UroGen Announces New Data from the OPTIMA II Study that Show Median Durability of Response of 24.4 Months for UGN-102, an Investigational Non-Surgical Chemoablative Treatment for Low-Grade, Intermediate-Risk Non-Muscle Invasive Bladder Cancer

December 2, 2022

-- Results from this ongoing, noninterventional rollover study were presented at the 23rd Annual Society of Urological Oncology (SUO) Meeting in San Diego

SAN DIEGO--(BUSINESS WIRE)--Dec. 2, 2022-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced new data from the OPTIMA II study designed to obtain long-term follow-up data on UGN-102 that shows median duration of response (DOR) of 24.4 months for UroGen's investigational drug UGN-102 currently in Phase 3 development for low-grade, intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). The study (Abstract #193) was presented at SUO on December 2.

"LG-IR-NMIBC is a challenging disease to treat because of the high recurrence rate managed by repetitive surgeries," says William C. Huang, M.D., FACS, Professor and Vice Chair of Urology at NYU Langone Health and Principal Investigator of the OPTIMA II trial. "The OPTIMA II Phase 2b trial showed significant tumor response benefit for patients using the novel chemoablative therapy UGN-102 for LG-IR-NMIBC and this latest analysis shows that the treatment benefit lasted for more than two years. I look forward to additional data on UGN-102, including evidence from the ENVISION Phase 3 study."

Patients who completed the OPTIMA II study were eligible to participate in this rollover study. Outcomes include DOR in patients who remained in complete response (CR) at the end of OPTIMA II, events of disease recurrence and progression, post-study treatments and death.

At the time of data cut off (February 25, 2022), data were available for 15 of the 25 patients. The median DOR among the 15 patients was 24.4 months (10.1 to 30.7 months). Seven patients remained in CR, six patients had recurrence of LG disease, one patient had progression to high-grade disease and one patient withdrew consent (no longer mobile) but remained in CR at the last evaluation prior to discontinuation. All patients were alive at the last contact, and five patients were known to have had post-study treatment with transurethral resection of the bladder tumors (three patients) or fulguration (two patients).

DOR was calculated as the time from documented CR in OPTIMA II to disease recurrence or death or last adequate disease assessment (for patients who remained in CR). The data cut off for this report is February 25, 2022.

"UGN-102 uses a similar combination with a simpler delivery method to our currently approved chemoablative medicine and has showed a similar durability of response in LG-IR-NMIBC," said Mark Schoenberg, M.D., Chief Medical Officer, UroGen. UroGen's proprietary RTGel™ technology allows medicines to dwell for several hours potentially improving the therapeutic effects of existing medicines. If approved, UGN-102 would be the only primary non-surgical treatment option for patients with LG-IR-NMIBC who often recur within one year of receiving surgery and continue to need repetitive surgeries for the rest of their life."

About the Phase 2b OPTIMA II Trial

OPTIMA II (OPTimized Instillation of Mitomycin for Bladder Cancer Treatment) is an open-label, single-arm, multi-center Phase 2b clinical trial of investigational agent UGN-102 (mitomycin) for intravesical solution to evaluate its safety and efficacy in patients with LG-IR-NMIBC.

Results showed:

- 65 percent CR at three months
- Kaplan-Meier analysis estimated duration of response to be 72.5 percent at 12 months from initiation of therapy (nine months from CR); median duration of response was not reached
- Treatment with UGN-102 was generally well tolerated, with mostly mild to moderate adverse events reported

Intermediate risk is defined as one or two of the following: multiple tumors, solitary tumor >3 cm, or recurrence (≥ 1 occurrence of LG-NMIBC within one year of the current diagnosis). Patients were to receive six weekly intravesical instillations of 75 mg UGN-102 in an office setting. The chemoablative effect of UGN-102 was assessed three months after initiation of study treatment with CR defined as a negative endoscopic examination, negative cytology, and when indicated, a negative for-cause biopsy. Patients achieving CR were followed quarterly to 12 months after initiation of study treatment to evaluate safety, efficacy, and durability.

About LG-IR-NMIBC

Approximately 800,000 people are living with bladder cancer in the U.S., of that 80,000 suffer from LG-IR-NMIBC. Patients with LG-IR-NMIBC face a future of recurrence and additional surgeries. Currently, the only primary treatment available is a surgical procedure known as transurethral resection of bladder tumor or TURBT, which requires anesthesia. Every time TURBT is performed it may impose more burden and serious risks on patients, including pain, bleeding, infection and injury (including perforation).

About UroGen Pharma Ltd.

UroGen is biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers because patients deserve better. 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 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 the ongoing non-interventional, rollover study of UGN-102; the ongoing ENVISION Phase 3 trial; the potential of UroGen's proprietary RTGel technology platform to improve therapeutic profiles of existing drugs; the potential benefits of UGN-102 for the treatment of LG-IR-NMIBC 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: results from initial reports of long term follow-up outcomes may not be indicative of results that may be observed in future clinical practice; the timing and success of clinical trials and potential safety and other complications thereof, including the ENVISION Phase 3 trial; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to maintain regulatory approval; complications associated with commercialization activities, including complications resulting from the COVID-19 pandemic; the labeling for any approved product; competition in our 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. 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 10, 2022 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/20221202005055/en/

INVESTORS:
Vincent Perrone Sr. 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.