UroGen Announces New Data from the OLYMPUS Trial that Shows Median Durability of Response of 28.9 Months for JELMYTO®, the Only Non-Surgical, Chemoablative Treatment for Adults with Low-Grade Upper Tract Urothelial Cancer

December 1, 2022

--Results from this ongoing, non-interventional, rollover study were presented at the 23rd Annual Society of Urologic Oncology (SUO) Meeting in San Diego

SAN DIEGO--(BUSINESS WIRE)--Dec. 1, 2022-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to creating novel solutions that treat urothelial and specialty cancers, today announced new data from the OLYMPUS registration trial designed to obtain long-term follow-up data on JELMYTO® (mitomycin) for pyelocalyceal solution that shows median durability of response (DOR) of 28.9 months. The study (Abstract #158) was presented at SUO on December 1.

“The clinical benefit of JELMYTO was demonstrated in the Phase 3 OLYMPUS study and data presented today highlighted the long-term durability of that benefit,” said Dr. Phillip Pierorazio, M.D., Chief, Section of Urology at Penn Presbyterian Medical Center, Philadelphia, PA. “JELMYTO provides an effective and durable kidney-sparing treatment option and should be considered as primary therapy for adult patients with LG-UTUC.”

Patients who completed OLYMPUS were eligible to participate in this rollover study. Outcomes of interest include DOR in patients who remain in complete response (CR) at the end of OLYMPUS, events of disease recurrence and progression, post-study treatments and death.

At the time of data cut off (February 25, 2022), data were available for 16 of 23 patients who had remained in CR at the end of the OLYMPUS study. The median DOR among the 16 patients was 28.9 months (14.6 to 47.6 months). Thirteen patients remained in CR, two patients had recurrence of low-grade upper tract urothelial carcinoma (LG-UTUC) on the same side as treated in OLYMPUS, and one patient underwent radical nephroureterectomy (RNU) due to ureteral stricture without evidence of UTUC at the time of surgery. No patient had progressed to high-grade disease.

“JELMYTO is an important addition to the urologist’s tool kit for treating LG-UTUC,” said Mark Schoenberg, M.D., Chief Medical Officer, UroGen. “These data are the first to show the potential for long-term recurrence free survival in patients treated with JELMYTO. We look forward to additional independent validation of this important observation.”

About the Pivotal OLYMPUS Study

OLYMPUS (Optimized DeLiverY of Mitomycin for Primary UTUC Study) was an open-label, single-arm Phase 3 clinical study of UGN-101 JELMYTO (mitomycin) for pyelocalyceal solution, to evaluate the safety, tolerability and tumor ablative effect of JELMYTO in patients with LG-UTUC. Seventy-one patients were treated at clinical sites across the United States and Israel. Study participants were treated with six weekly instillations of JELMYTO administered via a standard catheter. Four to six weeks following the last instillation, patients underwent a Primary Disease Evaluation (PDE) to determine CR, the primary endpoint of the study. PDE involved a ureteroscopy and wash cytology, a standard microscopic test of cells obtained from the urine to detect cancer and for cause biopsy. Patients who achieved a CR at the PDE timepoint were eligible for the maintenance phase of the trial, during which they could receive monthly maintenance instillations for up to 12 months and were assessed quarterly to determine the durability of response with JELMYTO.

In the OLYMPUS study, data was generated for the retrograde administration of JELMYTO. In that study population ureteric stenosis was reported in 58% (n=41) of patients receiving JELMYTO, with only 17% (n=12) of patients experiencing a Grade 3 event.

About LG-UTUC

LG-UTUC is a rare disease managed by endoscopic methods and radical nephroureterectomy. Endoscopic resection and laser ablation attempt to preserve the kidney, though there is a high risk of recurrence that may eventually necessitate removal of the kidney. Although kidney removal is the current standard for treatment of high-grade UTUC, it may be over-treatment in LG-UTUC, as kidney removal offers similar five-year survival as kidney-sparing procedures but is associated with significant morbidity.

About UroGen Pharma Ltd.

UroGen is 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 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. UroGen’s first commercial product JELMYTO (mitomycin) for pyelocalyceal solution, 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 Twitter, @UroGenPharma.

About JELMYTO®

JELMYTO® (mitomycin) for pyelocalyceal solution is a mitomycin-containing reverse thermal gel containing 4 mg mitomycin per mL gel indicated for primary chemoablative treatment of LG-UTUC in adults. It is recommended for primary treatment of biopsy-proven LG-UTUC in patients deemed appropriate candidates for renal-sparing therapy. 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.

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 U.S. Food and Drug Administration. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. 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 ongoing, non-interventional, rollover study of JELMYTO and the design thereof; the potential benefits of JELMYTO, including its durability of response and halting of disease progression; the potential of RTGel™ to improve the 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 initial reports of long-term follow-up outcomes may not be indicative of results that may be observed in future clinical practice; potential safety and other complications from the use of JELMYTO; and the ability to maintain regulatory approval. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of UroGen's Form 10-Q filed with the SEC on November 10, 2022 and other filings that UroGen makes with the SEC from time to time (which are available at http://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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20221201005672/en/

INVESTORS:
Vincent Perrone
Sr. Director, Investor Relations
vincent.perrone@urogen.com
609-460-3588 ext. 1093

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

Source: UroGen Pharma Ltd.