UroGen Pharma Reports Fourth Quarter and Full Year 2024 Financial Results and Announces Updated 18-Month Duration of Response (DOR) of 80.6% from the Phase 3 ENVISION Trial of UGN-102

March 10, 2025

- *UGN-102 Pivotal ENVISION trial demonstrated an 18-month DOR of 80.6% (95% CI: 74.0, 85.7), by Kaplan-Meier estimate, for patients who achieved a complete response (CR) at three months after the first instillation of UGN-102*
- *New drug application (NDA) for UGN-102 under review by the FDA; Prescription Drug User Fee Act (PDUFA) target action date set for June 13, 2025*
- *Acquired a next-generation investigational oncolytic virus (ICVB-1042) and announced multiple strategic research collaborations in support of long-term growth strategy*
- *JELMYTO® achieved net product revenue of $90.4 million in 2024, compared with $82.7 million in 2023, driven by underlying demand revenue growth of 12% for 2024 and 15% for Q4 2024*
- *$241.7 million in cash, cash equivalents and marketable securities as of December 31, 2024*
- *Conference call and webcast to be held today at 10:00 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--Mar. 10, 2025-- 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 fourth quarter and full year ended December 31, 2024, and provided an overview of recent developments.

“2024 was a pivotal year for UroGen with achievements across our commercial business and pipeline,” said Liz Barrett, President and Chief Executive Officer of UroGen. “The progress with UGN-102 for low-grade intermediate-risk non-muscle invasive bladder cancer, including completion of the submission of our NDA ahead of schedule and compelling DOR data from the Phase 3 ENVISION trial, further positions us to launch a product that we believe will represent a paradigm shift in care, if approved. UGN-102 is supported by a compelling body of clinical data, and we are very pleased to report today the 18-month DOR of 80.6% from the ENVISION trial, maintaining duration of response consistent with 12-month DOR of 82.5% after a three-month CR was achieved. If approved, UGN-102 will address an estimated market opportunity of over $5 billion. We continue to advance our early-stage pipeline, including through the recent purchase of ICVB-1042, a next generation investigational oncolytic virus therapy with potential applications in bladder and other cancers.”

2024 and Recent Business Highlights:

UGN-102 (mitomycin) for intravesical solution:

- UroGen’s 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), is currently under review by the FDA. The FDA assigned a PDUFA target action date of June 13, 2025.
- The Company reported updated 18-month DOR data from the Phase 3 ENVISION trial that evaluated UGN-102 in patients with LG-IR-NMIBC. The 18-month DOR data is consistent with prior Kaplan-Meier estimates, with a DOR rate of 80.6% (95% CI: 74.0, 85.7) at 18-months (N=101) compared to 82.5% (95% CI: 76.1, 87.3) at 12-months (N=146). Median follow-up time at 18 months was 18.7 months after the three-month CR.
- Results from a subgroup analysis of the ENVISION trial were featured in a poster at the American Society of Clinical Oncology’s Genitourinary Cancers Symposium (ASCO-GU 2025) in February 2025. The analysis of pre-specified subgroups (presented by Sandip M Prasad, MD, MPhil et al) suggests that tumor burden and the number of tumors at baseline did not significantly affect the CR rate or DOR for patients treated with UGN-102.

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

- Generated net product revenue of $90.4 million and $82.7 million for the years ended December 31, 2024 and 2023, respectively. Underlying demand revenue increased 12% year-over-year, partially offset by a decrease in CREATES Act sales, which totaled $3.0 million in 2024 compared to $4.4 million in 2023. “New prescribers” and “new patient” starts increased 33% and 13%, respectively, in 2024.
- A long-term study on JELMYTO titled, “Durability of Response of UGN-101: Longitudinal Follow-Up of Multicenter Study,” was published online in *Urologic Oncology: Seminars and Investigations* in January 2025. The results showed 68% recurrence-free survival rate at three years across a broad patient population with LG-UTUC.
- A long-term follow-up study to the OLYMPUS trial evaluating JELMYTO was presented at the Society for Urologic Oncology annual meeting in December 2024. The study reported a median DOR of 47.8 months in patients who achieved a CR after primary chemoablation with JELMYTO. These results were also published online in the *Journal of Urology*.

Next-generation novel mitomycin-based formulation for urothelial cancers

- Enrollment is ongoing in the Phase 3 UTOPIA trial of investigational drug UGN-103 (mitomycin) for intravesical solution in

patients with LG-IR-NMIBC. UGN-103 is a next generation investigational medicine 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. To learn more about the UTOPIA trial, refer to clinicaltrials.gov/NCT06331299.

- UroGen plans to initiate a Phase 3 trial to explore the safety and efficacy of UGN-104, its next generation product for LG-UTUC in the first half of 2025.

UGN-301 (zalifrelimab), an anti-CTLA4 antibody for use in high-grade non-muscle invasive bladder cancer

- Completed enrollment of the dose escalation phase of UGN-301, alone and in combination with fixed dose UGN-201 or gemcitabine. The treatments demonstrated an acceptable safety profile and were generally well tolerated across dose levels. Responses were observed in both monotherapy and combination therapy arms, and patient follow-up is ongoing to further assess the durability of these responses.

Next-generation investigational oncolytic virus ICVB-1042

- In February 2025, UroGen expanded its pre-clinical oncology portfolio with the purchase of the product candidate ICVB-1042 and certain related assets from IconOVir Bio, Inc. ICVB-1042 is being developed to be a potent, fast-replicating, next-generation oncolytic virus therapy that can be administered locally to treat bladder cancer and potentially other specialty cancers. UroGen hosted a webinar to discuss ICVB-1042 on February 20, 2025 and a replay can be accessed on the Company's website here.

Fourth Quarter and Full Year 2024 Financial Results

JELMYTO Revenue: JELMYTO net product revenue was $24.6 million in the fourth quarter of 2024, compared to $23.5 million for the same period in 2023. Underlying demand revenue increased 15% year-over-year, partially offset by a decrease in CREATES Act sales, which totaled $0.2 million in the fourth quarter of 2024, compared to $2.4 million in the same period in 2023. JELMYTO net product revenue for the full year ended December 31, 2024 was $90.4 million, compared with $82.7 million for the full year ended December 31, 2023.

R&D Expense: Research and development expenses for the fourth quarter of 2024 were $14.9 million, as compared to $11.3 million for the same period in 2023. Research and development expenses for the full year 2024 were $57.1 million compared with $45.6 million for the full year 2023.

SG&A Expense: Selling, general and administrative expenses for the fourth quarter of 2024 were $34.9 million compared to $24.6 million in the same period in 2023. Selling, general and administrative expenses were $121.2 million for the full year 2024 compared with $93.3 million for the full year 2023.

Financing on Prepaid Forward Obligation: UroGen reported non-cash financing expense related to the prepaid forward obligation to RTW Investments of $6.1 million in the fourth quarter of 2024, compared to $5.5 million in the same period in 2023. Non-cash financing expense related to RTW Investments were $23.4 million in the full year 2024 compared with $21.6 million in the full year 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 $3.9 million and $12.5 million, respectively, for the fourth quarter and full year 2024, compared with $3.6 million and $14.7 million, respectively for the fourth quarter and full year 2023.

Net Loss: UroGen reported a net loss of $37.5 million or ($0.80) per basic and diluted share in the fourth quarter of 2024 compared with a net loss of $26.0 million or ($0.72) per basic and diluted share in the same period in 2023. Net loss was $126.9 million or ($2.96) per basic and diluted share in 2024 compared with a net loss of $102.2 million or ($3.55) per basic and diluted share in 2023.

Cash & Cash Equivalents: As of December 31, 2024, cash, cash equivalents and marketable securities totaled $241.7 million.

For further details on the Company's financials, refer to Form 10-K, filed with the SEC.

2025 JELMYTO Revenue and Company Operating Expense Guidance: The Company expects full-year 2025 net product revenues from JELMYTO to be in the range of $94 to $98 million. This implies a year-over-year growth rate of approximately 8% to 12% over the $87.4 million in demand driven JELMYTO sales in 2024, which excludes the $3.0 million in CREATES Act sales reported in 2024. Full-year 2025 operating expenses are expected to be in the range of $215 to $225 million, including non-cash share-based compensation expense of $11 million to $14 million.

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 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)

	December 31, 2024	December 31, 2023
Cash and cash equivalents and marketable securities	$ 241,707	$ 141,470
Total assets	$ 285,711	$ 178,311
Total liabilities	$ 294,514	$ 243,523
Total shareholders' deficit	$ (8,803)	$ (65,212)

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

	Three months ended December 31,		Year ended December 31,	
	2024	2023	2024	2023
Revenue	$ 24,565	$ 23,530	$ 90,398	$ 82,713
Cost of revenue	2,471	2,286	8,881	9,361
Gross profit	22,094	21,244	81,517	73,352
Operating expenses:				
Research and development expenses	14,894	11,302	57,145	45,614
Selling, general and administrative expenses	34,858	24,551	121,154	93,274
Total operating expenses	49,752	35,853	178,299	138,888
Operating loss	(27,658)	(14,609)	(96,782)	(65,536)
Financing on prepaid forward obligation	(6,063)	(5,505)	(23,411)	(21,552)
Interest expense on long-term debt	(3,892)	(3,586)	(12,521)	(14,715)
Interest and other income, net	2,750	1,538	8,672	3,479
Loss before income taxes	(34,863)	(22,162)	(124,042)	(98,324)
Income tax expense	(2,649)	(3,854)	(2,832)	(3,920)
Net loss	$ (37,512)	$ (26,016)	$ (126,874)	$ (102,244)
Net loss per ordinary share basic and diluted	$ (0.80)	$ (0.72)	$ (2.96)	$ (3.55)
Weighted average shares outstanding, basic and diluted	47,030,820	36,153,634	42,876,737	28,834,303

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 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 be associated with 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 completed the NDA submission in August, ahead of schedule. The FDA accepted the NDA for UGN-102 and assigned a PDUFA goal date of June 13, 2025.

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 LG-UTUC 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. To learn more visit www.urogen.com 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: statements related to UroGen's NDA submission, and expected PDUFA target action date for UGN-102; conclusions from the ENVISION data; the estimated patient population and demographics for LG-IR-NMIBC and UTUC; the potential applications of ICVB-1042 in treating bladder cancer as well as other types of cancers; the ENVISION subgroup analyses providing evidence of UGN-102's potential to become an effective and durable treatment option for patients with recurrent LG-IR-NMIBC, regardless of tumor size or focality; plans for UGN-103 to follow the potential FDA approval and launch of UGN-102; the timing for the planned Phase 3 trial to explore the safety and efficacy of UGN-104 in the first half of 2025; the potential for ICVB-1042 to be potent, fast-replicating, and administered locally to treat cancer; 2025 financial guidance; 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. Words such as "estimate," "believe," "if," "may," "plan," "potential," "suggest," "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; findings from the durability of response endpoint from the ENVISION Phase 3 study may not 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 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 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 September 30, 2024, filed with the SEC on November 6, 2024, and in UroGen's Annual Report on Form 10-K for the year ended December 31, 2024, being filed with the SEC later today, 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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INVESTOR CONTACT:
Vincent Perrone
Senior Director, Investor Relations
vincent.perrone@UroGen.com
609-460-3588 ext. 1093

MEDIA CONTACT:
Cindy Romano
Director, Corporate Communications
cindy.romano@UroGen.com
609-460-3583 ext. 1083

Source: UroGen Pharma Ltd.