New Long-Term Follow-Up Data from OPTIMA II Study of UGN-102 Demonstrates Median Duration of Response of Two Years in Patients with LG-IR-NMIBC

April 27, 2025

- Data Presented at the American Urological Association 2025 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 innovative solutions that treat urothelial and specialty cancers, today announced new data from the OPTIMA II Phase 2b study of UGN-102 (mitomycin) for intravesical solution demonstrate clinically meaningful two-year duration of response (24.2 months) by Kaplan-Meier analysis. UGN-102 is UroGen's sustained-release formulation of mitomycin being developed for the treatment of recurrent low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC).

"The median duration of response of two years highlights UGN-102's durability, even in patients with recurrent disease and multiple prior TURBT procedures," said Neal Shore, MD, Medical Director, Carolina Urologic Research Center, Myrtle Beach, South Carolina. "These long-term data provide encouraging evidence of UGN-102's sustained impact."

The majority of patients included in OPTIMA II had recurrent disease at baseline (77.8%), with multiple prior transurethral resection of bladder tumor (TURBT) procedures. Among the 41 patients achieving a complete response (CR) at three months, 25 remained in CR at 12 months, and 17 of these patients entered long-term follow-up. The median Kaplan–Meier estimate of duration of response for the 41 patients that achieved CR was 24.2 months (95% CI 9.72, 47.18), with a median follow-up time of 33.6 months (95% CI 10.78, 42.94). Twenty patients (48.8%) experienced recurrence of low-grade disease. One patient progressed to high-grade disease and one patient died due to a cardiac disorder. Five patients remained disease-free at the time of the four-year data analysis.

"As the burden of LG-IR-NMIBC persists, the need for long-lasting treatment options becomes increasingly urgent," said Mark Schoenberg, Chief Medical Officer, UroGen. "The growing body of evidence supporting UGN-102 underscores its potential to address this unmet need. These results emphasize UGN-102's potential to deliver meaningful and sustained responses, offering hope to patients who have long struggled with recurrence and limited treatment options."

UroGen completed the submission of the UGN-102 rolling new drug application (NDA) to the U.S. Food and Drug Administration (FDA) for UGN-102 in August 2024, ahead of schedule. The FDA accepted the NDA for UGN-102 with a Prescription Drug User Free Act (PDUFA) goal date of June 13, 2025.

The most common treatment-emergent adverse events (TEAEs) in the ENVISION trial were dysuria, hematuria, urinary tract infection, pollakiuria, fatigue, and urinary retention. The TEAEs were typically mild-to-moderate in severity and either resolved or were resolving. The ENVISION trial demonstrated a similar safety profile to that observed in other studies of UGN-102.

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 recurrent 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 submission of the rolling NDA for UGN-102 in August 2024, ahead of schedule. The FDA accepted the NDA for UGN-102 and assigned a PDUFA goal date of June 13, 2025.

About Non-Muscle Invasive Bladder Cancer (NMIBC)

LG-IR-NMIBC affects around 82,000 people in the U.S. every year and of those, an estimated 59,000 are recurrent. Bladder cancer primarily affects older populations with increased risk of comorbidities, with the median age of diagnosis being 73 years. 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 OPTIMA II

OPTIMA II (OPTimized Instillation of Mitomycin for Bladder Cancer Treatment) was an open-label, single-arm, multi-center Phase 2b clinical trial of investigational drug UGN-102 to evaluate its safety and efficacy in patients with LG-IR-NMIBC.

Learn more about the Phase 2b OPTIMA II trial at **www.clinicaltrials.gov** (NCT03558503).

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 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 durability potential for UGN-102 and the potential to benefit patients and address unmet needs; statements related to UroGen's NDA submission and the expected PDUFA goal date for UGN-102; the estimated patient population and demographics for 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. Words such as "expect" and "potential," or other words that convey uncertainty of future events or outcomes are used to identify these forward-looking statements. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: initial 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 and reporting data; even though the NDA for UGN-102 has been accepted for filing by the FDA, there is no guarantee that such NDA will be sufficient to support approval of UGN-102 on the timeframe expected, or at all; the PDUFA goal date may be delayed due to various factors outside UroGen's control; the ability to obtain and maintain adequate intellectual property rights and adequately protect and enforce such rights; the ability to obtain and maintain regulatory approval; 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; UroGen may not successfully develop and receive regulatory approval of any other product that incorporates *RTGel* technology; and UroGen's *RTGel* technology may not perform as expected. 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 ending 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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Source: UroGen Pharma Ltd.