FDA approves gene therapy for tough-to-treat bladder cancer

December 19 2022, by Cara Murez HealthDay Reporter

Patients with a high-risk bladder cancer now have a new option to treat

it.

The U.S. Food and Drug Administration has approved a gene therapy called Adstiladrin, which is designed to work for patients who have what's called high-risk non-muscle-invasive bladder cancer (NMIBC) that hasn't responded to the standard treatment, Bacillus Calmette-Guérin (BCG), but hasn't spread. BCG is a vaccine typically used for tuberculosis.

"This approval provides health care professionals with an innovative treatment option for patients with high-risk NMIBC that is unresponsive to BCG therapy," Dr. Peter Marks, director of the FDA's Center for Biologics Evaluation and Research, said in an agency news release. "Today's action addresses an area of critical need. The FDA remains committed to facilitating the development and approval of safe and effective cancer treatments."

About 75% to 80% of newly diagnosed bladder cancers have grown through the lining of the bladder, but not yet invaded the muscle. About 30% to 80% of cases recur and risk spreading. Treatment typically involves removing the tumor and using BCG to reduce the risk that the cancer will recur.

But for patients whose cancer is unresponsive to BCG, there are few treatment options.

Patients will receive Adstiladrin once every three months into the bladder through a urinary catheter.

About 57,000 men and 18,000 women are diagnosed with bladder cancer annually, according to the U.S. Centers for Disease Control and Prevention. About 12,000 men and 4,700 women die from the disease each year in the United States.

FDA officials approved Adstiladrin based on a multicenter clinical study of 157 patients, 98 of whom had BCG-unresponsive disease that hadn't spread.

Patients received Adstiladrin once every three months for up to 12 months or until the treatment became too toxic. About 51% of enrolled patients using Adstiladrin therapy saw all signs of cancer disappear. Median response was 9.7 months. About 46% of patients who responded were in complete remission for at least one year.

The most common adverse reactions associated with Adstiladrin were bladder discharge, fatigue, bladder spasm, urinary urgency, presence of blood in urine, chills, fever and painful urination. People who are immunosuppressed or immune-deficient should not use Adstiladrin, the FDA said.

More information: The American Cancer Society has more on bladder cancer.