UroGen Pharma Reports 2024 Third Quarter Financial Results and Business Highlights, Including the Potential Launch of UGN-102 in 2025

November 6, 2024

- *New Drug Application for UGN-102 accepted by US FDA; PDUFA target action date set for June 13, 2025*
- *JELMYTO® achieved net product sales of $25.2 million in Q3 2024, compared to $20.9 million in Q3 2023*
- *$254.2 million in cash, cash equivalents and marketable securities as of September 30, 2024*
- *Conference call and webcast to be held today at 10:00 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--Nov. 6, 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 third quarter ended September 30, 2024, and provided an overview of recent developments.

“The recent FDA acceptance of our New Drug Application for UGN-102 marks a significant milestone in our mission to deliver breakthrough treatments to patients,” said Liz Barrett, President and Chief Executive Officer of UroGen. “Supported by robust clinical evidence, we believe UGN-102 has the potential to redefine the treatment of low-grade intermediate-risk non-muscle invasive bladder cancer and provide longer treatment-free intervals for patients that currently face the burden of repetitive surgeries under general anesthesia. We look forward to a potential FDA approval by the PDUFA target action date of June 13, 2025. If approved, we believe UGN-102 will represent a paradigm shift in care, addressing a total market opportunity of over $5 billion. Our team is fully focused on preparing for a successful commercial launch of UGN-102, if approved, in 2025.”

Q3 2024 and Recent Business Highlights:

UGN-102 (mitomycin) for intravesical solution:

- In October 2024, the U.S. Food and Drug Administration (FDA) accepted UroGen’s New Drug Application (NDA) for investigational drug UGN-102 (mitomycin) for intravesical solution as a treatment for low-grade intermediate risk non-muscle invasive bladder cancer (LG-IR-NMIBC). The FDA granted a Prescription Drug User Fee Act (PDUFA) target action date of June 13, 2025.
- The NDA for UGN-102 is supported by a comprehensive development program which demonstrated a clinically meaningful complete response rate and a strong duration of response across three late phase clinical trials and an acceptable safety profile. The Phase 3 ENVISION trial successfully met its primary endpoint by demonstrating that patients treated with UGN-102 had a 79.6% (95% CI, 73.9%, 84.5%) complete response (CR) rate at three months following the first instillation of UGN-102. More recently, durability data from ENVISION showed that UGN-102 demonstrated an 82.3% (95% CI, 75.9%, 87.1%) 12-month duration of response (DOR) by Kaplan-Meier estimate in patients who had achieved a complete response at three months after the first instillation of UGN-102. This is the highest DOR ever reported in this patient population. The ENVISION trial demonstrated that UGN-102 has an acceptable safety profile that is consistent with the side effect profile observed in previous clinical trials.

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

- Generated net product revenue of $25.2 million in the third quarter of 2024, compared to $20.9 million in the third quarter of 2023, primarily driven by strong underlying demand growth and also reflecting non-patient purchases.

Next-generation novel mitomycin-based formulation for urothelial cancers

- In October 2024, the first patient was dosed in the Phase 3 UTOPIA clinical trial of investigational drug UGN-103 (mitomycin) for intravesical solution in patients with LG-IR-NMIBC. UGN-103 is a next generation product that combines UroGen’s RTGel ® technology with a novel mitomycin formulation licensed from medac GmbH. UGN-103 is planned to follow the potential FDA approval and launch of UGN-102 for LG-IR-NMIBC. UroGen plans to initiate a Phase 3 study early next year to explore the safety and efficacy of UGN-104, its next generation product for the treatment of LG-UTUC. To learn more about the UTOPIA trial, refer to clinicaltrials.gov/NCT06331299.
- In September 2024, UroGen received a Notice of Allowance from the United States Patent and Trademark Office for a patent application covering, among other things, the use of UGN-103 in the treatment of LG-IR-NMIBC. The U.S. patent, once issued, will have an expiration date in December 2041.

Corporate

- In October 2024, UroGen appointed Chris Degnan as Chief Financial Officer. Mr. Degnan brings broad expertise in financial strategy, investor relations, SEC reporting, accounting and compliance, having previously held CFO roles at Galera Therapeutics and Verrica Pharmaceuticals. He replaced Don Kim who left the Company to pursue other opportunities.

Third Quarter 2024 Financial Results

JELMYTO Revenue: JELMYTO net product revenues were $25.2 million and $20.9 million for the three months ended September 30, 2024, and 2023, respectively.

R&D Expense: Research and development expenses for the third quarter of 2024 were $11.4 million, including non-cash share-based compensation expense of $0.6 million as compared to $10.2 million, including non-cash share-based compensation expense of $0.4 million, for the same period in 2023.

SG&A Expense: Selling, general and administrative expenses for the third quarter of 2024 were $28.9 million, including non-cash share-based compensation expense of $2.9 million. This compares to $21.8 million, including non-cash share-based compensation expense of $1.8 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 $5.9 million in the third quarter of 2024, compared to $5.5 million in the same period in 2023.

Interest Expense on Long-Term Debt: Interest expense related to the outstanding $125 million term loan facility with funds managed by Pharmakon Advisors was $2.7 million in the third quarter of 2024, compared to $3.8 million in the same period in 2023.

Net Loss: UroGen reported a net loss of $23.7 million or ($0.55) per basic and diluted share in the third quarter of 2024 compared with a net loss of $21.9 million or ($0.68) per basic and diluted share in the same period in 2023.

Cash & Cash Equivalents: As of September 30, 2024, cash, cash equivalents and marketable securities totaled $254.2 million.

For further details on the Company's financials, including results for the 9-month period ended September 30, 2024, refer to Form 10-Q, filed with the SEC.

2024 Revenue, Operating Expense, and RTW Expense Guidance: With respect to the Company's previously provided full-year 2024 JELMYTO revenue guidance, the Company expects full-year revenues to be below the low end of the guidance range. The Company does expect JELMYTO to achieve low double-digit year-over-year revenue growth for the full year 2024. With respect to the Company's previously provided full-year 2024 operating expenses guidance, the Company expects to be toward the midpoint of the guidance range, including 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)

	September 30, 2024	December 31, 2023
Cash and cash equivalents and marketable securities	$ 254,217	$ 141,470
Total assets	$ 301,943	$ 178,311
Total liabilities	$ 276,428	$ 243,523
Total shareholders' equity (deficit)	$ 25,515	$ (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 September 30,		Nine months ended September 30,	
	2024	2023	2024	2023
Revenue[1]	$ 25,204	$ 20,852	$ 65,833	$ 59,183
Cost of revenue	2,453	2,367	6,410	7,075
Gross profit	22,751	18,485	59,423	52,108
Operating expenses:				
Research and development expenses	11,355	10,230	42,251	34,312
Selling, general and administrative expenses	28,941	21,755	86,296	68,723
Total operating expenses	40,296	31,985	128,547	103,035
Operating loss	(17,545)	(13,500)	(69,124)	(50,927)
Financing on prepaid forward obligation	(5,915)	(5,479)	(17,348)	(16,047)
Interest expense on long-term debt	(2,721)	(3,815)	(8,629)	(11,129)
Interest and other income, net	2,599	906	5,922	1,941

Loss before income taxes	$ (23,582)	$ (21,888)	$ (89,179)	$ (76,162)
Income tax expense	(91)	9	(183)	(66)
Net loss	$ (23,673)	$ (21,879)	$ (89,362)	$ (76,228)
Net loss per ordinary share basic and diluted	$ (0.55)	$ (0.68)	$ (2.36)	$ (2.89)
Weighted average shares outstanding, basic and diluted	43,100,237	32,298,182	37,797,492	26,358,719

1. JELMYTO net revenues include $2.6 million and $1.1 million in CREATES Act sales during the three months ended September 30, 2024, and 2023 respectively.

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 under regulatory review for approval in 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. An NDA for UGN-102 is currently under review by the FDA with a potential decision expected by June 13, 2025. The U.S. market for LG-IR-NMIBC that UGN-102 can address, if approved, is valued at approximately $5 billion.

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: the potential timing for a decision from the FDA on the UGN-102 NDA; the potential launch timing for UGN-102, if approved; the potential patient benefits and opportunities for UGN-102, if approved; the estimated size of the U.S. market that could be addressed by UGN-102, if approved; the plans for UGN-103 to follow the potential FDA approval and launch of UGN-102; the timing for the planned Phase 3 trial of UGN-103 and UGN-104 and the potential approval of both product candidates; the anticipated intellectual property protection for UGN-103; 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," "believe," "expect," "if," "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: 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 though the NDA for UGN-102 has been accepted by the FDA, there is no guarantee that such NDA will be sufficient to support approval of UGN-102 on the timeframe expected, or at all; the PDUFA target action date may be delayed due to various factors outside UroGen's control; the FDA may schedule an advisory committee meeting for UGN-102, and the impact of any such advisory committee meeting or recommendation on the approval decision by the FDA is uncertain; the ability to obtain and maintain adequate intellectual property rights and adequately protect and enforce such rights; past results are not necessarily indicative of results that may be seen in the future, including in larger patient populations; the ability to obtain and maintain regulatory approval within the timeframe expected, or at all; 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) therefor and the rate and degree of market acceptance thereof vis-à-vis alternative therapies or procedures, such as surgery; UroGen's ability to attract or retain key management, members of the board of directors and other 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 for the quarter ended June 30, 2024, filed with the SEC on August 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 is 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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