UGN-102, in Development as the Potential First Non-Surgical Therapy for LG-IR-NMIBC, Met Primary Endpoints in Both Phase 3 ATLAS and ENVISION Clinical Trials

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-- In ATLAS, UGN-102 Demonstrated Superiority to TURBT with a 55% Reduction of Risk for Recurrence, Progression or Death in Patients who Received UGN-102 --

-- ENVISION Showed a Complete Response Rate of 79.2% at 3-Months --

-- Side Effect Profile in ATLAS and ENVISION Consistent with Previous Clinical Trials of UGN-102 --

-- UroGen Will Review ATLAS and ENVISION Top-line Data Today at 10:00 a.m. Eastern Time --

PRINCETON, N.J.--(BUSINESS WIRE)--Jul. 27, 2023-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing novel solutions that treat urothelial and specialty cancers, today announced positive topline data from its Phase 3 trials ATLAS and ENVISION studying UGN-102 (mitomycin) for intravesical solution in patients with low-grade, intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). Both ATLAS and ENVISION trials met their primary endpoints.

In the ATLAS trial, UGN-102 met its primary endpoint of disease-free survival, reducing risk of recurrence, progression, or death by 55%. UGN-102 also showed a 64.8% complete response rate at three months for patients who only received UGN-102, compared to a 63.6% complete response rate at three months for patients who only received a TURBT.

The ENVISION trial met its primary endpoint by demonstrating that patients treated with UGN-102 had a 79.2% rate of complete response at 3-months following the initial treatment. Additional data evaluating the secondary endpoint of duration of response from ENVISION, and the submission of a New Drug Application (NDA) (assuming additional positive findings) to the U.S. Food and Drug Administration (FDA) are anticipated in 2024.

“UGN-102 has demonstrated a robust and consistent therapeutic profile across multiple clinical trials, providing a compelling picture of its potential to be a transformational product and advance the standard of care away from repetitive surgery to a minimally invasive, non-surgical option for LG-IR-NMIBC,” says Liz Barrett, President and Chief Executive Officer of UroGen. “If approved, we anticipate UGN-102 to be a significant growth driver for UroGen as the first-ever non-surgical treatment option for a disease afflicting approximately 82,000 new patients in the U.S. each year. We are on track to deliver on our previously shared guidance for JELMYTO and now find ourselves on the precipice of a new era in bladder cancer care, and that is a very exciting place to be.”

In both trials, UGN-102, a sustained release, hydrogel-based formulation that is designed to enable longer exposure of bladder tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means, was generally well tolerated, with a side effect profile similar to that of previous clinical trials.

“While TURBT is the standard treatment for bladder cancer, the recurrent nature of LG-IR-NMIBC means that patients will undergo multiple surgeries that come with risks for this older patient population,” says Sandip Prasad, MD, M.Phil., Director of Genitourinary Surgical Oncology, Morristown Medical Center/Atlantic Health System, NJ. “Based on these compelling data, I am optimistic that UGN-102, if approved, may change the treatment paradigm for these patients who lack non-surgical options to manage the ongoing burden of this highly recurrent disease.”

The Phase 3 ATLAS clinical trial, the predecessor to ENVISION, evaluated the efficacy, durability, and safety of UGN-102 with or without TURBT vs. TURBT alone in 282 patients with LG-IR-NMIBC. ENVISION evaluated the efficacy and safety of UGN-102 as a primary chemoablative therapy in 240 patients with LG-IR-NMIBC.

UGN-102 ATLAS & ENVISION Top-line Data Review

The company is hosting a data event featuring a panel discussion with leading bladder cancer experts on Thursday, July 27, 2023 at 10:00 a.m. Eastern Time to discuss these findings and will highlight topline results from the Phase 3 ATLAS and ENVISION clinical trials.

Please register for the webinar under the Events & Presentations section of the Company’s Investor Relations site (https://investors.urogen.com/events-and-presentations).

Following the live webcast, a replay will be available on the Company's website (https://urogen.com).

About UGN-102

UGN-102 (mitomycin) for intravesical solution is an investigational drug formulation of mitomycin in Phase 3 development for the treatment of LG-IR-NMIBC. Utilizing UroGen’s proprietary RTGel ® technology, a 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 a standard urinary catheter in an outpatient setting. Assuming positive findings from the ENVISION Phase 3 study, UroGen anticipates submitting a New Drug Application (NDA) for UGN-102 in 2024. If approved, UGN-102 would be the first non-surgical primary therapeutic to treat a subset of bladder cancer characterized by high recurrence rates and multiple surgeries.

About ENVISION

The Phase 3 ENVISION trial is a single-arm, multinational, multicenter study evaluating the efficacy and safety of UGN-102 (mitomycin) for intravesical solution as primary chemoablative therapy in patients with low-grade, intermediate-risk NMIBC. The Phase 3 ENVISION trial completed target enrollment with approximately 240 patients across 56 sites. Study participants received six once-weekly intravesical instillations of UGN-102. The primary endpoint evaluated the complete response rate at the 3-month assessment after the first instillation, and the key secondary endpoint will

evaluate durability over time in patients who achieved a complete response at the three-month assessment. Based on discussions with the FDA, and assuming positive findings, UroGen anticipates submitting an NDA for UGN-102 in 2024. Learn more about the Phase 3 ENVISION trial at www.clinicaltrials.gov (NCT05243550)

About ATLAS

ATLAS is a global, open-label, randomized controlled Phase 3 trial designed to assess the efficacy and safety of UGN-102, with or without TURBT, vs. TURBT alone in patients diagnosed with LG-IR-NMIBC. The trial enrolled 282 patients in clinical sites in the U.S., Europe and Israel. Patients were randomized 1:1 to either UGN-102 or TURBT. Patients in the UGN-102 arm were treated with six weekly intravesical instillations of UGN-102. At the 3-month time point, patients were assessed for response. Patients who demonstrated a complete response to either UGN-102 or TURBT, were assessed for long-term follow-up for evidence of recurrence. Patients who demonstrated presence of persistent disease at 3-months, in either arm, underwent a TURBT and continued for long-term follow-up for evidence of recurrence. The primary endpoint of the study is disease-free survival. Learn more about the ATLAS trial at www.clinicaltrials.gov (NCT04688931)

About UroGen Pharma Ltd.

UroGen is a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers 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. JELMYTO® (mitomycin) for pyelocalyceal solution and investigational treatment UGN-102 (mitomycin) for intravesical solution for patients with low-grade non-muscle invasive bladder cancer are designed to ablate tumors by non-surgical means. UroGen is headquartered in Princeton, NJ 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, statements regarding: plans with respect to a regulatory submission for UGN-102 and the timing thereof; the potential for UGN-102 to be the first non-surgical primary therapeutic to treat a subset of bladder cancer characterized by high recurrence rates and multiple surgeries; the potential for UGN-102 to be transformational and advance the standard of care for LG-IR-NMIBC; the anticipation that UGN-102, if approved, would be a significant growth driver for UroGen; the expected timing for additional data evaluating the secondary endpoint of duration of response from ENVISION; the anticipated submission of an NDA for UGN-102 in 2024; the estimated addressable patient population; and the potential of UroGen's proprietary RTGel technology to improve therapeutic profiles of existing drugs and UroGen's sustained release technology making local delivery potentially more effective as compared to other treatment options. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: preliminary results may not be indicative of results that may be observed in the future; the timing and success of clinical trials and potential safety and other complications thereof; unforeseen delays that may impact the timing of progressing clinical trials and reporting data; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to maintain regulatory approval; complications associated with commercialization activities; the labeling for any approved product; competition in UroGen's industry; 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; UroGen's ability to attract or retain key management, members of the board of directors and personnel; UroGen's RTGel technology may not perform as expected; UroGen may not successfully develop and receive regulatory approval of any other product that incorporates RTGel technology; UroGen's financial condition and need for additional capital in the future. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of UroGen's Quarterly Report on Form 10-Q for the quarter ended March 31, 2023 filed with the SEC in May 11, 2023 (which is 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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