UroGen Submits Completed UGN-102 NDA Seeking Approval as the First FDA-Approved Treatment for Low-Grade Intermediate-Risk Non-Muscle Invasive Bladder Cancer

August 14, 2024

PRINCETON, N.J.--(BUSINESS WIRE)--Aug. 14, 2024-- UroGen Pharma Ltd. (Nasdaq: URGN), a leading biotech company specializing in novel therapies for urothelial and specialty cancers, today announced the successful completion of its New Drug Application (NDA) submission for investigational drug UGN-102, (mitomycin) for intravesical solution, a significant step forward in potentially addressing the urgent need for innovative treatments for low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). UroGen anticipates potential FDA approval in early 2025, if the NDA is accepted for filing by the FDA and priority review is granted.

"The completion of the NDA submission for UGN-102 marks a crucial milestone for UroGen and underscores our dedication to advancing this groundbreaking treatment for patients with LG-IR-NMIBC," said Liz Barrett, President and CEO of UroGen. "By providing a viable alternative to repeated surgeries, if approved UGN-102 may offer patients quality of life benefits and clinically meaningful recurrence-free intervals. The high recurrence rates associated with LG-IR-NMIBC make the need for innovative therapies like UGN-102 urgent. UGN-102 could become a valuable new option for managing this challenging disease."

The NDA is supported by the clinical program for UGN-102, including the long-term durability results from the Phase 3 ENVISION study. The ENVISION trial met its primary endpoint by demonstrating that patients treated with UGN-102 had a 79.6% complete response (CR) rate at three months following the first instillation. UGN-102 demonstrated an 82.3% 12-month duration of response (DOR) by Kaplan-Meier estimate (n=108) in patients who achieved a CR at three months. The DOR estimates at 15 (n=43) and 18 (n=9) months after 3-month CR were both 80.9%.

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.

For more information and perspective on the ENVISION study results and the unmet need for patients with LG-IR-NMIBC click here to view the webcast: UGN-102 12-Month Durability of Response Data Event.

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.

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 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 treatment for LG-IR-NMIBC; the estimated annual U.S. patient population and demographics for LG-IR-NMIBC; the potential patient benefits and opportunities for UGN-102, if approved, the potential timing for a decision from the FDA on the UGN-102 NDA submission and the potential priority review; 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 NDA may not be accepted for filing by the FDA, and even if it is, there is no guarantee that such NDA will be given priority review or 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 March 31, 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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INVESTOR CONTACT:
Vincent Perrone
Senior Director, Investor Relations
vincent.perrone@UroGen.com
609-460-3588 ext. 1093

MEDIA CONTACT:
Cindy Romano
Director, Communications
cindy.romano@urogen.com
908-963-7827

Source: UroGen Pharma Ltd.