UroGen Delivers Updated Complete Response (CR) and Durability Data from the UGN-101 Phase 3 OLYMPUS Trial

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Complete Response (CR) Rate Consistent at 59 Percent

Six-month Durability Strong with 89 Percent of Evaluable Patients Remaining in CR

Full Phase 3 Data Anticipated for 2H 2019

NEW YORK--(BUSINESS WIRE)--May 5, 2019-- UroGen Pharma Ltd. (Nasdaq: URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in the field of urology, today announced findings from a secondary analysis from the pivotal Phase 3 OLYMPUS trial which showed that UGN-101 (mitomycin gel) for instillation, an investigational mitomycin formulation, demonstrated a 59 percent complete response rate in a subset of patients with endoscopically unresectable low-grade upper tract urothelial cancer (UTUC). Findings were presented by Seth Paul Lerner, M.D., FACS, Professor of Urology at Baylor College of Medicine, in an oral presentation during the plenary session at the 114thAmerican Urological Association (AUA) Annual Meeting in Chicago.

The analysis showed that in the OLYMPUS intent-to-treat population, 71 patients had undergone PDE at the time of the analysis and 42 of the 71 patients (59 percent) achieved a CR. Forty-one patients entered follow-up. Of the evaluated complete responses to date, 27 patients have undergone a six-month evaluation, and 24 out of 27 patients (89 percent) have remained disease free at six months. Overall, 5 of 41 patients who achieved a CR have relapsed at any time during the study.

Of these 71 patients, 34 were initially characterized by the treating physician as having endoscopically unresectable tumor at baseline, and 20 of 34 of these patients (59 percent) achieved a CR at the PDE.

This data is summarized in the table below.

	Response Rate	
	Overall (n=71)	Endoscopically Unresectable Tumors 48% (34/71)
CR at PDE	59% (42/71)	59% (20/34)
6 Month CR Durability*	89% (24/27)	85% (17/20)

**Forty-one patients entered follow-up. At the time of the analysis, 66 percent (27/41) of patients have completed a six-month evaluation.*

The most common adverse events observed were urinary tract infection, ureteral narrowing and stricture formation. The majority of ureteral events were reported as mild to moderate and have resolved.

“The results from the OLYMPUS trial continue to be compelling for new and recurrent LG UTUC, as well as for those who have unresectable tumors and would be immediate candidates for kidney removal. For this typically elderly patient population, kidney preservation is paramount, and these findings provide evidence-based support for the concept of chemoablation with UGN-101 as an initial kidney-sparing treatment option for low-grade UTUC,” said Mark P. Schoenberg, MD, Chief Medical Officer at UroGen. “The analysis also advances our understanding of durability of response, which we are pleased to see has remained consistent as the number of patients who reach the six-month follow-up timepoint increases.”

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 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 primary disease evaluation (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 complete response 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 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 plans to conduct an early stage feasibility evaluation, the potential of UGN-101 for LG UTUC, the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs, and the potential of UGN-102 for LG NMIBC, which 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 pivotal Phase 3 trial and potential safety and other complications thereof; the ability to obtain and maintain regulatory approval; the labeling for any approved product; the scope, progress and expansion of developing and commercializing UroGen Pharma'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 Pharma'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 Pharma's Form 10-K filed with the SEC on February 28, 2019 and other filings that UroGen Pharma 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 Pharma'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 Pharma as of the date of this release.

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