UroGen Pharma Presents Data Showcasing Novel Clinical Data at 2021 American Urological Association (AUA) Annual Meeting

September 13, 2021

- *Final data from Phase 2b OPTIMA II trial of UGN-102 showed complete and durable response for intravesical instillation in patients with low-grade intermediate risk NMIBC*
- *Long-term data from pivotal OLYMPUS trial underscores durable benefit of Jelmyto® for adult patients with low-grade upper tract urothelial cancer*
- *Post-hoc analysis of female patients in OLYMPUS trial showed complete response rates and complete response durability similar to male patients*

PRINCETON, N.J.--(BUSINESS WIRE)--Sep. 13, 2021-- UroGen Pharma Ltd. (Nasdaq: URGN) today announced final data from two key trials, evaluating the safety and efficacy of investigational agent UGN-102 (mitomycin) for intravesical solution in adult patients with low-grade intermediate risk non-muscle invasive bladder cancer (LG IR-NMIBC) and *Jelmyto®* (mitomycin) for pyelocalyceal solution in adult patients with low-grade upper tract urothelial cancer (LG-UTUC). The results were presented at the virtual 2021 American Urological Association (AUA) Annual Meeting and published as a supplement to the September 10, 2021 issue of *The Journal of Urology®*.

"RTGel™, the proprietary technology at the core of*Jelmyto* and UGN-102, significantly increases dwell time and is the first major advancement in many years for the localized treatment of low-grade upper tract urothelial cancer and non-muscle invasive bladder cancer, where invasive surgery was considered the standard of care," said Dr. Mark Schoenberg, Chief Medical Officer at UroGen. "These data support our objective of changing the way these types of cancers are treated, and further, we believe this success validates the broader platform both in low grade disease, with UGN-102, as well as our expansion into high grade disease and other tumor types."

Phase 2b OPTIMA II

A podium presentation of the final OPTIMA II trial results [21-8601-Podium Presentation] showed that 65% (41/63) of patients receiving UGN-102 achieved a complete response (CR) three months after the start of therapy. In this subset of patients, 95% (39/41) of patients, 73% (30/41) of patients and 61% (25/41) of patients who were present for evaluation at each timepoint, remained disease free at six, nine and 12 months following treatment initiation, respectively. Thirteen patients had documented recurrences. The probability of durable response nine months after CR (12 months after treatment initiation) was estimated to be 73% by Kaplan-Meier analysis.

"Low-grade intermediate-risk non-muscle-invasive bladder cancer is a recurrent disease, requiring repetitive transurethral surgeries," said William C. Huang, M.D., FACS, Professor of Urology and Radiology and Vice Chair of Urology at NYU Langone Health and Principal Investigator of the OPTIMA II trial. "Having to endure repeated surgeries may lead to post-operative and long-term morbidity for this patient population. The final results from OPTIMA II, showing significant treatment response and sustained durability, indicate that UGN-102 may provide a non-surgical treatment option for these chronically relapsing patients."

The most common adverse events (≥10%) were reported as mild to moderate and included dysuria, hematuria, urinary frequency, fatigue, urgency and urinary tract infection.

Phase 3 OLYMPUS

Results of 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, demonstrated clinically meaningful response in adults with LG-UTUC. Of 71 patients who initiated treatment, trial results showed 58% (41/71) achieved CR with durability of response at 12 months estimated to be 81.8% by Kaplan-Meier analysis. In this subset of patients, 56% (23/41) remained in CR after 12 months, including 50% (6/12) who did not receive any maintenance instillations and 59% (17/29) who received ≥1 maintenance instillation. The most common adverse reactions (≥20%) reported in the OLYMPUS trial were ureteric obstruction, urinary tract infection, hematuria, flank pain, nausea, dysuria, renal dysfunction, vomiting, fatigue, and abdominal pain.

Results from a separate, post-hoc analysis of female patients from the OLYMPUS trial [21-9960-Moderated poster –Linehan], showed similar CR and durability of CR to male patients, with 65.2% of female patients achieving CR and 71.4% maintaining durable CR at 12 months compared to 81% for the entire patient population. The most common adverse events were urinary tract infection, hematuria, ureteric stenosis, flank pain, vomiting, hydronephrosis, dysuria, and nausea. Additional research is warranted to more clearly define gender-related outcomes for female patients with LG-UTUC.

"Urothelial carcinoma is less common in men than women but some studies have shown worse outcomes. In the subgroup analysis of the Olympus trial, women had 71% CR and durability of 12 months compared to 81% with the whole cohort. This is comparable and not statistically significant," said Jennifer Linehan, M.D., Associate Professor of Urology and Urologic Oncology at Providence St. John's Cancer Institute and an Investigator of the OLYMPUS trial. "We are encouraged by this follow-up data to the OLYMPUS trial affirming that regardless of gender, *Jelmyto* can be an important, kidney-sparing alternative to patients living with this cancer."

About the Phase 2b OPTIMA II Trial

OPTIMA II (OPTimized Instillation of Mitomycin for Bladder Cancer Treatment) is an open-label, single-arm, multi-center Phase 2b clinical trial [21-8601-Podium Presentation] of investigational agent UGN-102 (mitomycin) for intravesical solution to evaluate its safety and efficacy in patients with low-grade non-muscle invasive bladder cancer (LG NMIBC) at intermediate risk of recurrence. Intermediate risk is defined as one or two of the following: multiple tumors, a lowgrade solitary tumor >3 cm, or recurrence of LG NMIBC within one year of the current diagnosis. Patients were to receive six weekly intravesical instillations of 75 mg UGN-102 in an office setting. The chemoablative effect of UGN-102 was assessed three months

after initiation of study treatment with complete response (CR) defined as a negative endoscopic examination, negative cytology, and when indicated, a negative for-cause biopsy. Patients achieving CR were followed quarterly to 12 months after initiation of study treatment to evaluate safety, efficacy, and durability.

About UGN-102

UGN-102 (mitomycin) for intravesical solution is an investigational drug formulation of mitomycin in Phase 3 development for the treatment of low-grade intermediate risk non-muscle invasive bladder cancer. Utilizing the RTGel™ Technology Platform, UroGen's proprietary sustained release, hydrogel-based formulation, UGN-102 is designed to enable longer exposure of bladder tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means. UGN-102 is delivered to patients using a standard urinary catheter. The Company reported final results from the Phase 2b OPTIMA II trial in November 2020 and initiated a Phase 3 study to further investigate UGN-102 in the treatment of this condition in December 2020. The results of the Optima II Phase 2b study have been accepted by The Journal of Urology for publication.

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 eligible for the maintenance phase of the trial, during which they could receive monthly maintenance instillations for up to 12 months and were assessed 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 a 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 takewater 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 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.

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. *Jelmyto*® (mitomycin) for pyelocalyceal solution and investigational treatment UGN-102 (mitomycin) for intravesical solution 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.

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: clinical trial results validating UroGen's broader platform both in low grade disease as well as the expansion into high grade disease and other tumor types; the potential for UGN-102 to provide a non-surgical treatment option; the possibility of *Jelmyto* being an important, kidney-sparing alternative to patients living with urothelial cancer, regardless of gender; 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; third parties, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to maintain regulatory approval; 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 August 4, 2021, 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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INVESTOR CONTACT:
Lee Roth
lroth@burnsmc.com
212-213-0006

MEDIA CONTACT:
Alice Sofield
Alice.Sofield@bcw-global.com
703-861-5654

Source: UroGen Pharma Ltd.