# UroGen Pharma Announces FDA Filing Acceptance and Priority Review of U.S. New Drug Application (NDA) for UGN-101

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*Potential for UGN 101 to be First Non-Surgical Therapy for the Treatment of Low-Grade Upper Tract Urothelial Cancer (LG UTUC)*

NEW YORK--(BUSINESS WIRE)--Dec. 19, 2019-- UroGen Pharma Ltd. (Nasdaq: URGN) today announced the U.S. Food and Drug Administration (FDA) accepted for filing and granted priority review for its New Drug Application (NDA) for UGN-101 (mitomycin gel) for instillation as a potential treatment for patients with low-grade upper tract urothelial cancer (LG UTUC). If approved, UGN-101 would be the first non-surgical treatment option for LG UTUC.

“The FDA filing acceptance and granting of priority review for UGN-101 is an important milestone in our mission to pioneer new treatments to improve patient care in specialty cancers and urologic diseases,” said Liz Barrett, President and Chief Executive Officer of UroGen. “There is a significant unmet need for a better treatment option for patients with LG UTUC, as the current standard of care involves surgical removal of the kidney or repetitive endoscopic tumor removal.”

The FDA grants priority review to applications for medicines that, if approved, would be significant improvements in the safety or effectiveness of the treatment, diagnosis, or prevention of serious conditions when compared to standard applications. Priority review designation shortens the review period from the standard 10 months to six months from the submission of the NDA. The FDA assigned a Prescription Drug User Fee Act (PDUFA) action date of April 18, 2020.

The company is on track for the potential launch of UGN-101 by mid-year 2020. The FDA previously granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to UGN-101 for the treatment of LG UTUC.

The NDA is supported by the positive results from the pivotal Phase 3 OLYMPUS clinical trial. Results from a final analysis of the primary endpoint showed that UGN-101 demonstrated a complete response rate of 59 percent in patients with LG UTUC. In addition, the durability of response was estimated as 89 percent at six months and 84 percent at 12 months by Kaplan Meier analysis. Median time to recurrence was estimated to be 13 months. The most commonly reported treatment emergent adverse events were ureteric stenosis (43.7%), urinary tract infection (32.4%), haematuria (31.0%), flank pain (29.6%) nausea (23.9%), dysuria (21.1%), renal impairment (19.7%) and vomiting (19.7%). The majority of these adverse events were mild to moderate, with 8.5% of patients having events of ureteric stenosis reported as severe.

**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 74 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 complete response (CR), 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 response with UGN-101.

**About UGN-101**

UGN-101 (mitomycin gel) for instillation is an investigational drug formulation of mitomycin 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 treatment of LG UTUC.

**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 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 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 potential for UGN-101 to be approved as a treatment option for LG UTUC; 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 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 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 November 12, 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.

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