OLYMPUS Long-Term Follow-Up Study Reports Nearly Four-Year Duration of Response in Subset of Patients with Low-Grade Upper Tract Urothelial Cancer Who Achieved a Complete Response to Initial JELMYTO Treatment

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- Data Presented at the 2025 American Urological Association Annual Meeting in Las Vegas, Nevada

PRINCETON, N.J.--(BUSINESS WIRE)--Apr. 27, 2025-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing novel solutions that treat urothelial and specialty cancers, today highlights a duration of response of nearly four years from a long-term follow-up study with JELMYTO® (mitomycin) for pyelocalyceal solution, which is FDA-approved for the treatment of low-grade upper tract urothelial cancer (LG-UTUC) in adult patients.

Among patients from the OLYMPUS trial who achieved a complete response after primary chemoablation with JELMYTO (n=41), the median duration of response was 47.8 months, irrespective of whether their cancer was new-onset or recurrent (median follow-up 28.1 months [95% CI 13.1, 57.5]). Of these patients, 21 had new-onset UTUC, and 20 had recurrent UTUC at baseline; there were no significant differences in durability between groups, with 8 patients in each group experiencing recurrence or death not due to treatment. Twenty patients entered long-term follow-up with a median follow-up of 53.3 months (95% CI 27.9, 65.3); median duration of response was not estimable (95% CI 43.5, not estimable) due to the low event rate.

“This sub-analysis highlights new evidence for the impressive long-term benefits of JELMYTO, in both new-onset and recurrent low-grade upper tract urothelial cancer patients,” said Brian Hu, MD, Associate Professor of Urology at Loma Linda University Health, CA. “These findings further establish JELMYTO as an effective treatment, providing patients with durable responses and a significant reduction in recurrence rates.”

LG-UTUC is a challenging disease often managed through endoscopically guided ablation, but recurrence is common, and patients face a lifetime of surveillance and potential complications. The OLYMPUS Phase 3 trial evaluated JELMYTO, a reverse thermal gel containing mitomycin (4 mg/mL), as a primary treatment for adults with LG-UTUC. While the overall trial demonstrated the potential of JELMYTO to significantly eradicate disease, this sub-analysis focuses specifically on patients with new-onset and recurrent LG-UTUC who achieved a complete response, providing new insights into the durability and long-term effectiveness of the treatment. Limitations of this study include the post-hoc nature of the analysis and potential selection bias regarding entry of patients into long-term follow-up.

“We are encouraged by the long-term outcomes evidence observed in both new-onset and recurrent LG-UTUC patients,” said Mark Schoenberg, MD, Chief Medical Officer, UroGen. “The sub-analysis provides further compelling evidence that JELMYTO offers durable disease control and clinically meaningful responses, which could significantly reduce the need for repeated interventions. We look forward to expanding our understanding of JELMYTO through the ongoing uTRACT Registry and further evaluating its potential in real-world settings.”

To further explore the potential of JELMYTO in treating patients with LG-UTUC, investigators are currently enrolling participants in the JELMYTO uTRACT Registry (NCT05874921) to gather longitudinal real-world usage data. As of April 2025, 22 sites have been activated with 251 patients enrolled.

About JELMYTO

JELMYTO® (mitomycin) for pyelocalyceal solution is a mitomycin-containing reverse thermal gel containing 4 mg mitomycin per mL gel approved for the treatment of 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 a 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

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 have multiple 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 be associated with 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: future results or outcomes implied from the long-term follow up study data; the estimated patient population and demographics for 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. Words such as "could," "encourage," "potential" or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: prior results may not be indicative of results that may be observed in the future; potential safety and other complications from JELMYTO use in diverse UTUC patient types; the timing and success of clinical trials and potential safety and other complications thereof; unforeseen delays that may impact the timing of progressing clinical trials and reporting data; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to obtain and maintain adequate intellectual property rights and adequately protect and enforce such rights; complications associated with commercialization activities; the labeling for any approved product; competition in UroGen's industry; the scope, progress and expansion of developing and commercializing UroGen's product candidates; the size and growth of the market(s) therefor and the rate and degree of market acceptance thereof vis-à-vis alternative therapies; UroGen's ability to attract or retain key management, members of the board of directors and other personnel; *RTGel* technology may not perform as expected; and UroGen may not successfully develop and receive regulatory approval of any other product that incorporates *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, 2024, filed with the SEC on March 10, 2025, 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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INVESTOR CONTACT:
Vincent Perrone
Senior Director, Investor Relations
vincent.perrone@urogen.com
609-460-3588 ext. 1093

MEDIA CONTACT:
Cindy Romano
Director, Corporate Communications
cindy.romano@urogen.com
609-460-3583 ext. 1083

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