UroGen Pharma Unveils New Phase 3 Development Plan for UGN-102 at Spotlight Event

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New, Single-arm Phase 3 Study Expected to Initiate in Early 2022

PRINCETON, N.J.--(BUSINESS WIRE)--Nov. 10, 2021-- UroGen Pharma Ltd. (Nasdaq: URGN), a biopharmaceutical company dedicated to building and commercializing novel solutions that treat urothelial and specialty cancers, is announcing at its virtual Spotlight Event being held today that, following recent discussions with the U.S. Food and Drug Administration ("FDA"), it plans to conduct a new, single-arm Phase 3 pivotal study of UGN-102 for the treatment of low-grade, intermediate-risk, non-muscle invasive bladder cancer ("NMIBC"). This new study, which is expected to initiate in early 2022, is expected to enroll approximately 220 patients across 90 sites.

"We have worked diligently with the FDA over the past several years to define the unmet need in low-grade NMIBC, with a particular focus on the intermediate risk population that typically experiences multiple recurrences," said Liz Barrett, President and Chief Executive Officer of UroGen. "We are preparing to initiate a single-arm pivotal study of UGN-102 to form the basis for a New Drug Application for UGN-102 in the treatment of low-grade, intermediate-risk NMIBC. We believe this new study increases the probability of regulatory success for UGN-102 given its streamlined design in addition to the encouraging results observed from our Phase 2 OPTIMA II study."

UroGen is grateful to the investigators and patients who are participating in the ATLAS study and believes the data generated will represent an important component of the planned UGN-102 NDA submission, which remains on track for 2024. In light of the new planned Phase 3 trial, UroGen will stop enrollment in the ATLAS trial. Patients already enrolled in ATLAS will have the option to remain in the study until completion.

In addition to the updated clinical plan for UGN-102, the Spotlight Event features presentations on UroGen's earlier-stage, locally administered immunotherapy candidates, UGN-301 and UGN-201, as well as two expert panel discussions on future directions in treating NMIBC, other types of bladder cancer and the potential benefits of UroGen's immunotherapy pipeline products in non-urologic cancers.

Distinguished panelists discussing the current treatment landscape and potential future innovations in NMIBC include:

- Dr. Sandip Prasad, a urologist in community practice with Atlantic Medical Group and the Morristown Medical Center;
- Dr. Gary Steinberg, Professor of Urology and Director of the Bladder Cancer Program at NYU Langone Medical Center; and
- Dr. William Huang, Professor of Urology and Radiology at NYU and Chief of Urology at Tisch Hospital.

The experts reviewing the unmet needs of patients with high-grade NMIBC and the potential for locally applied combination therapy include:

- Dr. Karim Chamie, Associate Professor of Urology at UCLA Medical Center; and
- Dr. Joshua Meeks, Associate Professor of Urology, Biochemistry and Molecular Genetics at Northwestern University Feinberg School of Medicine.

"Immunotherapy for the treatment of high-grade bladder cancer, with primary attention toward our TLR-7 agonist, UGN-201 and our anti-CTLA4 antibody, UGN-301, is a key area of focus of our earlier-stage pipeline," said Dr. Mark Schoenberg, Chief Medical Officer of UroGen. "We have pursued a series of pre-clinical studies to determine whether our RTGel™ platform might provide a method for delivering highly potent immunomodulators directly to the bladder surface, avoiding the toxicity associated with systemic administration. Our studies conducted to-date suggest bladder cancer treated with a combination of a TLR-7 agonist and an anti-CTLA4 antibody using our RTGel technology, produces improved survival relative to treatment with other checkpoint inhibitors in RTGel, either alone or in combination with UGN-201."

UroGen is currently conducting non-human primate toxicity studies to facilitate the initiation of a multi-arm Phase 1 study of UGN-301 in early 2022 to be followed by UGN-301 in combination with other agents. This approach leverages UroGen's unique platform for drug delivery and provides an opportunity to evaluate intravesical delivery of its anti-CTLA4 monoclonal antibody in combination with other immuno-modulators, chemotherapies, gene therapy and innate immune stimulators, including UGN-201.

About UroGen Pharma Ltd.

UroGen is a biopharmaceutical company dedicated to building novel 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. UroGen's first commercial product, 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 statements regarding the new single-arm Phase 3 study for UGN-102 and the timing thereof, the timing of the planned NDA for UGN-102, and the planned Phase 1 studies of UGN-301 and the timing thereof. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: clinical trial enrollment challenges that may impact the expected timing of our planned clinical trials, including challenges related to the ongoing COVID-19 pandemic; the timing and success of clinical trials and potential complications thereof; UroGen's ability to attract or retain key management, members of the board of directors and personnel; and any negative effects on UroGen's business, commercialization and product development plans caused by or associated with COVID-19. 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 4, 2021 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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