UGN-102 Showed Promising Long-Term Results in the Phase 3 ENVISION Trial, Potentially Paving the Way for First FDA-Approved Treatment for LG-IR-NMIBC

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- UGN-102 demonstrated 82.3% duration of response (DOR) at 12 months in patients who achieved complete response at 3 months
- 79.6% complete response rate at 3 months in patients treated with UGN-102
- Safety profile consistent with prior clinical trials of UGN-102

PRINCETON, N.J.--(BUSINESS WIRE)--Dec. 5, 2024-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced the presentation of the Phase 3 ENVISION trial's efficacy and safety results at the Society of Urologic Oncology (SUO) annual meeting in Dallas, TX. These results, published online in the *Journal of Urology* in October, demonstrate that treatment with investigational therapy UGN-102, a mitomycin-based intravesical solution, resulted in a high and clinically meaningful complete response rate that was durable in patients with recurrent low-grade intermediate-risk non-muscle-invasive bladder cancer (LG-IR-NMIBC).

"Finding options for patients with recurrent low-grade bladder cancer continues to be a major unmet need," says Max Kates, R. Christian B. Evensen Professor and an Associate Professor of Urology and Oncology, and Director, Division of Urologic Oncology at the Brady Urological Institute at Johns Hopkins and ENVISION study investigator. "We are excited by the overall results of the ENVISION trial, especially the durability of response data, that support the potential of UGN-102 as a viable treatment option for these patients."

In the ENVISION study, UGN-102 treatment showed an impressive 82.3% (95% CI: 75.9%, 87.1%) duration of response (DOR) at 12 months, according to the Kaplan-Meier estimate, in patients who achieved a complete response (CR) at 3 months following the initial treatment with UGN-102. The DOR at 15 months (n=43) and 18 months (n=9) remained robust, both at 80.9% (95% CI: 73.9%, 86.2%) according to the Kaplan-Meier estimates. These results build upon the trial's positive primary endpoint, a 79.6% (95% CI: 73.9%, 84.5%) CR rate 3 months after the first instillation of UGN-102.

The side effect profile of UGN-102 was consistent with previous clinical trials, further supporting its potential as a new treatment option for patients with LG-IR-NMIBC.

"We are excited by the progress made in advancing UGN-102 as a potential treatment for LG-IR-NMIBC and securing a PDUFA goal date of June 13, 2025, from the FDA," said Mark Schoenberg, M.D., Chief Medical Officer, UroGen. "The strong durability of response observed in the ENVISION study highlights UGN-102's promising potential for patients. Given that many LG-IR-NMIBC patients are elderly and endure multiple surgeries under general anesthesia for their condition that impact their health and quality of life, there is an urgent need for alternative treatment options that can prolong recurrence-free periods and enhance patient outcomes."

The most common treatment-emergent adverse events (TEAEs) in the ENVISION trial were dysuria, hematuria, urinary tract infection, pollakiuria, fatigue, and urinary retention. TEAEs were typically mild-to-moderate in severity and resolved or 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 LG-IR-NMIBC. Utilizing UroGen's proprietary *RTGel*® technology, a sustained release, hydrogel-based formulation, UGN-102 is designed to treat tumors by enabling longer exposure of bladder tissue to mitomycin. 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, 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)

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 increased risk of comorbidities, with the median age of diagnosis being 73 years. Guideline recommendations for the management of NMIBC include trans-urethral resection of bladder tumor (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. LG-IR-NMIBC is characterized by early or frequent recurrences, multiple tumors, or a solitary tumor larger than three centimeters.

About ENVISION

The Phase 3 ENVISION trial is a single-arm, multinational, multicenter study evaluating the efficacy and safety of UGN-102 (mitomycin) for intravesical solution as a therapy for patients with LG-IR-NMIBC. The Phase 3 ENVISION trial completed target enrollment with approximately 240 patients across 56 sites. Study participants received six once-weekly intravesical instillations of UGN-102. The primary endpoint evaluated the CR rate at the three-month assessment after the first instillation, and the key secondary endpoint evaluated durability over time in patients who achieved a CR at the three-month assessment. Learn more about the Phase 3 ENVISION trial at www.clinicaltrials.gov (NCT05243550).

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 potential for UGN-102 as the first FDA-approved treatment for LG-IR-NMIBC; the long-term results in the ENVISION trial supporting the potential of UGN-102 as a viable treatment option for LG-IR NMIBC; the estimated annual U.S. patient population and demographics for LG-IR-NMIBC; the potential benefits to patients and opportunities for UGN-102, if approved; statements related to the PDUFA goal date for UGN-102 and the potential approval and timing thereof; 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. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: 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 ability to 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'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 September 30, 2024, filed with the SEC on November 6, 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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