UroGen Pharma Announces Podium Presentation of Final Jelmyto® Data From Pivotal OLYMPUS Trial at the Annual Meeting of the Society of Urologic Oncology

December 3, 2020

- *Clinically Meaningful Response Underscores Potential for Jelmyto to Become New Standard of Care for Patients with Low-Grade Upper Tract Urothelial Cancer*
- *Of the 58% of Patients who Achieved a Complete Response, 12-Month Durability of Response Estimated at 81.8% by Kaplan-Meier Analysis*
- *Median Time to Recurrence was Not Reached*

PRINCETON, N.J.--(BUSINESS WIRE)--Dec. 3, 2020-- UroGen Pharma Ltd. (Nasdaq: URGN) a biopharmaceutical company dedicated to building and commercializing novel solutions that treat specialty cancers and urologic diseases, today announced the presentation of final data from the UGN-101 Jelmyto® (mitomycin) for pyelocalyceal solution Phase 3 OLYMPUS trial in patients with low-grade upper tract urothelial cancer (LG-UTUC). The results will be presented as a virtual podium presentation at the 21st Annual Meeting of the Society of Urologic Oncology (SUO):

- **Oral Session:** Best of Bladder Cancer
- **Abstract #:** 1003
- **Title:** Durability of Response to Chemoablative Treatment of Low-Grade Upper Tract Urothelial Carcinoma with a Mitomycin-Containing Reverse Thermal Hydrogel: Final Results of the OLYMPUS Trial
- **Presenter:** Surena F. Matin, M.D., Professor of Urology at The University of Texas MD Anderson Cancer Center in Houston, TX
- **Session Date & Time:** Saturday, December 5, 2020; 2:00 pm Eastern Time

Final results from the Phase 3 OLYMPUS trial of Jelmyto, the first and only non-surgical kidney-sparing treatment approved by the U.S. Food and Drug Administration (FDA) for adults with LG-UTUC, show that Jelmyto demonstrated clinically meaningful response in adults with LG-UTUC. In both the OLYMPUS intent-to-treat population and in the sub-population of patients who were deemed to have unresectable disease at study entry, 58% of patients achieved a complete response (CR) with durability of response at 12-months estimated to be 81.8% by Kaplan-Meier analysis. Median time to recurrence was not reached.

“Current options for patients with low-grade upper tract urothelial carcinoma are not optimal. They include multiple endoscopic procedures or the removal of the kidney and ureter, both of which have consequences impacting patient health and quality of life,” said Surena F. Matin, M.D., Professor of Urology at The University of Texas MD Anderson Cancer Center in Houston, TX and Investigator of the OLYMPUS trial. “The results of the OLYMPUS study represent the first prospective trial in this space, supporting the use of Jelmyto as a less-invasive, kidney-preserving, durable treatment for low-grade upper tract urothelial carcinoma, which may reduce the need for multiple endoscopic procedures or loss of a kidney.”

The safety profile in the final OLYMPUS data was consistent with previously reported results. The most common adverse events (AE) were uretic stenosis, urinary tract infection, hematuria, flank pain, nausea, dysuria, renal dysfunction, abdominal pain and vomiting.

About the Phase 3 OLYMPUS Trial

OLYMPUS (Optimized DeLiverY of Mitomycin for Primary UTUC Study) is an open-label, single-arm Phase 3 clinical trial of UGN-101, Jelmyto (mitomycin) for pyelocalyceal solution, to evaluate the safety, tolerability and tumor ablative effect of Jelmyto in patients with low-grade 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 Complete Response (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 continued onto the maintenance phase of the trial, during which they were to receive monthly maintenance instillations for up to 12 months to determine the durability of response with Jelmyto.

About Jelmyto®

Jelmyto (mitomycin) for pyelocalyceal solution, is a drug formulation of mitomycin indicated for the treatment of adult patients with low-grade upper tract urothelial cancer (LG-UTUC). Utilizing the RTGel™ technology platform, UroGen’s proprietary sustained release, hydrogel-based formulation, Jelmyto is designed to enable longer exposure of urinary tract tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means. Jelmyto is delivered to patients using standard ureteral catheters or nephrostomy tube. The U.S. FDA previously granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to Jelmyto for the treatment of LG-UTUC. On April 15, 2020, the FDA approved Jelmyto, making it the first drug approved for the treatment of LG-UTUC in adult patients.

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: side pain, urinary tract infection, blood in your urine, kidney problems, tiredness, nausea, stomach pain, trouble with urination, vomiting, low red blood cell count, frequent urination, itching, chills, and fever.

You are encouraged to report negative side effects of prescription drugs to the FDA. 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 at www.jelmyto.com.

About UroGen Pharma Ltd.

UroGen is a biopharmaceutical company dedicated to building novel solutions that treat specialty cancers and urologic diseases 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 approved product, Jelmyto (mitomycin) for pyelocalyceal solution, and investigational treatment UGN-102 (mitomycin) for intravesical solution, are designed to ablate tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade upper tract urothelial cancer and low-grade non-muscle invasive bladder cancer, respectively. UroGen is headquartered in Princeton, NJ with operations in Israel. Visit www.urogen.com to learn more or follow us on Twitter, @UroGenPharma.

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: the potential of Jelmyto®, to transform the treatment of LG-UTUC; the potential for Jelmyto to become the new standard of care for patients with LG-UTUC; the potential for the use of Jelmyto to reduce the need for multiple endoscopic procedures or loss of a kidney; and the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of clinical trials and potential safety and other complications thereof; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to maintain regulatory approval; complications associated with commercialization activities, including complications resulting from the ongoing COVID-19 pandemic; the labeling for any approved product; 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 personnel; and any negative effects on UroGen's business, commercialization and product

development plans caused by or associated with COVID-19. 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 9, 2020, 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.

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