UroGen Submits Investigational New Drug Application Supporting Planned Phase 1 Clinical Study of UGN-301 (zalifrelimab) Intravesical Solution in Recurrent Non-Muscle Invasive Bladder Cancer

March 1, 2022

-- UGN-301 is an investigational immunotherapy designed for monotherapy and combination therapy in treating low-grade and high-grade disease --

PRINCETON, N.J.--(BUSINESS WIRE)--Mar. 1, 2022-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced the submission of an Investigational New Drug application (IND) for UGN-301 (zalifrelimab) to the U.S. Food and Drug Administration (FDA) in support of the planned initiation of a multi-arm, Phase I clinical study for its anti-CTLA4 antibody. The study is expected to begin in the second quarter of 2022 and will evaluate the safety and tolerability of UGN-301 as monotherapy and in combination with other immunomodulators and chemotherapies in recurrent non-muscle invasive bladder cancer (NMIBC). This clinical program builds on encouraging preclinical data showing that intravesical administration of anti-CTLA4 and a TLR agonist leveraging the RTGel™ platform can produce clinical benefit in the setting of high-grade bladder cancer. It will take approximately 12 months to complete the monotherapy arm of the study.

“UroGen has shown that combination immunotherapy, when delivered intravesically, is potentially synergistic. While CTLA-4 has long been considered a good target for overcoming the immune suppression produced by tumor cells, anti-CTLA4 antibodies are associated with toxicities when administered systemically,” said Mark Schoenberg, Chief Medical Officer, UroGen Pharma. “Local delivery using our RTGel™ proprietary technology may permit us to leverage the power of a potent antibody while avoiding the toxicity associated with intravenous administration. This is critical because anti-CTLA4 antibodies stimulate cytotoxic T cells while inhibiting suppressive T-regulatory cells, making this class a potentially more comprehensively acting immunomodulator than antibodies to PD-1 and PD-L1.”

In the nonclinical study of UGN-301 in cynomolgus monkeys, UroGen assessed the potential toxicity via instillation into the urinary bladder delivered with RTGel reverse thermal hydrogel. The results showed that instillation of UGN-301 at different dose concentrations for a pre-defined period was not associated with mortality, changes in body weights, organ weight differences or meaningful changes among other endpoints, or macroscopic or microscopic findings following a specified recovery period.

According to Dr. Schoenberg, “We view intravesical delivery of our anti-CTLA4 antibody UGN-301 as a cornerstone of the urologic cancers program that we are pursuing in collaboration with MD Anderson Cancer Center, and we are thrilled at the prospect of advancing this program in the form of a multi-arm, Phase 1 study of UGN-301 in combination with other agents. We believe that this approach is unique and leverages our proprietary drug delivery technology and provides an opportunity to evaluate a variety of novel immunomodulatory drug combinations.”

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. UroGen’s first commercial product and the investigational treatment UGN-102 (mitomycin) for intravesical solution in development 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: the planned Phase 1 studies of UGN-301 (zalifrelimab) and the design thereof; the potential benefits of the intravesical delivery of zalifrelimab using our RTGel™ proprietary technology; the development of UGN-301 and other investigational agents; and our opportunities to evaluate a variety of novel immunomodulatory drug combinations. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: there is no guarantee that our submitted IND for zalifrelimab will be cleared by the FDA, and without that clearance, we will be unable to advance zalifrelimab into a Phase 1 clinical trial; results from nonclinical studies are not necessarily indicative of results that may be observed in clinical trials; clinical trial site initiation and 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 safety and other 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 November 15, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20220301005474/en/

INVESTOR:
Vincent Perrone
Senior Director, Investor Relations
vincent.perrone@urogen.com
609-460-3588 ext. 1093

MEDIA:
Cindy Romano
Director, Corporate Communications
cindy.romano@urogen.com
609-460-3583 ext. 1083

Source: UroGen Pharma Ltd.