UroGen Pharma Announces First Presentation of Data from Phase 2b Study of UGN-102 in Patients with Difficult to Treat Type of Bladder Cancer

May 15, 2020

- *65% Complete Response in Patients with Low-Grade Intermediate Risk Non-Muscle Invasive Bladder Cancer*
- *Of Patients who Achieved a Complete Response and Underwent an Evaluation at Each Timepoint, 97%, 86% and 85% Remained Disease Free at Six, Nine and 12 Months Following Initiation of Therapy, Respectively*
- *Positive Data Continue to Validate UroGen's Proprietary Technology Platform and Value of Intravesical Therapy Dwell Time*
- *American Urological Association Abstract Presentations Available Online In Lieu of Annual Meeting*

PRINCETON, N.J.--(BUSINESS WIRE)--May 15, 2020-- UroGen Pharma Ltd. (Nasdaq: URGN) today announced the presentation of positive interim data from the Phase 2b OPTIMA II trial evaluating the safety and efficacy of investigational agent UGN-102 (mitomycin) for intravesical solution in patients with low-grade intermediate risk non-muscle invasive bladder cancer (LG IR-NMIBC). The study was accepted for the 2020 American Urological Association (AUA) Annual Meeting, published as a supplement to the April 2020 issue of *The Journal of Urology*® and presented as part of the AUA Virtual Experience. The presentation can be accessed via the AUA website here.

Trial results showed that 65% (41/63) of patients treated with UGN-102 achieved a complete response (CR) three months after the start of therapy. In this subset of patients, 97% (35/36) of patients (95% Confidence Interval), 86% (24/28) of patients and 85% (11/13) of patients who were present for evaluation at each timepoint, remained disease free at six, nine and 12 months following treatment initiation, respectively. Follow-up will continue until all patients have reached the 12-month time point.

"With UGN-102, we have an opportunity to fundamentally change the way low-grade intermediate risk non-muscle invasive bladder cancer is treated and help patients avoid recurrence of their cancer and repetitive surgeries," said Dr. Mark Schoenberg, Chief Medical Officer at UroGen. "The positive data presented today, coupled with previously presented data in low-grade upper tract urothelial cancer, continue to validate our technology platform and our hypothesis that increased dwell time significantly improves the effectiveness of intravesical therapy."

Non-Muscle Invasive Bladder Cancer (NMIBC) is stratified into three risk categories: low, intermediate, and high risk.[1] Patients with low risk disease require relatively modest intervention, while those with high risk disease are at risk for disease progression necessitating organ removal. Patients with intermediate risk disease have a clinical course that is best characterized as chronically recurrent. These patients often undergo repetitive transurethral resection of bladder tumors (TURBT), which can lead to increased morbidity and risks of repetitive anesthesia. Some patients require more than one or two surgical procedures per year.[2] It is estimated that 80,000 Americans suffer from intermediate risk NMIBC.[3,4,5]

"The current approach to treating patients with low-grade non-muscle invasive bladder cancer is transurethral surgery. Unfortunately, there is a high rate of recurrence and these patients often need repetitive surgeries, which can result in an increased risk of complications," said William C. Huang, M.D., FACS, Associate Professor and Vice Chair of Urology at NYU Langone Health and Principal Investigator of the OPTIMA II trial. "The interim results from OPTIMA II indicate that UGN-102, if approved, may provide an effective, non-surgical treatment option for these patients that is both well-tolerated and durable."

In OPTIMA II the most common adverse events (≥ 10%) were reported as mild to moderate and include dysuria, hematuria, urinary frequency, fatigue, urgency and urinary tract infection. Discussions for the planned Phase 3 clinical trial protocol for the study of UGN-102 in patients with LG IR-NMIBC are underway with the U.S. Food and Drug Administration.

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 UGN-102 (mitomycin) for intravesical solution to evaluate its safety and efficacy in patients with low-grade non-muscle invasive bladder (LG NMIBC) cancer 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 1 year of the current diagnosis).

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 completed enrollment in the Phase 2b OPTIMA II trial in September 2019 and intends to advance the program to a pivotal study to further investigate UGN-102 in the treatment of this condition.

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 *Jelmyto*™ (mitomycin) for pyelocalyceal solution, and pipeline 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 opportunity for UGN-102 to fundamentally change the way low-grade intermediate risk non-muscle invasive bladder cancer is treated; the potential for UGN-102 to help patients avoid recurrence of their cancer and repetitive surgeries; the potential of UGN-102 to provide an effective treatment option that is both well-tolerated and durable; the potential advancement of UGN-102 to a pivotal study; 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; 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 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 Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission (SEC) on May 7, 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. UroGen undertakes no obligation to revise or update the information in this press release to reflect events or circumstances after the date hereof, except as required by law.

References:

1. Chang SS, Boorjian SA, Chou R, et al.: Diagnosis and Treatment of Non-Muscle Invasive Bladder Cancer: AUA/SUO Guideline. J Urol. 2016;196(4):1021–9. 10.1016/j.juro.2016.06.049
2. Huang W, Chevli K, Trainer A, et al.: Can TURBT Be Avoided? Primary Chemoablation with a Reverse Thermal Gel Containing Mitomycin (UGN-102) in Patients with Low Grade Intermediate Risk Non-Muscle Invasive Bladder Cancer: 2020 by American Urological Association Education and Research, Inc.
3. National Cancer Institute: Surveillance, Epidemiology, and End Results (SEER) Program (www.seer.cancer.gov) SEER*Stat Database: Incidence - SEER 21 Regs Research Data, released April 2019, based on the November 2018 submission.
4. Babjuk M, Burger M, CoShariat SF, Sylvester R, Zigeuner R, Capoun O, Cohen D, Escrig JLD, Hernández V, Peyronnet B, Seisen T, Soukup V. European Association of Urology Guidelines on Non-muscle-invasive Bladder Cancer (TaT1 and mpérat EM, Gontero P, Mostafid AH, Palou J, van Rhijn BWG, Rouprêt M, Carcinoma In Situ) - 2019 Update. European Urology 76(5):639-657, Nov 2019
5. Simon M, Bosset P-O, Rouanne M, Benhamou S, Radulescu C, Molinié V, et al. (2019) Multiple recurrences and risk of disease progression in patients with primary low-grade (TaG1) non–muscle-invasive bladder cancer and with low and intermediate EORTC-risk score. PLoS ONE 14(2): e0211721.

View source version on businesswire.com: https://www.businesswire.com/news/home/20200515005286/en/

INVESTOR CONTACT:
Kate Bechtold
Senior Director, Investor Relations
Kate.Bechtold@urogen.com
914-552-0456

MEDIA CONTACT:
Eric Van Zanten
Senior Director, Communications
Eric.VanZanten@urogen.com
610-529-6219

Source: UroGen Pharma Ltd.