FDA Approves Jelmyto for Upper Tract Urothelial Cancer

More than half of patients treated with mitomycin experienced complete tumor remission.

April 17, 2020 By Food and Drug Administration (FDA)

FDA approves mitomycin for low-grade upper tract urothelial cancer

On April 15, 2020, the Food and Drug Administration approved mitomycin (Jelmyto, UroGen Pharma) for adult patients with low-grade upper tract urothelial cancer (LG-UTUC).

Efficacy determination was based on OLYMPUS (NCT02793128), an ongoing, single-arm, multicenter trial enrolling 71 patients with treatment-naïve or recurrent low-grade non-invasive UTUC with at least one measurable papillary tumor located above the ureteropelvic junction. Patients who had larger tumors could have had prior tumor debulking. Patients received weekly JELMYTO 4 mg per mL instillations via ureteral catheter or nephrostomy tube for 6 weeks. For patients with a complete response (CR) at 3 months, instillations were to be administered monthly for a maximum of 11 additional instillations.

The major efficacy outcome measures were CR and CR durability. CR was defined as complete absence of tumor lesions 3 months after Jelmyto initiation and was assessed by urine cytology and ureteroscopy. If warranted, a biopsy was performed.

Forty-one patients (58%) achieved a CR three months following treatment initiation and were continued in follow-up; 29 patients received at least one dose of maintenance therapy.

Durability of response in those with CRs was evaluated at 3, 6, 9 and 12 months, following the CR determination.Seven patients had documented recurrences and nineteen patients remained in CR at 12-months following CR determination. The median response duration had not been reached (range: 0, 18.8+ months).

The most common adverse reactions (≥ 20%) in patients who received Jelmyto were ureteric obstruction, flank pain, urinary tract infection, hematuria, renal dysfunction, fatigue, nausea, abdominal pain, dysuria, and vomiting. Ureteric obstruction occurred in 58% of those receiving Jelmyto and required ureteral stent placement in 88% of these patients.

The recommended Jelmyto dose is 4 mg per mL instilled via ureteral catheter or nephrostomy tube, with total instillation volume based on volumetric measurements using pyelography, not

exceeding 15 mL (60 mg mitomycin).

View full prescribing information for Jelmyto.

This review used the Assessment Aid, a voluntary submission from the applicant to facilitate the FDA's assessment.

FDA granted this application priority review, fast track and breakthrough therapy designation. 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.

This announcement was originally published on the Food and Drug Administration website on April 15, 2020.
