UroGen Announces Unprecedented 82.3% Duration of Response at 12 Months in the ENVISION Trial Investigating UGN-102 as Potentially the First FDA-Approved Non-Surgical Treatment for LG-IR-NMIBC

June 13, 2024

- Kaplan-Meier Estimate of Duration of Response at 12 Months in Patients Who Achieved a Complete Response at Three Months was 82.3% (95% CI, 75.9%, 87.1%)
- Side Effect Profile Consistent with Previous Clinical Trials of UGN-102
- UroGen will Host a Virtual Event Today at 11:00 AM Eastern Time

PRINCETON, N.J.--(BUSINESS WIRE)--Jun. 13, 2024-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing novel solutions that treat urothelial and specialty cancers, today announced 82.3% (95% CI, 75.9%, 87.1%) 12-month duration of response (DOR) data by Kaplan-Meier estimate (n=108) from its Phase 3 ENVISION trial in patients who achieved complete response (CR) at three months after the first instillation of investigational drug UGN-102 (mitomycin) for intravesical solution. 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.

The ENVISION Phase 3 study is investigating UGN-102 in patients with low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). In addition to the 12-month data, the DOR Kaplan Meier estimates at 15 (n=43) and 18 (n=9) months were both 80.9% (95%CI, 73.9%, 86.2%).

“UGN-102 has demonstrated a strong clinical profile across multiple trials, with these latest results of 79.6% three-month complete response rate and 82.3% DOR at 12 months reinforcing its potential to be the first FDA-approved non-surgical option for treatment of LG-IR-NMIBC,” said Liz Barrett, President and Chief Executive Officer of UroGen. “We estimate 82,000 patients suffer from this highly recurrent disease in the U.S. each year and may benefit from an innovative approach to treating their 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. The ENVISION trial demonstrated a similar safety profile to that observed in other studies of UGN-102.

“These DOR findings continue to support the development of UGN-102 as a non-surgical alternative to the current standard of care of repeated surgeries for LG-IR-NMIBC, which can impact patients’ physical health and quality of life,” said Sandip Prasad, MD, M.Phil., Director of Genitourinary Surgical Oncology, Morristown Medical Center/Atlantic Health System, NJ. “These results from the ENVISION study make me very optimistic about the opportunity for UGN-102, if approved, to provide another option for patients living with this highly recurrent disease.”

“While LG-IR-NMIBC’s highly recurrent nature often means patients must undergo numerous surgeries throughout their lifetime, I am excited about the potential for patients to better manage the ongoing burden of this disease,” said Mark Schoenberg, M.D., Chief Medical Officer, UroGen. “UGN-102, if approved, could provide a minimally invasive option for LG-IR-NMIBC patients whose existing treatment options currently center around repetitive surgeries.”

In January 2024, 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. The latest DOR data are expected to support the UGN-102 NDA, which the Company plans to complete in the third quarter of 2024, with a potential FDA decision as early as the first quarter of 2025.

UGN-102 ENVISION DATA Virtual Event

The Company is hosting a data event featuring a panel discussion with leading bladder cancer experts today, Thursday, June 13, 2024, at 11:00 a.m. Eastern Time to discuss results from the Phase 3 ENVISION clinical trial.

Please register for the webinar under the Events & Presentations section of the Company’s Investor Relations site (https://investors.urogen.com/events-and-presentations).

Following the live webcast, a replay will be available on the Company's website (https://urogen.com).

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 anticipates completing its NDA submission for UGN-102 in the third quarter of 2024 with a potential FDA decision as early as the first quarter of 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 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 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 primary 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. Based on discussions with the FDA, UroGen anticipates completing its NDA submission for UGN-102 in the third quarter of 2024. 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 non-surgical treatment for LG-IR-NMIBC; the estimated annual U.S. patient population and demographics for LG-IR-NMIBC; the potential opportunities for UGN-102, if approved, including to provide a minimally invasive option for patients with LG-IR-NMIBC and to help such patients better manage the ongoing burden of the disease; statements related to UroGen's planned NDA submission for UGN-102 and the potential timing for a decision from the FDA thereon; 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: the ENVISION DOR data may not be sufficient to support an NDA submission for UGN-102; even if an NDA for UGN-102 is accepted 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 obtain regulatory approval within 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; UroGen may not successfully develop and receive regulatory approval of any other product that incorporates *RTGel* technology; UroGen's financial condition and need for additional capital in the future. 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 March 31, 2024, filed with the SEC on May 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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