UroGen Pharma Ahead of Schedule to Complete UGN-102 NDA Submission and Reports 2024 Second Quarter Financial Results and Business Highlights

August 13, 2024

- *Potential for an FDA decision as early as the first quarter of 2025, assuming priority review*
- *UGN-102 Phase 3 ENVISION trial demonstrated an unprecedented 82.3% Duration of Response at 12 Months by Kaplan-Meier analysis in LG-IR-NMIBC patients who achieved a complete response at three months*
- *Ended Q2 2024 with $241.3 million in cash, cash equivalents and marketable securities*
- *Reported JELMYTO® Q2 2024 net product revenues of $21.8 million*
- *Conference call and webcast to be held today at 10:00 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--Aug. 13, 2024-- 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, 2024, and provided an overview of recent developments.

“Our immediate priority is completing the submission of a New Drug Application in the very near term for UGN-102, which we believe has the potential to be a practice-changing therapy for the treatment of low-grade intermediate-risk non-muscle invasive bladder cancer,” said Liz Barrett, President and Chief Executive Officer of UroGen. “The compelling body of clinical data, including the ENVISION trial, which demonstrated an unprecedented 82.3% 12-month duration of response by Kaplan-Meier analysis in patients who had previously achieved a complete response at three months, reinforces the opportunity for UGN-102 to be the first FDA-approved medicine for the treatment of low-grade intermediate-risk non-muscle invasive bladder cancer.”

Ms. Barrett continued, “We estimate that approximately 82,000 patients suffering from this highly recurrent disease each year may benefit from an innovative treatment, creating an estimated five-billion-dollar market opportunity. Our immediate commercial focus is preparing for UGN-102’s potential approval and launch with the goal to establish our leadership in urothelial cancers.”

Q2 2024 and Recent Business Highlights:

UGN-102 (mitomycin) for intravesical solution:

- In June 2024, UroGen reported positive 12-month duration of response (DOR) data from the Phase 3 ENVISION pivotal trial evaluating UGN-102 (mitomycin) for intravesical solution in patients with low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). The 12-month DOR was 82.3% (95% CI, 75.9%, 87.1%) by Kaplan-Meier estimate in patients who had achieved complete response (CR) at three months from the first instillation of investigational drug UGN-102. The ENVISION trial previously met its primary endpoint by demonstrating that patients treated with UGN-102 had a 79.6% (95% CI, 73.9%, 84.5%) CR rate at three months following the first instillation of UGN-102. UGN-102 was well tolerated, with a safety profile that was consistent with previous clinical trials.

- The ENVISION 12-month DOR data were presented in a virtual event “New Horizons in Bladder Cancer” hosted by UroGen on June 13. This event included presentations by company management and several key opinion leaders with expertise in urology. There was also a panel discussion on the treatment of LG-IR-NMIBC and insights from a patient from the ENVISION trial. A replay of the event can be accessed here.

- The latest DOR data is expected to support a New Drug Application (NDA) for UGN-102 as a treatment for LG-IR-NMIBC, which the Company plans to complete in the very near term. There is potential for an FDA decision as early as the first quarter of 2025, assuming the FDA grants priority review. UroGen initiated submission of the rolling NDA for UGN-102 in January 2024.

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

- Generated net product revenue of $21.8 million in the second quarter of 2024, compared to $21.1 million in the second quarter of 2023.

- JELMYTO was featured in three presentations at the AUA 2024 Annual Meeting. Independent long-term, real-world analyses explored use of the product in broad patient types, and with different methods of administration. The results showed that JELMYTO treatment appears to demonstrate favorable recurrence-free survival rates for patients with LG-UTUC who respond to initial induction. There does not appear to be a recurrence difference according to the intent of JELMYTO induction, original tumor size, multifocality or tumor location**.**

Next-generation novel mitomycin-based formulation for urothelial cancers

- UroGen is developing UGN-103 and UGN-104, next-generation novel mitomycin-based formulations for UGN-102 and JELMYTO, respectively. These candidates combine UroGen’s RTGel® technology with a novel mitomycin formulation

licensed from medac GmbH in an agreement signed in January 2024. The development programs potentially offer both manufacturing efficiencies and additional intellectual property protection for the Company's low-grade urothelial cancer franchise.

- In April 2024, the U.S. FDA accepted the Company's Investigational New Drug (IND) application for UGN-103. If approved, UGN-103 is expected to provide several advantages related to production, cost, supply, and product convenience.

- UroGen has initiated the Phase 3 study and has onboarded three clinical sites to explore the safety and efficacy of UGN-103 in LG-IR-NMIBC. UroGen plans to initiate a Phase 3 study of UGN-104 in LG-UTUC early next year.

Corporate

- In June 2024, UroGen appointed David Lin as Chief Commercial Officer and member of the Executive Leadership Team. Mr. Lin is spearheading UroGen's commercial strategy and will be leveraging his extensive experience to prepare for the potential launch of UGN-102, if approved.

Public offering of ordinary shares and pre-funded warrants

- In June 2024, the Company completed an underwritten public offering of 5,000,000 ordinary shares at a price to the public of $17.50 per ordinary share, and, to certain investors in lieu of issuing ordinary shares, pre-funded warrants to purchase 1,142,857 ordinary shares at a purchase price of $17.499 per pre-funded warrant, which equals the public offering price per ordinary share less the $0.001 per share exercise price for each pre-funded warrant. Gross proceeds to UroGen from the offering, before deducting underwriting discounts and commissions and estimated offering expenses, were approximately $107.5 million.

- In July 2024 the underwriters exercised their option to purchase the full 921,428 additional shares. This yielded further gross proceeds to the Company of $16.1 million, before deducting underwriting discounts and commissions and estimated offering expenses.

Second quarter 2024 financial results

JELMYTO Revenue: JELMYTO net product revenues were $21.8 million and $21.1 million for the three months ended June 30, 2024, and 2023, respectively.

R&D Expense: Research and development expenses for the second quarter of 2024 were $15.4 million, including non-cash share-based compensation expense of $0.6 million as compared to $11.6 million, including non-cash share-based compensation expense of $0.5 million, for the same period in 2023.

SG&A Expense: Selling, general and administrative expenses for the second quarter of 2024 were $30.1 million, including non-cash share-based compensation expense of $3.0 million. This compares to $22.5 million, including non-cash share-based compensation expense of $1.7 million, for the same period in 2023.

Financing on Prepaid Forward Obligation: UroGen reported non-cash financing expense related to the prepaid forward obligation to RTW Investments of $5.8 million in the second quarter of 2024, compared to $5.3 million in the same period in 2023.

Interest Expense on Long-Term Debt: Interest expense related to the up to $200 million term loan facility with funds managed by Pharmakon Advisors was $3.5 million in the second quarter of 2024, compared to $3.8 million in the same period in 2023.

Net Loss: UroGen reported a net loss of $33.4 million or ($0.91) per basic and diluted share in the second quarter of 2024 compared with a net loss of $24.1 million or ($1.03) per basic and diluted share in the same period in 2023.

Cash & Cash Equivalents: As of June 30, 2024, cash, cash equivalents and marketable securities totaled $241.3 million.

2024 Revenue, Operating Expense, and RTW Expense Guidance: With respect to the Company's previously provided full-year 2024 JELMYTO revenue guidance, the Company sees a path toward the lower end of the guidance range. With respect to the Company's previously provided full-year 2024 operating expense guidance, the Company expects to be toward the higher end of the guidance range, with a revised non-cash share-based compensation expense of $9 to $13 million, subject to market conditions. The anticipated full year 2024 non-cash financing expense related to the prepaid obligation to RTW Investments is unchanged and expected to be in the range of $21 to $26 million. The rate for the cash component of the RTW obligation will be 13% of global net product sales of JELMYTO in 2024.

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://investors.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, 2024	December 31, 2023
Cash and cash equivalents and marketable securities	$ 241,280	$ 141,470
Total assets	$ 281,849	$ 178,311
Total liabilities	$ 251,535	$ 243,523

Total shareholders' equity (deficit)	$ 30,314	$ (65,212)

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,	
	2024	2023	2024	2023
Revenue	$ 21,848	$ 21,139	$ 40,629	$ 38,331
Cost of revenue	2,229	2,443	3,957	4,708
Gross profit	19,619	18,696	36,672	33,623
Operating expenses:				
Research and development expenses	15,402	11,584	30,896	24,082
Selling, general and administrative expenses	30,056	22,494	57,355	46,968
Total operating expenses	45,458	34,078	88,251	71,050
Operating loss	(25,839)	(15,382)	(51,579)	(37,427)
Financing on prepaid forward obligation	(5,773)	(5,344)	(11,433)	(10,568)
Interest expense on long-term debt	(3,461)	(3,761)	(5,908)	(7,314)
Interest and other income, net	1,708	405	3,323	1,035
Loss before income taxes	$ (33,365)	$ (24,082)	$ (65,597)	$ (54,274)
Income tax expense	(38)	(54)	(92)	(75)
Net loss	$ (33,403)	$ (24,136)	$ (65,689)	$ (54,349)
Net loss per ordinary share basic and diluted	$ (0.91)	$ (1.03)	$ (1.87)	$ (2.33)
Weighted average shares outstanding, basic and diluted	36,821,915	23,462,016	35,106,524	23,371,878

About JELMYTO

JELMYTO (mitomycin) for pyelocalyceal solution is a mitomycin-containing reverse thermal gel containing 4 mg mitomycin per mL gel indicated for the treatment of adult patients with LG-UTUC. 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 innovative drug formulation of mitomycin, currently 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 by a trained healthcare professional. UroGen anticipates completing its NDA submission for UGN-102 in the very near term with a potential FDA decision as early as the first quarter of 2025, assuming priority review.

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 the 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. Our first product to treat low-grade upper tract urothelial cancer and investigational treatment UGN-102 (mitomycin) for intravesical solution for patients with LG-IR-NMIBC 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 X (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: expected timing for completing the NDA submission for UGN-102 and potential FDA approval; the potential of UGN-102, including to be a practice-changing therapy and the first FDA approved medicine for LG-IR-NMIBC; the estimated patient population and market opportunity for UGN-102; the expectation that the ENVISION DOR data will support an NDA for UGN-102; the potential and advantages of UGN-103 and UGN-104, including to potentially offer manufacturing efficiencies and additional intellectual property protection for UroGen's low-grade urothelial cancer franchise; the ongoing and planned clinical trials for UGN-103 and UGN-104; UroGen's goals and commercial focus; 2024 financial guidance; the estimated patient population and demographics for UTUC; the potential of UroGen's proprietary RTGel technology to improve therapeutic profiles of existing drugs; and the potential of UroGen's sustained release technology to make local delivery more effective as compared to other treatment options. Words such as "anticipate," "assume," "could," "plan," "potential," "will," or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. 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; results from the ENVISION Phase 3 trial may not be sufficient to support an NDA submission for UGN-102; even if an NDA for UGN-102 is accepted by the FDA, there is no guarantee that such NDA will be given priority review or that such NDA will be sufficient to support approval of UGN-102 on the timeframe expected, or at all; the ability to obtain regulatory approval within the timeframe expected, or at all; the ability to obtain and maintain adequate intellectual property rights and adequately protect and enforce such rights; 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) for UroGen's product and product candidates 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; and 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 Quarterly Report on Form 10-Q, filed with the SEC on May 13, 2024, 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 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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