site of origin in the body," said FDA commissioner Scott Gottlieb, MD. "Its approval reflects advances in the use of biomarkers to guide drug development and the more targeted delivery of medicine. We now have the ability to make sure that the right patients get the right treatment at the right time. This type of drug development program, which enrolled patients with different tumors but a common gene mutation, wouldn't have been possible a decade ago because we knew a lot less about such cancer mutations…We're committed to continuing to advance a more modern framework of clinical trial designs that support more targeted innovations across disease types based on our growing understanding of the underlying biology of diseases like cancer."

Click here for full Vitrakvi prescribing information.

Click here for an FDA press release about Vitrakvi approval.

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