New Real-World Study on Retrograde Instillation of JELMYTO® for Treatment of Low-Grade Upper Tract Urothelial Cancer Reports Similar Complete Response Rate and Lower Stricture Rate Compared to OLYMPUS Pivotal Trial

May 4, 2024

- **60% Complete Response Rate after JELMYTO Induction**
- **25% of Patients Received a Ureteral Stent**

PRINCETON, N.J.--(BUSINESS WIRE)--May 4, 2024-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing novel solutions that treat urothelial and specialty cancers, today highlights the results of a sub-analysis from a real-world patient cohort review of JELMYTO (mitomycin) for pyelocalyceal solution presented at the American Urological Association Meeting 2024 in San Antonio, TX. The study includes a stepwise approach to retrograde administration of JELMYTO and reports that retrograde administration of JELMYTO in the clinic (n=20) produced a 60% complete response rate in patients and ureteral stents were placed in 25% of patients, which is lower than the rate reported in the pivotal OLYMPUS study.

“The retrograde instillation of JELMYTO for the clinical management of low-grade upper tract urothelial cancer is FDA-approved and offers a safe and efficacious mode of administration, and this study in a real-world population adds to the growing body of evidence supporting retrograde administration in appropriate patients,” said Khurshid Ridwan Ghani, MBChB, MS, FRCS, Professor of Urology, Director, Michigan Urological Surgery Improvement Collaborative. “The stepwise approach outlined in this study offers valuable insights into achieving positive efficacy and safety outcomes, in the clinic with the procedure done under local anesthesia, with notable durability of response observed.”

In the OLYMPUS study all enrolled patients (n=71) received retrograde administration of JELMYTO. In the OLYMPUS study 58% (n=41) of patients achieved a complete response, defined as the absence of tumor lesions 3 months after initiation of JELMYTO treatment assessed by urine cytology and ureteroscopy. Ureteric obstruction* was reported in 58% (n=41) of patients treated with JELMYTO and 88% (n=36) of that subgroup went on to receive ureteral stent placement. For additional information about the retrograde administration of JELMYTO, consult the JELMYTO Instructions for Administration accompanying the Full Prescribing Information.

In this retrospective real-world study, 20 patients with a mean tumor burden of 1.67 cm received at least one dose of JELMYTO via the retrograde mode of administration, of which 16 (80%) completed six instillations. Twelve patients (60%) had a complete response and seven patients (35%) received at least one dose of monthly maintenance therapy, of which five demonstrated durability of response (4 patients remained tumor-free at 14.25-month follow-up and 1 patient at 24 months). Six patients (30%) had an adverse event related to the urinary system. Ureteral stents were placed in five patients for stenosis (25%); of which four were transient, with no subsequent obstruction. Only one patient (5%) required permanent stenting. Three patients who were unable to tolerate the retrograde approach had antegrade administration of JELMYTO via a nephrostomy tube.

"These findings emphasize the versatility of JELMYTO administration and also underscore the significance of continuous research and innovation in elevating treatment standards for urothelial cancers,” remarked Mark Schoenberg, M.D., Chief Medical Officer at UroGen.

The limitations of this sub-analysis include the small sample size, the retrospective design, lack of a control group, and the lack of a centralized pathology review and standardized clinicopathologic assessment.

To further explore the full potential of JELMYTO for the treatment of patients with upper tract urothelial cancers (UTUC), investigators are in the process of enrolling the prospective and retrospective uTRACT Registry to capture data in a large-scale, standardized manner to report further on patient outcomes following JELMYTO treatment including longitudinal follow-up.

*Includes hydronephrosis, obstructive uropathy, pelvic-ureteric obstruction, ureteric obstruction, ureteric stenosis, and urinary tract obstruction.

About JELMYTO

JELMYTO® (mitomycin) for pyelocalyceal solution is a mitomycin-containing reverse thermal gel containing 4 mg mitomycin per mL gel indicated for the treatment of adult patients with low grade-UTUC (LG-UTUC). JELMYTO is a viscous liquid when cooled and becomes a semi-solid gel at body temperature. The drug slowly dissolves over four to six hours after instillation and is removed from the urinary tract by normal urine flow and voiding. It is approved for administration in a retrograde manner via ureteral catheter or antegrade through nephrostomy tube. The delivery system allows the initial liquid to coat and conform to the upper urinary tract anatomy. The eventual semisolid gel allows for chemoablative therapy to remain in the collecting system for four to six hours without immediately being diluted or washed away by urine flow.

About Upper Tract Urothelial Cancer (UTUC)

Urothelial cancer is the ninth most common cancer globally and the eighth most lethal neoplasm in men in the U.S. Between five percent and ten percent of primary urothelial cancers originate in the ureter or renal pelvis and are collectively referred to as UTUC. In the U.S., there are approximately 6,000 - 7,000 new or recurrent LG-UTUC patients annually. Most cases are diagnosed in patients over 70 years old, and these older patients often face comorbidities. There are limited treatment options for UTUC, with the most common being endoscopic surgery or nephroureterectomy (removal of the entire kidney and ureter). These treatments can lead to a high rate of recurrence and relapse.

About UroGen Pharma Ltd.

UroGen is a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers because patients deserve better options. UroGen has developed *RTGel*® reverse-thermal hydrogel, a proprietary sustained-release, hydrogel-based platform technology that has the potential to improve the therapeutic profiles of existing drugs. UroGen’s sustained release technology is designed to enable longer exposure of the urinary tract tissue to medications, making local therapy a potentially more effective treatment option. Our first product to treat

LG-UTUC and investigational treatment UGN-102 (mitomycin) for intravesical solution for patients with low-grade non-muscle invasive bladder cancer are designed to ablate tumors by non-surgical means. UroGen is headquartered in Princeton, NJ with operations in Israel. Visit www.urogen.com to learn more or follow us on X (Twitter), @UroGenPharma.

APPROVED USE FOR JELMYTO

JELMYTO® is a prescription medicine used to treat adults with a type of cancer of the lining of the upper urinary tract including the kidney called low-grade Upper Tract Urothelial Cancer (LG-UTUC).

IMPORTANT SAFETY INFORMATION

You should not receive JELMYTO if you have a hole or tear (perforation) of your bladder or upper urinary tract.

Before receiving JELMYTO, tell your healthcare provider about all your medical conditions, including if you:

- are pregnant or plan to become pregnant. JELMYTO can harm your unborn baby. You should not become pregnant during treatment with JELMYTO. Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with JELMYTO. **Females who are able to become pregnant:** You should use effective birth control (contraception) during treatment with JELMYTO and for 6 months after the last dose. **Males being treated with JELMYTO:** If you have a female partner who is able to become pregnant, you should use effective birth control (contraception) during treatment with JELMYTO and for 3 months after the last dose.
- are breastfeeding or plan to breastfeed. It is not known if JELMYTO passes into your breast milk. Do not breastfeed during treatment with JELMYTO and for 1 week after the last dose.
- **Tell your healthcare provider if you take water pills (diuretic).**

How will I receive JELMYTO?

- Your healthcare provider will tell you to take a medicine called sodium bicarbonate before each JELMYTO treatment.
- You will receive your JELMYTO dose from your healthcare provider 1 time a week for 6 weeks. It is important that you receive all 6 doses of JELMYTO according to your healthcare provider's instructions. If you miss any appointments, call your healthcare provider as soon as possible to reschedule your appointment. Your healthcare provider may recommend up to an additional 11 monthly doses.
- JELMYTO is given to your kidney through a tube called a catheter.
- During treatment with JELMYTO, your healthcare provider may tell you to take additional medicines or change how you take your current medicines.

After receiving JELMYTO:

- JELMYTO may cause your urine color to change to a violet to blue color. Avoid contact between your skin and urine for at least 6 hours.
- To urinate, **males and females should sit** on a toilet and flush the toilet several times after you use it. After going to the bathroom, wash your hands, your inner thighs, and genital area well with soap and water.
- Clothing that comes in contact with urine should be washed right away and washed separately from other clothing.

JELMYTO may cause serious side effects, including:

- **Swelling and narrowing of the tube that carries urine from the kidney to the bladder (ureteric obstruction).** If you develop swelling and narrowing, and to protect your kidney from damage, your healthcare provider may recommend the placement of a small plastic tube (stent) in the ureter to help the kidney drain. Tell your healthcare provider right away if you develop side pain or fever during treatment with JELMYTO.
- **Bone marrow problems.** JELMYTO can affect your bone marrow and can cause a decrease in your white blood cell, red blood cell, and platelet counts. Your healthcare provider will do blood tests prior to each treatment to check your blood cell counts during treatment with JELMYTO. Your healthcare provider may need to temporarily or permanently stop JELMYTO if you develop bone marrow problems during treatment with JELMYTO.
- **The most common side effects of JELMYTO include:** urinary tract infection, blood in your urine, side pain, nausea, trouble with urination, kidney problems, vomiting, tiredness, stomach (abdomen) pain.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1800FDA1088. You may also report side effects to **UroGen Pharma** at 1-855-987-6436.

Please see JELMYTO Full Prescribing Information, including the Patient Information, for additional information.

Forward-Looking Statements

This press release contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, including, without limitation, statements regarding the results from the real-world study of the retrograde instillation of JELMYTO enhancing the prospects of favorable patient outcomes; the enrollment of the prospective and retrospective data from the uTRACT Patient Registry and plans to analyze and report on such data; the estimated patient population for UTUC and LG-UTUC; the potential of UroGen's proprietary *RTGel* technology to improve therapeutic profiles of existing drugs; and UroGen's sustained release technology making local delivery potentially more effective as compared to other treatment options. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: results from the real-world study of the retrograde instillation of JELMYTO may not be indicative of results that may be observed in future clinical practice and may differ from additional analysis of the data from the study or uTRACT Patient Registry; potential safety and other complications from JELMYTO use in diverse UTUC patient types; the ability to maintain regulatory approval; the ability to obtain and maintain adequate intellectual property rights and adequately protect and enforce such rights; complications associated with commercialization activities; UroGen's *RTGel* technology may not perform

as expected; and UroGen may not successfully develop and receive regulatory approval of any other product that incorporates UroGen's *RTGel* technology. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of UroGen's Annual Report on Form 10-K for the year ended December 31, 2023, filed with the SEC on March 14, 2024 (which is available at www.sec.gov), the events and circumstances discussed in such forward-looking statements may not occur, and UroGen's actual results could differ materially and adversely from those anticipated or implied thereby. Any forward-looking statements speak only as of the date of this press release and are based on information available to UroGen as of the date of this release.

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