Investigational Therapy UGN-102 May Be Delivered at Home for the Treatment of Bladder Cancer

February 15, 2023

--In a Small Feasibility Study, UGN-102 Achieves a Complete Response in 75% (6 of 8) Patients

--UGN-102 is Being Studied in Low-Grade Intermediate-Risk Non-Muscle Invasive Bladder Cancer

PRINCETON, N.J.--(BUSINESS WIRE)--Feb. 15, 2023-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced preliminary results of a study to assess the feasibility of home instillation of UGN-102 (mitomycin) for intravesical solution, an investigational therapy in development for primary chemoablation of low-grade, intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). In this study, UGN-102 was suitable to administer at home by a visiting nurse under the supervision of a treating physician and resulted in 75% of patients achieving a complete response, defined as no detectable disease 3 months after starting treatment.

“Many bladder cancer patients are elderly and suffer from comorbidities that make frequent office visits for treatment a real burden,” said study investigator David Morris, M.D., F.A.C.S., Urologist at Urology Associates PC, Nashville, TN. “Moving healthcare out of the clinical setting and into a patient’s home would represent a new treatment paradigm for bladder cancer, which may reduce clinic and hospital costs while increasing patient comfort and convenience.”

Eight patients were enrolled and treated with UGN-102 by 4 investigators. Median age was 75 years (range 55-84). Six patients completed treatment (6 doses), and 2 patients discontinued the study (5 doses and 4 doses, respectively) due to adverse events (AEs) unrelated to treatment. All 8 patients were evaluable for treatment response, and 6 of 8 (75%) achieved a complete response 3 months after starting treatment. The 2 patients who discontinued were assessed as non-responders. Treatment-related AEs were mild to moderate in severity (most common: dysuria and fatigue in 2 patients), and 3 patients with significant comorbidities had a serious AE, all of which were unrelated to treatment with UGN-102.

Patients, nurses and investigators completed home instillation feasibility questionnaires. These standardized feasibility questionnaires highlighted that all 8 patients preferred at-home to in-office treatment, and 5 of 6 patients recommended UGN-102 home instillation instead of transurethral resection of bladder tumors (TURBT). Home instillation was reported as feasible for visiting nurses, and 3 of 4 investigators considered at-home treatment “not different” than in-office treatment.

“As a urologic oncologist, I feel confident that the home instillation study results, appearing on the heels of our Phase 2 program and following the complete enrollment of our pivotal Phase 3 ENVISION study for UGN-102, provide us with even more reasons to believe that our novel approach to treating LG-IR-NMIBC has the potential to address genuine unmet needs of patients with bladder cancer,” said Arie Belldegrun, M.D., Board Chair of UroGen.

The study enrolled patients with recurrent LG-IR-NMIBC who agreed to receive UGN-102 at home. Six weekly doses of UGN-102 were administered. The first dose was administered on-site by a physician and the five remaining doses were administered at home by a visiting nurse. Patients were followed for AEs and treatment response was evaluated by visual observation, for cause biopsy, and urine cytology 3 months after treatment initiation. Complete response was defined as the absence of disease by white light cystoscopy, cytology, and histopathology.

“This study is another example of how UroGen continues to advance its mission to pioneer the way urothelial cancers are treated,” says Liz Barrett, President and Chief Executive Officer, UroGen. “2023 will be a pivotal year for UroGen as we report on the ATLAS study of UGN-102 and start combinatorial treatments in the Phase 1 study of UGN-301. UroGen is in a strong position to achieve leadership in uro-oncology.”

About LG-IR-NMIBC

Approximately 800,000 people are living with bladder cancer in the U.S., of that 80,000 suffer from LG-IR-NMIBC. Patients with LG-IR-NMIBC face a future of recurrence and additional surgeries. Currently, the only primary treatment available is a surgical procedure known as TURBT, which requires anesthesia. Every time TURBT is performed it may impose more burden and serious risks on patients, including pain, bleeding, infection and injury (including perforation).

About UGN-102

UGN-102 (mitomycin) for intravesical solution is an investigational drug formulation of mitomycin 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. Assuming positive findings from the ENVISION Phase 3 study, UroGen anticipates submitting a New Drug Application (NDA) for UGN-102 in 2024. If approved, UGN-102 would be the first non-surgical primary therapeutic to treat a subset of bladder cancer characterized by high recurrence rates and multiple surgeries.

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 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. UroGen’s first commercial product, 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 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 statements regarding the Home Instillation Study of UGN-102, and the encouraging results from this study; the design and potential benefits of UGN-102 for the treatment of LG-IR-NMIBC, including benefits related to at home administration; the ongoing Phase 3 ENVISION trial and the design, timing and potential benefits thereof; UroGen's plans to submit an NDA for UGN-102 and the expected timing thereof; UroGen's optimism regarding the clinical potential of UGN-102, including UroGen's belief that UGN-102 meets unmet needs of patients with bladder cancer; UroGen's expectations for 2023, including that 2023 will be a pivotal year, the reporting on the ATLAS study of UGN-102 and start of combinatorial treatments in the Phase 1 study of UGN-301; management's belief that UroGen is in a strong position to achieve leadership in uro-oncology; 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: preliminary results from the Home Instillation Study of UGN-102 may not be indicative of results that may be observed in the future; the timing and success of clinical trials and potential safety or other complications thereof, including the Phase 3 ENVISION trial; unforeseen delays that may impact the timing of progressing clinical trials and reporting data; the ability to obtain regulatory approval within the timeframe expected, or at all, and to maintain regulatory approval; complications associated with commercialization activities; UroGen's ability to attract or retain key management, members of the board of directors and personnel; the labeling for any approved product; competition in UroGen's industry; the scope, progress and expansion of developing and commercializing UroGen's product and 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; and 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 our 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 Form 10-Q filed with the SEC on November 10, 2022 and other filings that UroGen makes with the SEC from time to time (which are available at 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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Source: UroGen Pharma Ltd.