FDA Approves First FGFR Inhibitor for People with Advanced Bladder Cancer

Balversa led to tumor regression in about a third of patients with susceptible genetic mutations.

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The Food and Drug Administration (FDA) has approved a new targeted therapy, Balversa (erdafitinib), for previously treated locally advanced or metastatic bladder cancer that carries genetic mutations known as FGFR2 or FGFR3.

"We're in an era of more personalized or precision medicine, and the ability to target cancer treatment to a patient's specific genetic mutation or biomarker is becoming the standard, with advances being made in new disease types," Richard Pazdur, MD, director of the FDA's Oncology Center of Excellence, said in a press release. "Today's approval represents the first personalized treatment targeting susceptible FGFR genetic alterations for patients with metastatic bladder cancer."

Urothelial carcinoma, also known as transitional cell carcinoma, affects the cells lining the bladder, urethra and other parts of the urinary tract. About 80,500 people will develop bladder cancer and around 17,700 will die from it this year, according to the American Cancer Society, making it the most common type of bladder cancer.

FGFR, or fibroblast growth factor receptor, plays a role in cell division and maturation. About one in five people with recurrent or refractory bladder cancer has genetic mutations in the genes that encode FGFR2 or FGFR3, which lead to increased cell growth. Balversa is the first kinase inhibitor that works against cancer with these mutations.

Balversa, from Janssen, was approved for adults with locally advanced or metastatic (spread beyond the bladder) cancer that has progressed despite platinum-based chemotherapy (drugs such as cisplatin or oxaliplatin). It is taken as a once-daily pill. The FDA also approved a companion diagnostic test to identify people with FGFR2 or FGFR3 mutations.

Approval of Balversa is supported by data from a Phase II trial (BLC2001) that included 87 advanced bladder cancer patients with FGFR gene mutations or fusions who had progressed after chemotherapy. The overall response rate—meaning complete or total tumor shrinkage—was 32 percent, including 2 percent with complete regression. Responses were seen in some people who did not respond to PD-1/PD-L1 checkpoint immunotherapy, the current standard treatment for

advanced bladder cancer. The average duration of response was 5.4 months.

Common side effects of Balversa include mouth sores, dry mouth, nail problems, hand-foot syndrome (redness, swelling and pain on the palms of the hands and soles of the feet), dry eyes, dry skin, fatigue, diarrhea, changes in the sense of taste (dysgeusia), elevated phosphate levels and certain other laboratory abnormalities.

More rarely, Balversa may cause serious eye problems, including inflammation and disorders of the retina. The FDA recommends that people using Balversa should receive regular eye exams and tell their providers immediately if they develop blurred vision, loss of vision or other visual changes. Balversa may cause fetal harm if used during pregnancy.

Balversa received accelerated approval, a mechanism that enables faster approval of treatments for serious conditions that fill an unmet medical need. Janssen is conducting further studies to confirm that Balversa offers longer-term clinical benefit such as delayed disease progression or improved survival.

Click here for full prescribing information for Balversa.

Click here to learn more about bladder cancer.
