Drug approved to help young patients battle a rare cancer

January 2 2023, by Cara Murez HealthDay Reporter

Children and adults with a rare type of soft tissue cancer will now have a

new treatment option that could have a big impact.

The U.S. Food and Drug Administration has approved the immunotherapy drug atezolizumab (Tecentriq) for use in patients with advanced alveolar soft part sarcoma (ASPS) that has spread to other parts of the body or cannot be removed by surgery.

"This approval will make a huge impact in terms of a rare disease that has been particularly challenging to treat," said Dr. Alice Chen, of the Developmental Therapeutics Clinic in the U.S. National Cancer Institute's (NCI) Division of Cancer Treatment and Diagnosis (DCTD).

This cancer begins in the soft tissue that connects and surrounds the organs and other tissues. It spreads slowly, but is typically deadly once it spreads. Chemotherapy doesn't work against it and new targeted treatments, including drugs called tyrosine kinase inhibitors, do not have lasting effectiveness.

About 80 people in the United States receive an ASPS diagnosis each year. About 50% of patients with metastatic disease are still alive after five years. The cancer mostly affects adolescents and young adults.

The approval was granted following the results of a non-randomized phase 2 trial led by the NCI, part of the U.S. National Institutes of Health (NIH). The drug is approved for people aged 2 and up.

About 40% of the patients in the trial were treated at the NIH Clinical Center in Maryland, said Dr. James Doroshow, director of the DCTD.

"Our ability to bring patients in from all over the world was a key factor in the ability to do the study," Doroshow said in an NCI news release.

This was the first study conducted in the NCI-funded Experimental

Therapeutics Clinical Trials Network that has resulted in a drug approval .

"This is a major milestone for investigators in the Experimental Therapeutics Clinical Trials Network, as well as for the ASPS patient community, and for research on rare cancers," said study leader Dr. Elad Sharon.

This approval also marks the first time the drug has been approved for children. The Pediatric Oncology Branch in NCI's Center for Cancer Research helped enroll children in the trial, Chen said.

"This study is an important example of collaboration between pediatric and medical oncology, allowing children with very rare cancers access to effective new therapies," said Dr. John Glod, of the Pediatric Oncology Branch. "The entire study team is grateful to the patients who participated in the study and made this work possible."

Atezolizumab works by helping the immune system respond more strongly to cancer. An anti-PD-L1 immune checkpoint inhibitor, the drug is approved for patients with several cancer types, including liver cancer, melanoma and lung cancer.

The FDA granted breakthrough therapy designation for the drug in 2020, to treat patients with metastatic ASPS.

This meant the drug had met the FDA's criteria for expedited development and review. Last year, the agency granted orphan drug designation to atezolizumab for soft tissue sarcoma in general, a status that provides incentives for companies to develop a drug for rare diseases.

The NCI's phase 2 trial enrolled 49 ethnically diverse patients aged 2 and

older with ASPS that had spread. The patients were given an infusion of atezolizumab every 21 days.

About one-third of the patients responded to the treatment with some degree of tumor shrinkage. Most of the other patients experienced stable disease.

Patients who had two years of treatment were given an opportunity to take a treatment break for up to two years with close monitoring, according to the study. None of those patients on the break had disease progression.

About 41% of patients who received the drug had serious side effects that included anemia, diarrhea, rash, dizziness, hyperglycemia and pain in the extremities, but no patients left the study because of side effects.

"This approval represents a victory for rare diseases, which are understudied in clinical trials," Chen said. "For this approval to go through in a rare disease, and to be able to make an impact on these young people's lives, is very significant."

Research teams are now conducting additional trials with atezolizumab in patients with ASPS. This includes giving the drug in combination with other therapies.

Genentech, a member of the Roche Group and the manufacturer of atezolizumab, provided the drug to the NCI through a cooperative research and development agreement.

More information: Dana-Farber Cancer Institute in Boston has more on alveolar soft part sarcoma in children.