UroGen Pharma Announces Enrollment of First Patient in Phase 3 Clinical Trial of MitoGel™ for Treatment of Low-Grade Upper Tract Urothelial Carcinoma

April 3, 2017

RA'ANANA, Israel and NEW YORK, April 03, 2017 (GLOBE NEWSWIRE) — UroGen Pharma Ltd., a privately held, clinical-stage biopharmaceutical company that is developing advanced non-surgical treatments to address unmet needs in the field of uro-oncology, announced today that the first patient has been enrolled in the OLYMPUS trial, an open-label, single-arm pivotal Phase 3 clinical trial of MitoGel, a novel sustained release formulation of Mitomycin C, for the treatment of non-muscle invasive, low-grade upper tract urothelial carcinoma (UTUC).

The OLYMPUS trial design is based on preliminary evidence of the safety and efficacy of MitoGel from an investigator-initiated Compassionate Use program for the treatment of severe, non-resectable, low-grade UTUC. The OLYMPUS trial is being conducted in the United States and Europe with anticipated enrollment of approximately 70 patients. Dr. Seth Lerner, MD FACS, Professor of Urology at Baylor College of Medicine, is the Principal Investigator of this study.

UTUC represents an unmet medical need for which there are no FDA approved drugs. UroGen Pharma has obtained U.S. Orphan Drug Designation for MitoGel for the treatment of UTUC.

Ron Bentsur, Chief Executive Officer of UroGen Pharma, stated, “Enrollment of the first patient into our pivotal study of MitoGel for low-grade UTUC represents an important milestone for the Company. Today, the treatment options for UTUC are a complicated endoscopic surgical procedure or surgical removal of the involved kidney and upper tract. MitoGel, representing a potential non-surgical local treatment for low-grade UTUC patients, may become the first drug ever approved to treat for UTUC."

Arie Belldegrun, MD, UroGen Pharma’s Chairman, commented, “We are thrilled to have taken an important step towards advancing the development of a potential treatment for low-grade UTUC that may benefit the families and individuals living with this condition. The commencement of the MitoGel pivotal study today highlights our commitment to developing transformative therapies for people suffering from uro-oncological conditions.”

About the OLYMPUS Trial

OLYMPUS (**O**ptimized De**L**ivery of **M**itomycin for **P**rimary **U**TUC **S**tudy) is an open-label, single-arm Phase 3 clinical trial of MitoGel to evaluate the safety, tolerability and tumor ablative effect of MitoGel in low grade UTUC patients. The trial, designed to be a single pivotal study for the potential approval of MitoGel in low-grade UTUC, is anticipated to enroll approximately 70 patients in clinical sites in the U.S. and Europe. The trial will also evaluate the durability of the tumor ablative effect of MitoGel.

Learn more about the OLYMPUS trial at www.clinicaltrials.gov.

About MitoGel

Utilizing RTGel, UroGen’s proprietary sustained release, hydrogel-based formulation, MitoGel is designed to enable longer exposure of Mitomycin C to the urinary tract tissue, thereby potentially enabling the treatment of tumors by non-surgical means. MitoGel is administered to patients using standard intravesical catheters.

About UTUC

Non-muscle invasive upper tract urothelial carcinoma (UTUC) has an estimated annual incidence in the United States of up to 7,500 cases – about 5% to 10% of all new cases of urothelial cancer. There are approximately 2,500 new cases of low-grade UTUC in the U.S. with a prevalence of approximately 14,500. UTUC refers to cancer of the upper tract, which connects the bladder to the kidney, and the renal pelvis. The current standard of care for this cancer is complete or partial surgical removal of the involved kidney and upper tract. For patients with a bilateral disease, an anatomic or functionally solitary kidney, medical comorbidities or low-grade disease that present with a limited number of tumors, a kidney-conserving alternative is considered, if possible. However, due to the specific anatomy and physiology of the upper tract and renal pelvis, the performance of organ-sparing endoscopic resection and instillation of neoadjuvant or adjuvant chemotherapy are often challenging, leading to high rates of recurrence and risk for progression. Difficulties in administering and maintaining Mitomycin C or any other drug in the upper tract due to low residual duration and short exposure time of the active agent in the treated area results in low treatment efficacy and limited use of chemoablative agents in patients with UTUC. Furthermore, there are currently no FDA-approved drugs for the treatment of UTUC.

About UroGen Pharma Ltd.

UroGen Pharma is a clinical stage biopharmaceutical company developing advanced non-surgical, local treatments to address unmet needs in the field of urology, with a focus on uro-oncology. The Company has developed RTGel, a proprietary sustained release, hydrogel-based formulation for potentially improving the efficacy and safety profiles of existing drugs. UroGen Pharma’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 Pharma’s lead product candidates, MitoGel and VesiGel, are designed to potentially remove tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade UTUC and bladder cancer. Moreover, UroGen Pharma has recently completed a worldwide licensing agreement with Allergan Pharmaceuticals International Limited, a wholly owned subsidiary of Allergan plc, for the use of RTGel with neurotoxins for the treatment of overactive bladder and related conditions. UroGen Pharma is headquartered in Israel and also maintains a corporate office in New York City.

Forward Looking Statements

This press release contains forward-looking statements. All statements contained herein other than statements of historical fact constitute forward-looking statements, including statements regarding UroGen Pharma's clinical development plan, clinical trial results, timing and completion of clinical trials and potential approval of MitoGel and other UroGen Pharma drug candidates. These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of preclinical studies and clinical trials conducted by or on

behalf of UroGen Pharma, including with respect to the efficacy and safety of its product candidates; UroGen Pharma's ability to obtain and maintain regulatory approval of its product candidates, and the labeling for any approved products; the scope, progress, expansion and costs of developing and commercializing UroGen Pharma's product candidates; UroGen Pharma's ability to obtain and maintain intellectual property protection for its product candidates; UroGen Pharma's anticipated growth strategies; UroGen Pharma's expectations regarding competition; the anticipated trends and challenges in UroGen Pharma's business and the markets in which it operates; UroGen Pharma's ability to attract or retain key management and personnel; the size and growth of the potential markets for UroGen Pharma's product candidates and its ability to serve those markets; the rate and degree of market acceptance of UroGen Pharma's product candidates vis-à-vis alternative therapies; UroGen Pharma's expectations regarding regulatory requirements; developments in applicable regulatory regimes; UroGen Pharma's ability to enter into and maintain collaborations; and the manner in which UroGen Pharma intends to use its cash resources and the sufficiency thereof. Moreover, UroGen Pharma operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. It is not possible for UroGen Pharma's management to predict all risks, nor can they assess the impact of all factors on its business or the extent to which any such factor or combination of factors may cause actual results to differ materially from those contained herein. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed herein may not occur, and UroGen Pharma's actual results could differ materially and adversely from those anticipated or implied by the forward-looking statements contained herein. UroGen Pharma undertakes no obligation to update any such forward-looking statements after the date hereof to conform to actual results or changes in expectations.