ENVISION Trial Results Published in the Journal of Urology Report 82.3% Duration of Response 12 Months after Achieving Complete Response for UGN-102, Potentially First FDA-Approved Non-Surgical Treatment for LG-IR-NMIBC

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- The Kaplan-Meier Estimate of Duration of Response at 12 Months in Patients Who Achieved a Complete Response at Three Months was 82.3%
- Patients Receiving UGN-102 had a 79.6% Complete Response Rate at Three Months
- Side Effect Profile Consistent with Previous Clinical Trials of UGN-102

PRINCETON, N.J,--(BUSINESS WIRE)--Oct. 28, 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 new long-term durability of response results from the Phase 3 ENVISION study of investigational drug UGN-102 in patients with low-grade intermediate-risk non-muscle-invasive bladder cancer (LG-IR-NMIBC) were published online in the *Journal of Urology*.

In ENVISION, UGN-102 treatment was associated with an unprecedented 82.3% (95% CI, 75.9%, 87.1%) 12-month duration of response (DOR) by Kaplan-Meier estimate (n=108) in patients who achieved complete response (CR) at three months after the first instillation of investigational drug UGN-102 (mitomycin) for intravesical solution. The DOR Kaplan-Meier estimates at 15 months (n=43) and 18 months (n=9) after 3-month CR were both 80.9% (95% CI, 73.9%, 86.2%). The ENVISION trial previously met its primary endpoint by demonstrating that patients treated with UGN-102 had a 79.6% (95% CI, 73.9%, 84.5%) CR rate at three months following the first instillation of UGN-102.

“These data demonstrate that treatment with UGN-102 results in a clinically meaningful CR rate and that the durability of the response in patients with LG-IR-NMIBC is robust,” said Sandip Prasad, MD, M.Phil., Director of Genitourinary Surgical Oncology, Morristown Medical Center/Atlantic Health System, NJ and Principal Investigator of the ENVISION trial. “This study adds to the mounting evidence supporting UGN-102 as a potentially valuable treatment option for patients with recurrent LG-IR-NMIBC.”

UroGen initiated the submission of a rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) for UGN-102 as a treatment for LG-IR-NMIBC in January 2024 and completed the NDA submission in August, ahead of schedule. The FDA accepted the NDA for UGN-102 with a PDUFA goal date of June 13, 2025.

Liz Barrett, President and CEO of UroGen, emphasized the significance of the DOR findings from the ENVISION study, stating, “LG-IR-NMIBC patients are typically elderly and currently treated with repeated surgeries under general anesthesia that are associated with physical and quality of life detriments. These new results continue to strengthen UGN-102’s potential as a treatment option for these patients that extends their recurrence-free interval. The highly recurrent nature of LG-IR-NMIBC underscores the urgent need for innovative options for patients facing this challenging disease."

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 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 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.

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 chemoablative therapy in 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 chemoablative, non-surgical treatment 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 UroGen's NDA submission 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 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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