UroGen Pharma Announces Positive Interim Results from Pivotal Phase 3 OLYMPUS Trial of UGN-101 (MitoGel™) for Non-Surgical Treatment of Upper Tract Urothelial Cancer (UTUC)

May 21, 2018

Interim Results Showed Complete Response (CR) Rate of 59 Percent in Patients with Low-Grade UTUC

CRs to Date Remain Durable at Three, Six and Nine-Month Follow-Up

RA'ANANA, Israel and NEW YORK, May 21, 2018 (GLOBE NEWSWIRE) -- UroGen Pharma Ltd. (Nasdaq:URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in the field of urology, with a focus on uro-oncology, today announced new findings from an interim analysis of the ongoing pivotal Phase 3 OLYMPUS clinical trial of UGN-101 (MitoGel™), an investigational mitomycin formulation for the non-surgical treatment of low-grade upper tract urothelial cancer (UTUC). Results were presented by Seth Paul Lerner, M.D., FACS, Principal Investigator of the OLYMPUS trial and Professor of Urology at Baylor College of Medicine in Houston in an oral presentation during the plenary session at the 113th American Urological Association's (AUA) Annual Meeting in San Francisco.

The interim analysis from this international, multi-center trial showed a complete response (CR) rate of 59 percent in 34 patients who were evaluated for primary disease evaluation (PDE, or the primary endpoint). PDE is conducted four to six weeks after completion of UGN-101 treatment, which was administered once weekly for six weeks. Results showed that 20 of the interim analysis intent to treat population of 34 patients (59 percent) achieved a CR, defined as a negative ureteroscopic evaluation and a negative wash cytology. In addition, five of 34 patients (15 percent) achieved a partial response. Approximately 39 percent of tumors treated were categorized as unresectable by surgery at baseline. The CRs to date have been durable. Of the 20 patients who achieved a CR, 13 patients have reached three-month follow-up and all remain in CR. Four of these 13 patients have reached six-month follow-up and one of the 13 patients has reached nine-month follow-up. All remain in CR.

UGN-101 appeared to be well-tolerated with most treatment-emergent adverse events characterized as mild or moderate and transient. These included urinary tract infection, flank pain, ureteral narrowing and hydronephrosis and time-limited creatinine elevation.

"These interim data from the OLYMPUS trial are promising and demonstrate the potential of UGN-101 to become the first drug ever approved for low-grade UTUC. We are pleased to see that the results to date from this trial improve upon the 44 percent complete response rate on an intent to treat basis observed in our similarly designed Compassionate Use program of UGN-101," said Mark Schoenberg, M.D., Chief Medical Officer of UroGen. "Currently, the thousands of patients living with UTUC do not have many therapeutic options to manage their disease. With UGN-101, we hope to change the treatment paradigm for low-grade UTUC by potentially enabling the treatment of these tumors by non-surgical means, sparing patients from the risks and complications associated with repetitive surgical procedures and potential kidney removal."

"We are excited about the interim analysis from our pivotal Phase 3 trial of UGN-101 presented today. This is an important step toward our anticipated Q1 2019 submission of a New Drug Application to the U.S. Food and Drug Administration," said Ron Bentsur, Chief Executive Officer of UroGen. "This interim analysis also demonstrates the potential of our proprietary RTGel™ sustained release platform, which we believe has the potential to transform existing treatment paradigms across a number of urologic conditions. We look forward to continuing to advance our pipeline towards this end."

The OLYMPUS trial continues to enroll patients, and top-line results are expected in the second half of 2018.

Details of AUA Oral Presentation
Abstract #: LBA-25
Session: Plenary, Next Frontier
Title: Non-Surgical Management of Low-Grade Upper Tract Urothelial Cancer: An Interim Analysis of the International Multicenter OLYMPUS Trial
Presenter: Seth Paul Lerner, M.D., FACS, Professor of Urology, Baylor College of Medicine
Time: Monday, May 21, 2018, 10:40-10:50 AM PDT
Location: MCC NORTH, Hall E, The Moscone Center

About the Phase 3 OLYMPUS Trial
OLYMPUS (Optimized DeLivery of Mitomycin for Primary UTUC Study) is a pivotal, open-label, single-arm Phase 3 clinical trial of UGN-101 (MitoGel™) to evaluate the safety, tolerability and tumor ablative effect of UGN-101 in patients with low-grade UTUC. The trial, designed to be a single pivotal study for the approval of UGN-101 in low-grade UTUC, is anticipated to enroll approximately 74 patients at clinical sites across the United States and Israel. Study participants are treated with six weekly instillations of UGN-101 administered via a standard catheter. Four to six weeks following the last instillation, patients undergo primary disease evaluation (PDE) to determine response, the primary endpoint of the study. PDE involves a ureteroscopy and wash cytology, a standard microscopic test of cells obtained from the urine to detect cancer. Patients who achieve a complete response at the PDE timepoint will be followed for up to 12 months to determine the durability of disease control with UGN-101.

About Upper Tract Urothelial Cancer (UTUC)
Upper tract urothelial cancer (UTUC) is a malignancy involving the lining of the ureters, renal pelvis and kidneys. In the United States, there are approximately 7,500 new cases of UTUC each year. Of these, about 2,500 cases are low-grade UTUC. Approximately 14,500 people are currently living with low-grade UTUC.

The current standard of care for UTUC is complete or partial surgical removal of the involved kidney or ureter. For patients with a bilateral disease, an anatomic or functionally solitary kidney, medical comorbidities or low-grade disease who present with a limited number of tumors, a kidney-conserving alternative is considered, if possible. However, due to the specific anatomy and physiology of the upper urinary tract and renal pelvis, organ-sparing endoscopic resection and instillation of neoadjuvant or adjuvant chemotherapy is often challenging, leading to high rates of recurrence. Additionally, continuous urine flow, the inability of the upper urinary tract to retain a liquid volume under normal circumstances, and the effects of peristalsis, or muscle contraction, result in short exposure time of active agents in the target area. This leads to poor efficacy and limited use of standard therapeutic agents in the treatment of UTUC.

About UGN-101 (MitoGel™)
UGN-101 is an investigational drug formulation of mitomycin in Phase 3 development for the treatment of low-grade UTUC. Utilizing the RTGel™ technology platform, UroGen's proprietary sustained release, hydrogel-based formulation, UGN-101 is designed to enable longer exposure of mitomycin C to the urinary tract tissue, thereby potentially enabling the treatment of tumors by non-surgical means. UGN-101 is delivered to patients using standard intravesical catheters.

The U.S. Food and Drug Administration has granted both Orphan Drug and Fast Track designations to UGN-101 for the treatment of low-grade UTUC.

About UroGen Pharma Ltd.
UroGen Pharma Ltd. (Nasdaq:URGN) is a clinical-stage biopharmaceutical company developing advanced non-surgical treatments to address unmet needs in the field of urology, with a focus on uro-oncology. UroGen has developed RTGel™, 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 lead product candidates, UGN-101 (MitoGel™, also known as mitomycin urothelial gel) and UGN-102 (VesiGel™, also known as mitomycin intravesical gel), are designed to potentially remove tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade upper tract urothelial carcinoma and bladder cancer, respectively. UroGen is headquartered in Ra'anana, Israel with U.S. headquarters in New York.

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 with respect to the timing and results of clinical development and commercial prospects of the product candidates in UroGen's pipeline, including UGN-101 (MitoGel™) and UGN-102 (VesiGel™), the scope and development of UroGen's product candidate pipeline, enrollment in the OLYMPUS trial, timing and expectations of results from the OLYMPUS trial, UroGen's expectations regarding its ability to fund its operations, and the ability of UroGen to become a leader in the field of uro-oncology, particularly in the treatment of low-grade UTUC, which 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 complications thereof, including with respect to enrollment; the timing of and the ability to obtain and maintain regulatory approval; interim data from the OLYMPUS trial not being indicative of future results; the labeling for any approved product; the scope, progress and expansion of developing and commercializing UroGen's product candidates; and the size and growth of the market(s) therefor and the rate and degree of market acceptance thereof vis-à-vis alternative therapies. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of our annual report for the year ended December 31, 2017 filed with the SEC on March 15, 2018 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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