UroGen Pharma Reports Record JELMYTO Quarterly Sales and Recent Corporate Developments

August 10, 2023

- *Both ENVISION and ATLAS Phase 3 trials of UGN-102 met primary endpoints in treating low-grade intermediate-risk non-muscle-invasive bladder cancer (LG-IR-NMIBC).*
- *Reported record JELMYTO® net product revenues in Q2 2023 of $21.1 million, an increase of ~27% from the same period last year*
- *Significantly strengthened balance sheet via $120 million private placement of ordinary shares and pre-funded warrants*

PRINCETON, N.J.--(BUSINESS WIRE)--Aug. 10, 2023-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced financial results for the second quarter ended June 30, 2023, and provided an overview of recent developments.

“We are in a transformative period for UroGen,” said Liz Barrett, President and Chief Executive Officer of UroGen Pharma. “Recently, we announced positive topline results from the ATLAS and ENVISION Phase 3 clinical trials of UGN-102 in LG-IR-NMIBC. Both trials met their primary endpoints, demonstrating consistent, compelling results for UGN-102. If approved, we believe UGN-102’s promising efficacy and safety profile across multiple trials support a potential shift from frequent surgeries to a minimally invasive, non-surgical option for the more than 82,000 underserved LG-IR-NMIBC patients diagnosed annually in the U.S. alone, positioning it as a potential key growth driver for UroGen.”

“Furthermore, increased adoption and expansion of JELMYTO use in a diverse LG-UTUC population, supported by a growing database of real-world outcomes data, translated to notable revenue growth during the second quarter of 2023,” continued Ms. Barrett. “Building on these achievements, the recently announced $120 million private placement of ordinary shares and pre-funded warrants with a select group of biotech investors provides us with the resources to support our business, including our prospective pre-commercialization and launch strategy for UGN-102.”

Business Highlights:

UGN-102 (mitomycin) for intravesical solution:

- The ENVISION Phase 3 clinical trial met its primary endpoint by demonstrating a 79.2% rate of complete response among ~240 LG-IR-NMIBC patients at 3-months after the first UGN-102 instillation.
- 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.
- The Journal of Urology published a peer-reviewed article highlighting that the ATLAS Phase 3 clinical trial met its primary endpoint of disease-free survival, with topline results demonstrating a reduced risk of recurrence, progression, or death of 55% for UGN-102 ± TURBT compared to TURBT monotherapy.
    - Patients who only received UGN-102 showed a 64.8% complete response rate at three months, compared to a 63.6% complete response rate at three months for patients who only received TURBT.
    - The estimated probability of remaining disease free 15-months after randomization was 72% for UGN-102 ± TURBT and 50% for TURBT monotherapy (hazard ratio 0.45).
- UroGen hosted an event to discuss and highlight topline results from the Phase 3 ATLAS and ENVISION clinical trials on July 27th, 2023. A replay of the event is available on the UroGen website at https://investors.urogen.com.

JELMYTO (mitomycin) for pyelocalyceal solution in low-grade upper tract urothelial cancer (LG-UTUC):

- Generated record quarterly net product revenue of $21.1 million for the second quarter of 2023, representing ~27% growth over the second quarter of 2022.
- Activated sites on August 1, 2023 were 1,058, compared to 1,009 on May 1, 2023, while repeat accounts on August 1, 2023 were 267, compared to 235 on May 1, 2023.
- Results from a retrospective study investigating whether patients with higher-volume low-grade disease could achieve disease-free status using partial ablation or biopsy before JELMYTO treatment during initial ureteroscopy (URS) showed no significant difference in rendered disease-free rates between complete ablation (78.6%), partial ablation (57.6%), or biopsy-only (66.7%) groups during initial URS (p=0.15). The analysis also showed that tumor size prior to JELMYTO

induction did not have a significant impact on rendered disease free (RDF) rates (p=0.09). The study aimed to find alternatives to nephroureterectomy for preserving kidney function and to assess JELMYTO's efficacy in managing larger volume disease.

Private Placement of Ordinary Shares

- Completed a private placement of ordinary shares and pre-funded warrants with gross proceeds of approximately $120 million before deducting placement agent commissions and other offering expenses to select institutional and accredited investors.

Second Quarter 2023 Financial Results:

JELMYTO Revenue: UroGen reported net product revenue of JELMYTO for the second quarter 2023 of $21.1 million, compared to $16.6 million in the second quarter of 2022.

R&D Expense: Research and development expenses for the second quarter 2023 were $11.6 million, including non-cash share-based compensation expense of $0.5 million as compared to $12.6 million, including non-cash share-based compensation expense of $0.7 million, for the same period in 2022.

SG&A Expense: Selling, general and administrative expenses for the second quarter 2023 were $22.5 million, including non-cash share-based compensation expense of $1.7 million. This compares to $20.8 million, including non-cash share-based compensation expense of $2.2 million, for the same period in 2022.

Financing on Prepaid Forward Obligation: UroGen reported non-cash financing expense related to the prepaid forward obligation to RTW Investments of $5.3 million for the second quarter 2023, compared to $5.8 million for the same period in 2022. The rate applied to cash payments incurred in 2023 is 13% based on global net product sales of JELMYTO in 2022.

Interest Expense on Long-Term Debt: Interest expense related to the $100 million term loan facility with funds managed by Pharmakon Advisors was $3.8 million for the second quarter of 2023, compared to $2.2 million for the same period last year due to the transaction closing in March 2022 and the final $25 million draw down under the term loan facility in December 2022.

Net Loss: UroGen reported a net loss of $24.1 million, or basic and diluted net loss per ordinary share of $1.03, for the second quarter 2023 as compared to $26.7 million, or basic and diluted net loss per ordinary share of $1.18, for the same period in 2022.

Cash & Cash Equivalents: As of June 30, 2023, cash, cash equivalents and marketable securities totaled $55.3 million. This figure does not include proceeds from the recent $120 million private placement of ordinary shares and pre-funded warrants.

2023 Revenue, Operating Expense and RTW Expense Guidance: The Company reiterates anticipated full year 2023 net product revenues from JELMYTO to be in the range of $76 to $86 million. The Company also reiterates anticipated full year 2023 operating expenses in the range of $135 to $145 million, including non-cash share-based compensation expense of $6.0 to $11.0 million, subject to market conditions. The Company also reiterates anticipated full year 2023 non-cash financing expense related to the prepaid obligation to RTW Investments in the range of $21.0 to $26.0 million. Of this amount approximately $9.9 to $11.2 million is expected to be in cash.

Conference Call & Webcast Information: Members of UroGen's management team will host a live conference call and webcast today at 10:00 AM Eastern Time to review UroGen's financial results and provide a general business update.

The live webcast can be accessed by visiting the Investors section of the Company's website at http://www.urogen.com. Please connect at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast.

UROGEN PHARMA LTD.
SELECTED CONSOLIDATED BALANCE SHEETS
(U.S. dollars in thousands)
(Unaudited)

	June 30, 2023	December 31, 2022
Cash and cash equivalents and marketable securities	$ 55,271	$ 99,963
Total assets	$ 95,361	$ 135,619
Total liabilities	$ 233,795	$ 224,980
Total shareholders' deficit	$ (138,434)	$ (89,361)

UROGEN PHARMA LTD.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(U.S. dollars in thousands, except share and per share data)
(Unaudited)

	Three months ended June 30,		Six months ended June 30,	
	2023	2022	2023	2022
Revenue	$ 21,139	$ 16,604	$ 38,331	$ 30,168
Cost of revenue	2,443	1,846	4,708	3,371
Gross profit	18,696	14,758	33,623	26,797
Operating expenses:				

Research and development expenses	11,584	12,640	24,082	25,336
Selling, general and administrative expenses	22,494	20,833	46,968	42,133
Total operating expenses	34,078	33,473	71,050	67,469
Operating loss	(15,382)	(18,715)	(37,427)	(40,672)
Financing on prepaid forward obligation	(5,344)	(5,833)	(10,568)	(11,659)
Interest expense on long-term debt	(3,761)	(2,239)	(7,314)	(2,521)
Interest and other income (expense), net	405	128	1,035	126
Loss before income taxes	$ (24,082)	$ (26,659)	$ (54,274)	$ (54,726)
Income tax expense	(54)	(32)	(75)	(357)
Net loss	$ (24,136)	$ (26,691)	$ (54,349)	$ (55,083)
Net loss per ordinary share basic and diluted	$ (1.03)	$ (1.18)	$ (2.33)	$ (2.43)
Weighted average shares outstanding, basic and diluted	23,462,016	22,703,572	23,371,878	22,667,825

About JELMYTO®

JELMYTO® (mitomycin) for pyelocalyceal solution is a mitomycin-containing reverse thermal gel containing 4 mg mitomycin per ml gel indicated for primary chemoablative treatment of LG-UTUC in adults. It is recommended for primary treatment of biopsy-proven LG-UTUC in patients deemed appropriate candidates for renal-sparing therapy. JELMYTO is a viscous liquid when cooled and becomes a semi-solid gel at body temperature. The drug slowly dissolves over four to six hours after instillation and is removed from the urinary tract by normal urine flow and voiding. It is approved for administration in a retrograde manner via ureteral catheter or antegrade through nephrostomy tube. The delivery system allows the initial liquid to coat and conform to the upper urinary tract anatomy. The eventual semisolid gel allows for chemoablative therapy to remain in the collecting system for four to six hours without immediately being diluted or washed away by urine flow.

APPROVED USE FOR JELMYTO

JELMYTO® is a prescription medicine used to treat adults with a type of cancer of the lining of the upper urinary tract including the kidney called low-grade Upper Tract Urothelial Cancer (LG-UTUC).

IMPORTANT SAFETY INFORMATION

You should not receive JELMYTO if you have a hole or tear (perforation) of your bladder or upper urinary tract.

Before receiving JELMYTO, tell your healthcare provider about all your medical conditions, including if you:

- are pregnant or plan to become pregnant. JELMYTO can harm your unborn baby. You should not become pregnant during treatment with JELMYTO. Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with JELMYTO. **Females who are able to become pregnant:** You should use effective birth control (contraception) during treatment with JELMYTO and for 6 months after the last dose. **Males being treated with JELMYTO:** If you have a female partner who is able to become pregnant, you should use effective birth control (contraception) during treatment with JELMYTO and for 3 months after the last dose.
- are breastfeeding or plan to breastfeed. It is not known if JELMYTO passes into your breast milk. Do not breastfeed during treatment with JELMYTO and for 1 week after the last dose.
- **Tell your healthcare provider if you take water pills (diuretic).**

How will I receive JELMYTO?

- Your healthcare provider will tell you to take a medicine called sodium bicarbonate before each JELMYTO treatment.
- You will receive your JELMYTO dose from your healthcare provider 1 time a week for 6 weeks. It is important that you receive all 6 doses of JELMYTO according to your healthcare provider's instructions. If you miss any appointments, call your healthcare provider as soon as possible to reschedule your appointment. Your healthcare provider may recommend up to an additional 11 monthly doses.
- JELMYTO is given to your kidney through a tube called a catheter.
- During treatment with JELMYTO, your healthcare provider may tell you to take additional medicines or change how you take your current medicines.

After receiving JELMYTO:

- JELMYTO may cause your urine color to change to a violet to blue color. Avoid contact between your skin and urine for at least 6 hours.
- To urinate, **males and females should sit** on a toilet and flush the toilet several times after you use it. After going to the bathroom, wash your hands, your inner thighs, and genital area well with soap and water.
- Clothing that comes in contact with urine should be washed right away and washed separately from other clothing.

JELMYTO may cause serious side effects, including:

- **Swelling and narrowing of the tube that carries urine from the kidney to the bladder (ureteric obstruction).** If you develop swelling and narrowing, and to protect your kidney from damage, your healthcare provider may recommend the placement of a small plastic tube (stent) in the ureter to help the kidney drain. Tell your healthcare provider right away if you develop side pain or fever during treatment with JELMYTO.

- **Bone marrow problems.** JELMYTO can affect your bone marrow and can cause a decrease in your white blood cell, red blood cell, and platelet counts. Your healthcare provider will do blood tests prior to each treatment to check your blood cell counts during treatment with JELMYTO. Your healthcare provider may need to temporarily or permanently stop JELMYTO if you develop bone marrow problems during treatment with JELMYTO.
- **The most common side effects of JELMYTO include:** urinary tract infection, blood in your urine, side pain, nausea, trouble with urination, kidney problems, vomiting, tiredness, stomach (abdomen) pain.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to **UroGen Pharma** at 1-855-987-6436.

Please see JELMYTO Full Prescribing Information, including the Patient Information, for additional information.

About Upper Tract Urothelial Cancer (UTUC)

Urothelial cancer is the ninth most common cancer globally and the eighth most lethal neoplasm in men in the U.S. Between five percent and ten percent of primary urothelial cancers originate in the ureter or renal pelvis and are collectively referred to as upper tract urothelial cancers (UTUC). In the U.S., there are approximately 6,000 - 7,000 new or recurrent low-grade UTUC patients annually. Most cases are diagnosed in patients over 70 years old, and these older patients often face comorbidities. There are limited treatment options for UTUC, with the most common being endoscopic surgery or nephroureterectomy (removal of the entire kidney and ureter). These treatments can lead to a high rate of recurrence and relapse.

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 secondary endpoint 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 the Phase 3 ENVISION Trial

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 secondary endpoint 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 the Phase 3 ATLAS Trial

ATLAS was 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 + / - TURBT 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; our belief that UGN-102, if approved, will support a potential shift from frequent surgeries to UGN-102, positioning it as a potential key growth driver for UroGen; financial guidance for 2023; 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) thereof 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; market conditions and third-party performance; 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 Form 10-Q filed with the SEC on May 11, 2023, as well as in the Risk Factors section of UroGen's Quarterly Report on Form 10-Q being filed with the SEC later today (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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