The Journal of Urology Publishes Results of UroGen Pharma's Phase 2b Study of UGN-102 in Patients with Low-Grade Intermediate Risk Non-Muscle Invasive Bladder Cancer (LG IR-NMIBC)

October 5, 2021

- *Clinically Meaningful Response and Sustained Durability Underscore Potential for UGN-102 to Become a Non-Surgical Primary Therapeutic Treatment for Patients with Highly Recurrent LG IR-NMIBC*
  - *65% Complete Response (CR) Rate at Three Months*
  - *61% of Patients Remain in CR at 12 Months*
- *Data Supports Ongoing Phase 3 Development of UGN-102 in LG IR-NMIBC as an Alternative to Surgery*

PRINCETON, N.J.--(BUSINESS WIRE)--Oct. 5, 2021-- UroGen Pharma Ltd. (Nasdaq: URGN) today announced *The Journal of Urology*® published results from the Phase 2b OPTIMA II trial, a Phase 2b, open-label, single-arm trial studying UGN-102 (mitomycin) for intravesical solution as primary non-surgical therapy for low-grade intermediate risk non-muscle invasive bladder cancer (LG IR-NMIBC). The study was published online and will be in the January print edition of *The Journal of Urology*®.

Approximately 50-60% of the 81,000 estimated new cases of non-muscle invasive bladder cancer (NMIBC) diagnosed in the U.S. in 2020 were low-grade. Patients with LG IR-NMIBC are chronically relapsing, and their only treatment option is repeated transurethral resection of bladder tumor (TURBT) with or without adjuvant chemotherapy. Some patients require multiple TURBT surgeries per year, which may lead to post-operative and long-term morbidity for this patient population.

The OPTIMA II Phase 2b results showed a significant treatment response with sustained durability in non-surgical chemoablation of 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, of whom 39 (95%), 30 (73%), and 25 (61%) remained disease-free at 6, 9, and 12 months after treatment initiation, respectively. The probability of durable response nine months after CR (12 months after treatment initiation) was estimated to be 72.5% by Kaplan-Meier analysis. Thirteen patients had documented recurrences. 57 of 63 (90%) patients completed all 6 instillations of UGN-102 according to study protocol.

"The data from the OPTIMA II trial represent a significant advancement in the development of UGN-102 for the treatment of LG IR-NMIBC and further support the contention that non-surgical therapy for this disease is both technically feasible and clinically meaningful," said Dr. Mark Schoenberg, Chief Medical Officer at UroGen. "With UGN-102, our goal is to change the treatment paradigm in LG IR-NMIBC by enabling non-surgical chemoablation. These results provide an important validation of our approach to treating low-grade disease, and our rationale for the continued evaluation of UGN-102 in the ongoing Phase 3 ATLAS study."

Treatment with UGN-102 was generally well tolerated and the safety profile was consistent with previously reported results. The most common adverse events (≥ 10%) were generally reported as mild to moderate and included dysuria, urinary frequency, hematuria, micturition urgency, urinary tract infection, and fatigue.

"The significant treatment response and sustained durability observed in this trial indicate that UGN-102 has the potential to become a non-surgical alternative for these chronically relapsing patients, who typically undergo repetitive surgeries," said William C. Huang, M.D., FACS, Professor of Urology and Radiology and Vice Chair of Urology at NYU Langone Health and Principal Investigator of the OPTIMA II trial. "Even more encouraging is that 12 months after treatment was initiated, approximately 73% achieved durable response."

Final top-line data were announced in November 2020 and additional details were presented at the American Urological Association (AUA) 2021 Annual Meeting in September 2021 [21-8601-Podium Presentation].

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, a low-grade solitary tumor >3 cm, or recurrence 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 3 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 final results from the Phase 2b OPTIMA II trial in November 2020 and initiated a Phase 3 study to further investigate UGN-102 in the treatment of this condition in December 2020. Additional details of the OPTIMA II Phase 2b study have been published in *The Journal of Urology*®.

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. Jelmyto® (mitomycin) for pyelocalyceal solution and investigational treatment UGN-102 (mitomycin) for intravesical solution 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, without limitation, statements regarding: the potential for UGN-102 to become the first non-surgical primary therapeutic treatment for patients with highly recurrent bladder cancer; and the potential of UroGen's proprietary RTGel technology platform to improve therapeutic profiles of existing drugs. 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; third parties, including regulatory agencies, may not accept or agree with our assumptions, estimates, calculations, conclusions or analyses or may interpret or weigh the importance of data differently, which could impact the value of the particular program, the approvability or commercialization of the particular product candidate or product and our company in general; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to maintain regulatory approval; 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 August 4, 2021, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20211005005335/en/

INVESTOR CONTACT:
Lee Roth
lroth@burnsmc.com

MEDIA CONTACT:
Alice Sofield
Alice.Sofield@bcw-global.com
703-861-5654

Source: UroGen Pharma Ltd.