FDA Approves New Targeted Therapy for Lung Cancer

Exkivity is approved for adults with advanced non-small-cell lung cancer with specific EGFR mutations.

September 16, 2021 By Liz Highleyman

On September 15, the Food and Drug Administration (FDA) granted accelerated approval of Exkivity (mobocertinib), a new targeted therapy for people with locally advanced or metastatic non-small-cell lung cancer (NSCLC) with EGFR exon 20 insertion mutations that has progressed despite platinum-based chemotherapy.

Exkivity (formerly known as TAK-788) is a tyrosine kinase inhibitor (TKI) that blocks epidermal growth factor receptor (EGFR), which plays a role in cancer cell growth and the development of blood vessels that feed tumors. The drug specifically binds to and inhibits EGFR with exon 20 insertion mutations, which occur in approximately 1% to 2% of people with NSCLC. Exkivity is the first oral medication designed to target these mutations, according to Takeda Pharmaceutical Company.

"EGFR exon 20 insertion-positive NSCLC is an underserved cancer that we have been unable to target effectively with traditional EGFR TKIs," Pasi Jänne, MD, PhD, of Dana-Farber Cancer Institute, said in a Takeda press release. The approval "marks another important step forward that provides physicians and their patients with a new targeted oral therapy specifically designed for this patient population that has shown clinically meaningful and sustained responses."

The accelerated approval is supported by the results of an international nonrandomized, open-label Phase I/II clinical trial (NCT02716116) that included 114 patients with locally advanced or metastatic NSCLC (mostly adenocarcinoma) with EGFR exon 20 insertion mutations whose disease had progressed during or after platinum-based chemotherapy. Two thirds were women, 60% were Asian, the median age was 60 years and 71% had never smoked. Studies have found that people with no history of smoking develop distinct types of lung cancer.

More than a quarter of participants had used three or more prior therapies. About 40% had tried immunotherapy, and 25% had previously tried an older EGFR kinase inhibitor. A third had brain metastasis, or cancer that had spread to the brain.

All study participants received Exkivity pills once daily until they experienced disease progression

or intolerable side effects.

As reported at this year's American Society of Clinical Oncology annual meeting, the overall response rate, meaning complete or partial tumor shrinkage, was 28%, and the median duration of response was 17.5 months. The median progression-free survival time was 7.3 months, and the median overall survival time was 24 months.

The most common adverse reactions in people taking Exkivity are diarrhea, nausea, vomiting, mouth sores (stomatitis), decreased appetite, fatigue, muscle or joint pain, rash, dry skin and inflammation around the nails (paronychia). The drug can cause depletion of white blood cells, which raises the risk of infections, and decreased hemoglobin. More serious side effects may include heart rhythm abnormalities, cardiac toxicity, lung inflammation and severe diarrhea.

Drugs that receive accelerated approval based on overall response rate are expected to undergo further testing to confirm whether they provide clinical benefits, such as improved survival, and the FDA can rescind the approval if they fail to measure up.

The approval highlights the importance of tumor genomic testing to see whether a treatment is likely to work. The FDA also approved a companion diagnostic next-generation sequencing test to determine whether patients are eligible to use Exkivity.

"Patients with EGFR exon 20 insertion-positive NSCLC have historically faced a unique set of challenges living with a very rare lung cancer that is not only underdiagnosed but also lacking targeted treatment options that can improve response rates," said Marcia Horn, executive director of the International Cancer Advocacy Network's Exon 20 Group. "As a patient advocate working with EGFR exon 20 insertion-positive NSCLC patients and their families every day for nearly five years, I am thrilled to witness continued progress in the fight against this devastating disease and am grateful for the patients, families, health care professionals and scientists across the globe who contributed to the approval of this promising targeted therapy."

Click here for full prescribing information for Exkivity.

Click here to learn more about lung cancer.

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