New Real-World Retrospective Analysis Presented at AUA 2024 Reports 86% Recurrence-Free Survival (RFS) at 24 Months with JELMYTO® Across All Studied Patient and Disease Characteristics in Cohort of Responders to Induction Therapy

May 5, 2024

- There Were No Differences in RFS Regarding Usage for Chemoablation or Post-Endoscopic Ablation, Tumor Size, Multifocality, or Tumor Location
- RFS Was 100% in Patients who Received Maintenance therapy vs 61% for Patients Who Did Not Receive Maintenance Therapy

PRINCETON, N.J.--(BUSINESS WIRE)--May 5, 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 the first and largest real-world patient cohort review of JELMYTO (mitomycin) for pyelocalyceal solution presented at the American Urological Association Meeting 2024 in San Antonio, TX. Among patients with low-grade Ta upper tract urothelial carcinoma (UTUC) who were complete responders to induction therapy (n=53), JELMYTO was associated with an 86% recurrence-free survival rate at 24 months across diverse patient types regardless of initial disease characteristics or usage for chemoablation versus post-endoscopic ablation. Among the 30% of the complete responders who received maintenance therapy, RFS at 24 months was 100%, vs. 61% for those who did not receive maintenance therapy.

“The 86% recurrence-free survival rate at the 24-month mark among low-grade UTUC patients (n=53) exhibited a favorable response to the initial induction of this new treatment,” said Adam Feldman, M.D., M.P.H., Urologic Oncologist in the Department of Urology at Massachusetts General Hospital, and Director of the Combined Harvard Urologic Oncology Fellowship. “Notably, there seems to be no discernible difference in disease recurrence based on tumor characteristics or timing of administration, including primary chemoablation or post-endoscopic ablation. These results offer additional evidence for the clinical utility of JELMYTO and may also offer hope for improved long-term outcomes for our patients.”

Furthermore, a different analysis of the same patient cohort, presented by Yair Lotan, M.D., Professor of Urology and Chief of Urologic Oncology at UT Southwestern and Parkland Health and Hospital System and study investigator, reports that “maintenance therapy with JELMYTO following successful induction treatment (n = 16) yielded a RFS rate of 100%, supplying more evidence of JELMYTO’s pivotal role in treating this challenging condition,” according to Dr. Lotan.

Data was collected from 15 centers on patients treated with JELMYTO for upper tract urothelial cancers (UTUC). Recurrence-free survival was calculated in 53 patients with LGTa disease at baseline who had no evidence of disease following JELMYTO induction. Chemoablative use was defined as the administration of JELMYTO treatment in the setting of known residual UTUC, while post-chemoablation use was defined as receipt of JELMYTO following visually complete endoscopic ablation. Exploratory analyses were performed to assess impact of size of tumor, presence of ureteral involvement, and multifocality of UTUC prior to JELMYTO induction on RFS at 24 months.

“These findings provide additional evidence reinforcing JELMYTO's position as a valuable therapeutic option for patients with low-grade upper tract urothelial cancer and add to the growing body of real-world evidence in extending the long-term positive outcomes for patients,” said Mark Schoenberg, MD, Chief Medical Officer at UroGen. “This real-world evidence reaffirms JELMYTO's role as a valuable tool in our fight against this disease.”

There were 136 cases of UTUC treated with JELMYTO with a cumulative median (IQR) follow-up of 22 (12-27) months including 107 cases of LGTa UTUC. After initial treatment, 74% of post-endoscopic ablation and 39% of chemoablative patients were disease-free totaling 53 cases with LGTa UTUC without evidence of disease following JELMYTO induction. The limitations of these sub-analyses include the sample size, 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 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 long-term longitudinal follow-up.

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). It is approved for adult patients with 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). Treatment with endoscopic surgery 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.

Disclosure: Dr. Yair Lotan is a paid consultant to UroGen Pharma Ltd.

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 JELMYTO offering hope for improved long-term outcomes for patients; the enrollment of the prospective and retrospective data from the uTRACT Patient Registry and plans to analyze and report on such data; 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 of the real-world study 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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