Complete and Durable Responses Observed in OPTIMA II Phase 2b Final Results for UGN-102 in Patients with Low-Grade Intermediate Risk Non-Muscle Invasive Bladder Cancer

November 17, 2020

- *65% Complete Response Rate at Three Months*
- *Duration of Response Estimated to be 72.5% at Nine Months (12-Months from Initiation of Therapy) by Kaplan-Meier Method; Median Duration of Response was Not Reached*
- *Treatment with UGN-102 was Generally Well Tolerated, with Mostly Mild to Moderate Adverse Events Reported*
- *Phase 3 Pivotal Trial Evaluating UGN-102 Versus Current Standard of Care Expected to Start by Year-End*

PRINCETON, N.J.--(BUSINESS WIRE)--Nov. 17, 2020-- UroGen Pharma Ltd. (Nasdaq: URGN) today announced final topline results from the single-arm, open-label OPTIMA II Phase 2b trial evaluating the efficacy and safety of investigational UGN-102 (mitomycin) for intravesical solution in patients with low-grade intermediate risk non-muscle invasive bladder cancer (LG IR-NMIBC). As previously reported, 65% (41/63) of patients receiving UGN-102 achieved a complete response (CR) three months after the start of therapy. In this subset of patients, duration of response at nine months (12-months from start of therapy) was estimated by Kaplan-Meier analysis to be 72.5%. Median duration of response was not reached. The Company expects to initiate a Phase 3 study evaluating UGN-102 versus current standard of care by the end of the year.

Treatment with UGN-102 was generally well tolerated and the safety profile was consistent with previously reported results. In the OPTIMA II trial, the majority of the most common adverse events (≥ 10%) were reported as mild to moderate in severity and include dysuria, urinary frequency, hematuria, urinary urgency, urinary tract infection and fatigue. No treatment related serious adverse events were reported.

“The current approach to treating patients diagnosed with low-grade intermediate risk non-muscle invasive bladder cancer is surgery. In most cases, the cancer comes back and repetitive surgical intervention is required. This puts a tremendous burden on patients and their families and can even be life-threatening,” said Andrea Maddox-Smith, Chief Executive Officer, Bladder Cancer Advocacy Network. “Being able to provide patients with an alternative, non-surgical treatment option that is effective, well-tolerated and durable, would greatly benefit those in the patient community.”

Patients with LG IR-NMIBC are chronically relapsing and currently, their only treatment option is repeated transurethral resection of bladder tumors (TURBT). Some patients require multiple TURBT surgeries per year, which can lead to increased morbidity and risks associated with repetitive anesthesia. It is estimated that 80,000 people in the U.S. are treated annually for LG IR-NMIBC. This includes newly diagnosed patients and patients who have a recurrence after surgery.

“We are extremely encouraged by the OPTIMA II data and believe UGN-102 has the potential to provide a safe, durable, outpatient treatment alternative for low-grade intermediate risk non-muscle invasive bladder cancer,” said Dr. Mark Schoenberg, Chief Medical Officer at UroGen. “We look forward to the expected initiation of our Phase 3 trial this year and further exploring the potential of our innovative technology in advancing new treatments for specialty cancers and urologic diseases.”

The Company anticipates submitting the data for presentation at an upcoming medical meeting as well as potential publication in a peer-reviewed journal.

About the Phase 2b OPTIMA II Trial

OPTIMA II (OPTimized Instillation of Mitomycin for Bladder Cancer Treatment) is an open-label, single-arm, multi-center Phase 2b clinical trial of investigational agent UGN-102 (mitomycin) for intravesical solution to evaluate its safety and efficacy in patients with low-grade non-muscle invasive bladder cancer (LG NMIBC) at intermediate risk of recurrence. Intermediate risk is defined as one or two of the following: multiple tumors, solitary tumor >3 cm, or recurrence (≥ 1 occurrence of LG NMIBC within one year of the current diagnosis). Patients were to receive six weekly intravesical instillations of 75 mg UGN-102 in an office setting. The chemoablative effect of UGN-102 was assessed three months after initiation of study treatment with complete response (CR) defined as a negative endoscopic examination, negative cytology, and when indicated, a negative for-cause biopsy. Patients achieving CR were followed quarterly to 12 months after initiation of study treatment to evaluate safety, efficacy, and durability.

About UGN-102

UGN-102 (mitomycin) for intravesical solution is an investigational drug formulation of mitomycin in Phase 2b development for the treatment of low-grade intermediate risk non-muscle invasive bladder cancer. Utilizing the RTGel Technology Platform, UroGen’s proprietary 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. The Company reported topline interim results from the Phase 2b OPTIMA II trial in May 2020 and intends to begin a Phase 3 study to further investigate UGN-102 in the treatment of this condition by year end 2020.

About UroGen Pharma Ltd.

UroGen is a biopharmaceutical company dedicated to building novel solutions that treat specialty cancers and urologic diseases 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 approved product, Jelmyto (mitomycin) for pyelocalyceal solution, and investigational treatment UGN-102 (mitomycin) for intravesical solution, are designed to ablate tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade upper tract urothelial cancer and low-grade non-muscle invasive bladder cancer, respectively. 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, without limitation: the potential of UroGen's proprietary RTGel technology platform to improve therapeutic profiles of existing drugs; the potential of UGN-102 for treatment of LG IR-NMIBC; the expected initiation of the Phase 3 trial of UGN-102 in LG IR-NMIBC by the end of 2020; and the submission of UGN-102 data for presentation at an upcoming medical meeting as well as potential publication in a peer-reviewed journal. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of clinical trials and potential safety and other complications thereof; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to maintain regulatory approval; complications associated with commercialization activities, including complications resulting from the ongoing COVID-19 pandemic; the labeling for any approved product; 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 personnel; and any negative effects on UroGen's business, commercialization and product development plans caused by or associated with COVID-19. 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 9, 2020, 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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