UroGen Investor Day Details Positive Clinical Updates, UGN-101 Launch Preparedness, and Pipeline Advances

September 24, 2019

UGN-101 and UGN-102 Demonstrate Positive Clinical Data in Low-Grade Upper Tract Urothelial Cancer (LG UTUC) and Low-Grade Non-Muscle Invasive Bladder Cancer (LG NMIBC)

UGN-101 Final Data Modules for NDA Submission On-Track for Q4 2019

UGN-101 Plans for Launch Readiness by January 2020

UGN-201 Plans for Advancement into Phase 1 for High-Grade NMIBC in 2020

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 urology, today presented updates on its advancing pipeline for urologic cancers and UGN-101 launch readiness at its Investor Day in New York on September 24, 2019.

“At UroGen, we are 100 percent committed to overcoming obstacles that have stunted much needed innovation in uro-oncology,” said Liz Barrett, President and Chief Executive Officer of UroGen. “While we prepare for a potential launch of UGN-101, we believe this is just the beginning of what is possible with our pipeline. The data from an interim analysis for UGN-102 unveiled at our Investor Day further highlight the potential of our RTGel™ platform to transform treatments in this space for an even larger patient population with bladder cancer. We look forward to pushing innovation beyond our foundational platform and moving UGN-201, our TLR 7/8 agonist for high-grade bladder cancer, into clinical studies in 2020.”

UroGen detailed data updates for its lead investigational product candidates UGN-101 and UGN-102:

- **UGN-101** (mitomycin gel) for instillation for patients with low-grade upper tract urothelial cancer (LG UTUC):
  - Complete response (CR) rate of 59 percent observed in 71 patients with LG UTUC from Phase 3 OLYMPUS trial. Data remain consistent with previously presented data.
  - Durability of response determined to be 89 percent at six months and 84 percent at twelve months.
  - In the OLYMPUS trial, 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.
  - At the time of database lock, the most common Grade 3 TEAE’s included ureteral stenosis (8.5%), hematuria, flank pain, and urinary tract infection (3% each). There was one Grade 4 TEAE of subdural hematoma (1.4%).
  - Rolling NDA submission is on track for Q4 2019 with potential approval and launch in 1H 2020.
  - If approved, UGN-101 will be the first drug for the primary chemoablative treatment of LG UTUC.

- **UGN-102** (mitomycin gel) for intravesical instillation for patients with intermediate risk low-grade non-muscle invasive bladder cancer (LG NMIBC):
  - In an interim analysis, 63% (20/32) of patients from the Phase 2b OPTIMA II trial achieved a CR.
  - In an interim analysis, the most common treatment emergent adverse events (TEAEs) observed were dysuria, pollakiuria, fatigue, hematuria and urinary tract infection. The majority were characterized as mild or moderate and transient.
  - Patients will continue to be followed with 12-month durability to be reported at a later date.

- Trial enrolled 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.
- Trial completed enrollment of 62 patients ahead of schedule.
- The Company intends to initiate a pivotal Phase 3 trial in 2020 following discussion with the FDA.

UroGen discussed its ongoing activities to build awareness of unmet needs in UTUC, educate the market and commercialize UGN-101 following anticipated regulatory approval:

- Increased scientific awareness of UGN-101 clinical data developments in urologic community.
- Engagement with payers and proactive market access strategy to ensure patient access and reimbursement.
- UGN-101 treatment expected to fit well into existing physician reimbursement models.
- Planned convenience kit for UGN-101 will facilitate preparation and administration for practitioners.
- Experienced commercial team with track record of success in uro-oncology.
- Nimble salesforce with seven Regional Business Managers (RBMs) hired, and 50 sales reps to be hired by end of 2019.

The Company also provided an update on **UGN-201**, it's investigational TLR 7/8 agonist for the treatment of high-grade NMIBC. UGN-201 is believed to stimulate innate and adaptive antitumor immunity. It likely works in conjunction with other potent immunoregulatory molecules. Nonclinical data shows an efficacy signal when UGN-201 is administered locally with anti-CTLA4 antibody in a murine model of high-grade bladder cancer. The Company plans to optimize combinations and advance into human studies.

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 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 potentially 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 timing for completion of pre-commercial activities and infrastructure build-out in anticipation of a potential commercial launch of UGN-101; the expected readiness of UroGen for a potential commercial launch of UGN-101 in 1H 2020 and the strength and timing of the potential commercial launch of

UGN-101; plans for the retention of field-based personnel in support of the launch of UGN-101; 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; plans to commence a pivotal trial for UGN-102 in LG NMIBC in 2020; UGN-102's potential to replace current standard of care in LG NMIBC; and plans to initiate a Phase 1 study with UGN-201. 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 and packaging 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 presentation.

View source version on businesswire.com: https://www.businesswire.com/news/home/20190924005941/en/

Source: UroGen Pharma Ltd.

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