UroGen Reports Positive Data from Two Important Studies: UGN-101 OLYMPUS Pivotal Trial in LG UTUC and UGN-102 Phase 2b OPTIMA II Trial in LG Bladder Cancer

September 24, 2019

UGN-101:

Consistent Complete Response (CR) Rate of 59 Percent in Patients with Low-Grade Upper Tract Urothelial Cancer (LG UTUC)

Durability of Response Determined to be 89 Percent at Six Months and 84 Percent at 12 Months

Rolling NDA Submission on Track for Q4 2019 with Planned Launch in 1H 2020

UGN-102:

Data Demonstrates CR Rate of 63 Percent in Patients with Intermediate Risk Low-Grade Non-Muscle Invasive Bladder Cancer (LG NMIBC)

Company Completes Enrollment Ahead of Schedule in OPTIMA II Phase 2b Trial

NEW YORK--(BUSINESS WIRE)--Sep. 24, 2019-- UroGen Pharma Ltd. (Nasdaq: URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in the field of uro-oncology, today announced updated findings from the UGN-101 Phase 3 OLYMPUS Trial in patients with low-grade upper tract urothelial cancer (LG UTUC), as well as initial CR data from the UGN-102 Phase 2b OPTIMA II Trial in patients with intermediate risk low-grade non-muscle invasive bladder cancer (LG NMIBC).

Results from a final analysis of the primary endpoint for pivotal Phase 3 OLYMPUS showed that investigational UGN-101 (mitomycin gel) for instillation demonstrated a 59 percent CR rate in patients with LG UTUC. Findings were consistent with previously presented results.

The final analysis of the primary endpoint showed that in the OLYMPUS intent-to-treat population, 42 of the 71 patients (59 percent) achieved a CR. Forty-one patients entered follow-up, which is still ongoing. Durability of response was determined by Kaplan-Meier to be 89 percent at 6 months and 84 percent at 12 months after primary disease evaluation (PDE). The estimated median time-to-recurrence was 13.0 months. Thirty four of the 71 patients treated in the study were initially characterized by the treating physician as having endoscopically unresectable tumor at baseline. Twenty of 34 patients (59 percent) achieved a CR at the PDE assessment and 12-month durability was identical for this subgroup.

In OLYMPUS, the most common treatment emergent adverse events (TEAE) included ureteral stenosis, urinary tract infection, hematuria, flank pain, dysuria, renal impairment, hydronephrosis and frequency. Most TEAEs were characterized as mild to moderate and transient. Sixty-seven percent (48/71) of patients in the trial experienced an adverse event involving the renal/urinary tract. Of these, 23 percent (11/48) did not require surgical intervention, 50 percent (24/48) required temporary ureteral stent placement, 23 percent (11/48) required a long-term ureteral stent and 4 percent (2/48) required nephroureterectomy. At the time of database lock, the most common Grade 3 TEAE's included ureteral stenosis (8.5 percent), hematuria, flank pain, and urinary tract infection (3 percent each). There was one Grade 4 TEAE of subdural hematoma (1.4 percent).

“We are pleased that the six-month durability from this analysis remains consistent with previously presented results and are very pleased with the durability observed at 12 months in evaluated patients. These findings provide further support for the concept of chemoablation with UGN-101 as an initial kidney-sparing treatment option for patients with LG UTUC,” said Liz Barrett, President and Chief Executive Officer of UroGen. “We are on track to complete our New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in Q4 2019 and will be prepared for anticipated approval and launch in 1H 2020 of the first drug for the non-surgical treatment of LG UTUC.”

The Company also presented interim results from the Phase 2b OPTIMA II trial of investigational UGN-102 (mitomycin gel) for intravesical instillation for patients with intermediate risk LG NMIBC, defined as those with one or two of the following criteria: multifocal disease, large tumors and rapid rates of recurrence. The single-arm, open label study trial recently completed enrollment of 62 patients at clinical sites across the U.S. and Israel. Patients are treated with six weekly instillations of UGN-102 and undergo assessment of CR (the primary endpoint) four to six weeks following the last instillation. In an interim cohort of 32 patients, 63 percent (20/32) achieved a CR.

	Response Rate
	Overall (n=32)
CR at PDE	63% (20/32)

“Achieving our enrollment goal ahead of schedule is a testament to the enthusiasm and need for this type of innovative approach to treatment in LG NMIBC. ‘Intermediate risk’ patients experience what can be viewed as a form of surgical failure, and many undergo multiple surgical procedures, known as transurethral resection of bladder tumor (TURBT), to manage these recurrences. We are encouraged by the data observed in this tough-to-treat population for whom the standard of care is really not effective,” said Mark Schoenberg, MD, Chief Medical Officer of UroGen. “While OPTIMA II remains ongoing and a Phase 3 study is anticipated, the results presented today further support our belief that UGN-102 has the potential to be an effective treatment option for this patient population of approximately 80,000, as there are no other options for these patients aside from repetitive surgical intervention. Based on literature, these patients have a high likelihood of recurrence at one year due to the chronicity of this disease, so we will continue to follow them and assess durability at 12 months.”

In the interim data from OPTIMA II, the most common adverse events observed were dysuria, pollakiuria, fatigue, hematuria and urinary tract infection. The majority of these treatment-emergent adverse events were characterized as mild or moderate and transient.

The Company intends to initiate a pivotal Phase 3 trial in 2020 following discussion with the FDA.

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 (mitomycin gel) for instillation to evaluate the safety, tolerability and tumor ablative effect of UGN-101 in patients with low-grade UTUC. The trial enrolled 71 patients at clinical sites across the United States and Israel. Study participants were treated with six weekly instillations of UGN-101 administered via a standard catheter. Four to six weeks following the last instillation, patients underwent a PDE to determine response, the primary endpoint of the study. PDE involved a ureteroscopy and wash cytology, a standard microscopic test of cells obtained from the urine to detect cancer. Patients who achieved a CR at the PDE timepoint were then followed for up to 12 months to determine the durability of disease control with UGN-101.

About UGN-101

UGN-101 (mitomycin gel) for instillation is an investigational drug formulation of mitomycin in Phase 3 development for the treatment of low-grade upper tract urothelial cancer (LG UTUC). Utilizing the RTGel™ technology platform, UroGen’s proprietary sustained release, hydrogel-based formulation, UGN-101 is designed to enable longer exposure of urinary tract tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means. UGN-101 is delivered to patients using standard ureteral catheters. The Company initiated its rolling submission of the UGN-101 New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA) in December 2018. The FDA previously granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to UGN-101 for the treatment of UTUC. If approved, UGN-101 would be the first drug approved for the non-surgical treatment of LG UTUC.

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 gel) for intravesical instillation to evaluate the safety and efficacy of UGN-102 in patients with intermediate risk low-grade non-muscle invasive bladder cancer (LG NMIBC) at intermediate risk of recurrence. Intermediate risk of progression 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). The trial enrolled 62 patients at clinical sites across the United States and Israel. Study participants were treated with six weekly instillations of UGN-102 administered via a standard intravesical catheter. Four to six weeks following the last instillation, patients undergo a PDE to determine response, the primary endpoint of the study. PDE involves a cystoscopy and wash cytology, a standard microscopic test of cells obtained from the urine to detect cancer. Patients who achieve a CR at the PDE timepoint are then followed for up to 9 months to determine the durability of disease control with UGN-102.

About UGN-102

UGN-102 (mitomycin gel) for instillation is an investigational drug formulation of mitomycin in Phase 2b development for the treatment of low-grade non-muscle invasive bladder cancer (LG NMIBC). 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 standard intravesical catheters. The Company completed enrollment in the Phase 2b OPTIMA II trial of UGN-102 for the treatment of LG NMIBC 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 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™ 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 lead investigational candidates, UGN-101 (mitomycin gel) for instillation, and UGN-102 (mitomycin gel) for intravesical instillation, 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 New York, NY with operations in Los Angeles, CA and Israel.

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 UGN-101 for LG UTUC; the timing for completion of the rolling NDA for UGN-101; the potential approval of UGN-101 and the timing thereof; the expectation that UGN-101, if approved, will be the first drug approved for the non-surgical treatment of LG UTUC; the expected readiness of UroGen for a potential commercial launch of UGN-101 in 1H 2020; the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs; the opportunity and potential of UGN-102 for LG NMIBC; and the planned initiation of a Phase 3 pivotal study of UGN-102 in LG NMIBC. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of clinical trials, including the OLYMPUS Phase 3 trial and the OPTIMA II Phase 2b trial 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 achieving commercial readiness for the launch of a new product; 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; and UroGen's ability to attract or retain key management, members of the board of directors and personnel. 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 9, 2019, 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/20190924005735/en/

Source: UroGen Pharma Ltd.

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