



## UroGen Announces Results from Subgroup Analyses of the Pivotal ENVISION Trial Evaluating Impact of Baseline Tumor Burden and Focality on Response to UGN-102

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- Results presented at the American Society of Clinical Oncology's Genitourinary Cancers Symposium (ASCO GU 2025)
- Treatment with UGN-102 resulted in durable complete response (CR) rates across subgroups with recurrent low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC)
- CR rates were:
  - 82.8% for patients with a tumor burden  $\leq 3$  cm compared to 73.2% for those with a tumor burden  $> 3$  cm
  - 79.3% for patients with multiple tumors compared to 82.9% for those with single tumors
- CR rates and duration of response (DOR) patterns were similar across tumor burden and focality subgroups

PRINCETON, N.J.--(BUSINESS WIRE)--Feb. 14, 2025-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced results from the subgroup analyses of the pivotal ENVISION study evaluating the impact of tumor burden and focality on the response to UGN-102, an investigational drug in development for treatment of LG-IR-NMIBC. Analyses of pre-specified subgroups showed comparable CR rates and DOR patterns across different levels of tumor burden (defined as the sum of the diameters of all visible tumors) and number of tumors at baseline in patients treated with UGN-102. The poster presentation titled, **Impact of Tumor Burden or Focality in Recurrent Low-Grade Intermediate-Risk Non-Muscle-Invasive Bladder Cancer on Response to Treatment with UGN-102: A Substudy of the Phase 3 ENVISION Trial** was presented today at ASCO GU 2025.

"In this subgroup analysis on tumor burden and focality from the ENVISION study, the overall CR rate was consistent, and a majority of patients maintained their response 12 months after achieving CR at three months," said Sandip M. Prasad, M.D., Director of Urology at Morristown Medical Center and lead author of the study. "These results provide evidence of UGN-102's potential to become an effective and durable treatment option for patients with recurrent LG-IR-NMIBC, regardless of tumor size or focality."

The ENVISION study included 240 patients with recurrent LG-IR-NMIBC who received at least one dose of UGN-102 and 95% (228 patients) received all six weekly intravesical instillations of UGN-102. In this pre-specified analysis CR rates were 82.8% for patients with a tumor burden  $\leq 3$  cm (n=149/180) compared to 73.2% for those with a tumor burden  $> 3$  cm (n=30/41). Of the patients with CR at three months, 15.4% vs 20.0% experienced recurrence of low-grade disease, progression (either in stage or grade), or death (unrelated to treatment) by 15 months. In the 191 patients with multiple vs single tumors who achieved a CR, the three-month CR was 79.3% vs 82.9%, with recurrence rates of 18.5% vs 11.8%.

"We are encouraged by these results, which highlight the potential of UGN-102 to provide durable responses in patients with recurrent LG-IR-NMIBC," said Mark Schoenberg, M.D., Chief Medical Officer of UroGen. "These pre-specified subgroup analyses further strengthen the growing body of evidence supporting UGN-102 as a treatment option for patients dealing with the challenges of disease recurrence and the need for repeated surgeries."

The ENVISION trial's primary efficacy and safety results, previously presented, showed that UGN-102 delivered a 79.6% CR rate at three months, with an unprecedented 82.3% probability of remaining in response 12 months later by Kaplan-Meier estimates.

These findings further support UGN-102's potential to alter the treatment landscape for patients with LG-IR-NMIBC, a condition where recurrence and progression remain significant clinical challenges.

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 Prescription Drug User Fee 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.

Study limitations include the small sample size of the comparator groups and the single arm design.

The analysis in another presentation titled, **Treatment of Low-Grade Intermediate-Risk Non-Muscle-Invasive Bladder Cancer with UGN-102: Results of the Phase 3 ATLAS and ENVISION Studies** highlights that in both studies, the CR rate in patients initially treated with UGN-102 was robust, with the majority of patients remaining event-free at 12 months after a three-month CR. A new analysis from the ATLAS trial highlights 54% decreased risk of recurrence, progression or death with UGN-102 compared to TURBT. The hazard ratio for DOR in ATLAS was 0.46 with a 95% confidence interval (0.24, 0.86) favoring the UGN-102 arm. Median DOR was not estimable in any arm due to low recurrence rates; the most common adverse event with UGN-102 in both studies was dysuria, occurring in 22.5% in ENVISION and 30.4% in ATLAS.

### 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 *RTGe*<sup>®</sup> 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 23,000 newly diagnosed bladder cancer patients each year and an estimated 59,000 recurrences annually among patients diagnosed in 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](http://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 *RTGeI* 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. To learn more, visit [www.UroGen.com](http://www.UroGen.com) 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 ENVISION subgroup analyses providing evidence of UGN-102's potential to become an effective and durable treatment option for patients with recurrent LG-IR-NMIBC, regardless of tumor size or focality and further strengthening the growing body of evidence supporting UGN-102 as a treatment option for patients dealing with the challenges of disease recurrence and repeated surgeries; the potential benefits to patients and opportunities for UGN-102, if approved, including to alter the treatment landscape for patients with LG-IR-NMIBC; statements related to UroGen's NDA submission and expected PDUFA target action date for UGN-102; the estimated annual U.S. patient population and demographics for LG-IR-NMIBC; the potential of UroGen's proprietary *RTGeI* 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: prior results may not be indicative of results that may be observed in the future; 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 *RTGeI* technology may not perform as expected; and UroGen may not successfully develop and receive regulatory approval of any other product that incorporates *RTGeI* 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](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:**

Vincent Perrone  
Senior Director, Investor Relations  
[vincent.perrone@urogen.com](mailto:vincent.perrone@urogen.com)  
609-460-3588 ext. 1093

#### **MEDIA CONTACT:**

Cindy Romano  
Director, Corporate Communications  
[cindy.romano@urogen.com](mailto:cindy.romano@urogen.com)  
609-460-3583 ext. 1083

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