First Patient Dosed in Phase 3 Clinical Trial of UGN-103, a Next Generation Mitomycin-Based Formulation in Development for the Treatment of Low-Grade Intermediate-Risk Non-Muscle Invasive Bladder Cancer

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- Anticipated advantages of UGN-103 include a shorter manufacturing process and a simpler reconstitution procedure

PRINCETON, N.J.--(BUSINESS WIRE)--Oct. 2, 2024-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced that the first patient was dosed in the Phase 3 clinical trial of investigational drug UGN-103 (mitomycin) for intravesical solution in development for the treatment of low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). UGN-103 is a next-generation novel mitomycin-based formulation.

“Reaching this Phase 3 trial milestone for UGN-103 highlights our drive to innovate and bring forward cutting-edge treatments for low-grade intermediate-risk non-muscle invasive bladder cancer,” said Liz Barrett, President and Chief Executive Officer, UroGen. “UGN-103 represents a significant step forward, offering potential improvements in manufacturing, convenience, and cost. We are excited about the potential of this next-generation formulation and look forward to furthering its development to advance the care of patients.”

The UGN-103 formulation uses UroGen’s *RTGel*® platform technology, a proprietary sustained-release, reverse-thermal hydrogel with the potential to improve the therapeutic profiles of existing drugs. The U.S. Food and Drug Administration (FDA) accepted the Investigational New Drug Application for UGN-103 in April 2024, permitting the drug’s investigational use in adults with LG-IR-NMIBC, a highly recurrent disease. UGN-103 is planned to follow the potential FDA approval and launch of UGN-102 (mitomycin) for intravesical solution for LG-IR-NMIBC. UroGen completed the New Drug Application (NDA) submission for UGN-102 ahead of schedule with potential FDA approval in early 2025, if the NDA is accepted for filing by the FDA and priority review is granted.

The UTOPIA study is a single-arm, multicenter study that will evaluate the efficacy and safety of UGN-103. UroGen is aiming to enroll 87 patients with LG-IR-NMIBC in the UTOPIA study. Patients will receive 75 mg of UGN-103 via intravesical instillation once a week for 6 weeks. Efficacy will be assessed by the complete response rate at the three-month visit. Patients who have a complete response at the three-month visit, defined as having no detectable disease in the bladder, will enter the follow-up period of the study. During the follow-up period, patients will return to the clinic every three months for evaluation of response. Patients will remain on study until disease recurrence, disease progression, death, or the last patient completes 12 months of follow-up (i.e., 15 months after the first instillation), whichever occurs first. To learn more about the UTOPIA trial, visit clinicaltrials.gov/NCT06331299.

About UGN-103

UGN-103 (mitomycin) for intravesical solution is an innovative drug formulation of mitomycin being developed for the treatment of LG-IR-NMIBC. Anticipated advantages of UGN-103 include a new formulation of mitomycin that may considerably shorten the manufacturing process and simplify the reconstitution procedure. UroGen announced on September 16, 2024, that it received from the United States Patent and Trademark Office (USPTO), a Notice of Allowance covering, among other things, the use of UGN-103 in the treatment of LG-IR-NMIBC. The U.S. patent, once issued, will have an expiration date in December 2041. UGN-103 will utilize UroGen’s proprietary *RTGel* technology, a sustained release, hydrogel-based formulation designed to enable longer exposure of bladder tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means.

About UGN-102

UGN-102 (mitomycin) for intravesical solution is an innovative drug formulation of mitomycin, currently in Phase 3 development for the treatment of LG-IR-NMIBC. Utilizing UroGen’s proprietary *RTGel* technology, a 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 in an outpatient setting by a trained healthcare professional. UroGen completed the NDA submission for UGN-102 in August 2024, ahead of schedule, with a potential FDA decision as early as the first quarter of 2025, if the NDA is accepted for filing by the FDA and priority review is granted.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

In the U.S. bladder cancer is the second most common urologic cancer in men. LG-IR-NMIBC represents approximately 22,000 newly diagnosed bladder cancer patients each year and an estimated 60,000 recurrences annually among patients diagnosed from previous years. Bladder cancer primarily affects older populations with the median age of diagnosis 73 years and an increased risk of comorbidities. Guideline recommendations for the management of NMIBC include TURBT as the standard of care. Up to 70 percent of NMIBC patients experience at least one recurrence and LG-IR-NMIBC patients are even more likely to recur and face repeated TURBT procedures.

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 low-grade upper tract urothelial cancer, and our investigational treatment UGN-102 (mitomycin) for intravesical solution for patients with LG-IR-NMIBC 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.

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 belief that UGN-103, if approved, will provide several advantages related to production, cost, supply, product convenience, and intellectual property protection; UroGen's anticipated receipt of a U.S. patent with an expiration date in December 2041; the potential for UGN-102 to introduce a new non-surgical treatment option for LG-IR-NMIBC; 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; the potential timing for a decision from the FDA on the UGN-102 NDA submission and the potential for priority review, as well as plans for UGN-103 to follow; the design and objectives of the UTOPIA study of UGN-103; and the estimated annual U.S. patient population and demographics for LG-IR-NMIBC. Words such as "aim," "anticipate," "can," "plan," "potential," "will," 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: the anticipated advantages of UGN-103 have not yet been demonstrated or achieved, as applicable, and it is possible one or more of these anticipated advantages will not be realized; preliminary results may not be indicative of results that may be observed in the future; 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, reporting data and initiating product launches; the ability to obtain regulatory approval within the timeframe expected, or at all; even if an NDA for UGN-102 is accepted for filing by the FDA, there is no guarantee that such NDA will be given priority review or that such NDA will be sufficient to support approval of UGN-102 on the timeframe expected, or at all; 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 *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 Quarterly Report on Form 10-Q for the quarter ended June 30, 2024, filed with the SEC on August 13, 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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