FDA Approves Keytruda for High-Risk Bladder Cancer

In a clinical trial, 46% of responding participants experienced a complete response lasting at least 12 months.

January 8, 2020 By Food and Drug Administration (FDA)

FDA approves pembrolizumab for BCG-unresponsive, high-risk non-muscle invasive bladder cancer

On January 8, 2020, the Food and Drug Administration approved pembrolizumab (KEYTRUDA, Merck & Co. Inc.) for the treatment of patients with Bacillus Calmette-Guerin (BCG)-unresponsive, high-risk, non-muscle invasive bladder cancer (NMIBC) with carcinoma in situ (CIS) with or without papillary tumors who are ineligible for or have elected not to undergo cystectomy.

Efficacy was investigated in KEYNOTE-057 (NCT, a multicenter, single-arm trial that enrolled 148 patients with high-risk NMIBC, 96 of whom had BCG-unresponsive CIS with or without papillary tumors. Patients received pembrolizumab 200 mg every 3 weeks until unacceptable toxicity, persistent or recurrent high-risk NMIBC or progressive disease, or up to 24 months of therapy without disease progression.

The major efficacy outcome measures were complete response (as defined by negative results for cystoscopy [with TURBT/biopsies as applicable], urine cytology, and computed tomography urography [CTU] imaging) and duration of response. The complete response rate in the 96 patients with high-risk BCG-unresponsive NMIBC with CIS was 41% (95% CI: 31, 51) and median response duration was 16.2 months (0.0+, 30.4+). Forty-six percent (46%) of responding patients experienced a complete response lasting at least 12 months.

The most common adverse reactions (incidence ≥10%) in patients who received pembrolizumab in KEYNOTE-057 were fatigue, diarrhea, rash, pruritis, musculoskeletal pain, hematuria, cough, arthralgia, nausea, constipation, urinary tract infection, peripheral edema, hypothyroidism, and nasopharyngitis.

The recommended pembrolizumab dose is 200 mg every 3 weeks.

View full prescribing information for KEYTRUDA.

Pembrolizumab was granted priority review. A description of FDA expedited programs is in the Guidance for Industry: Expedited Programs for Serious Conditions-Drugs and Biologics.

Healthcare professionals should report all serious adverse events suspected to be associated with the use of any medicine and device to FDA's MedWatch Reporting System or by calling 1-800-FDA-1088.

For assistance with single-patient INDs for investigational oncology products, healthcare professionals may contact OCE's Project Facilitate at 240-402-0004 or email OncProjectFacilitate@fda.hhs.gov.

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