UroGen Pharma Reports Third Quarter 2022 Financial Results and Recent Corporate Developments

November 10, 2022

- *Full enrollment of ENVISION Phase 3 pivotal trial with UGN-102 in low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC) expected as soon as the end of November 2022; NDA submission anticipated in first half of 2024*
- *Reported JELMYTO® net product revenue of $16.1 million, an increase of 41% from the same period last year*
- *FDA authorized extension of the in-use period for JELMYTO® admixture from 8 to 96 hours following reconstitution further simplifying workflow for urologists*

PRINCETON, N.J.--(BUSINESS WIRE)--Nov. 10, 2022-- **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, 2022, and provided an overview of recent developments.

“From a consistent vision to pipeline advancement and product commercialization, we remain highly encouraged by the inroads we have made creating a new market in how urologic cancers are treated. Despite slightly lower revenue compared to the previous quarter, JELMYTO’s favorable growth trends, which include a substantial increase of year-over-year net product revenues, new site activations, and recent real-world outcomes data, continues to give us confidence in the LG-UTUC opportunity as we work to replicate the success we’ve had in certain key markets,” said Liz Barrett, President and Chief Executive Officer of UroGen. “We anticipate that the FDA authorization this quarter to extend JELMYTO’s in-use period for the admixture from 8 hours to 96 hours following reconstitution will provide yet another significant opportunity towards driving market adoption and expansion.”

“In parallel, we are rapidly advancing perhaps the most exciting investigational product in our pipeline, UGN-102, which has the potential to transform the treatment of bladder cancer and significantly broaden the utilization of our RTGel™ reverse-thermal hydrogel technology,” continuedMs. Barrett. “ENVISION, our Phase 3 pivotal trial of UGN-102 in LG-IR-NMIBC, is on pace to complete enrollment as soon as the end of the month, bringing us one step closer to realizing UGN-102’s potential as the first non-surgical primary therapeutic to treat this important subset of bladder cancer patients. As we advance each of these programs, as well as our broader portfolio, we continue a disciplined and balanced approach to capital preservation in support of growing our business and maintaining a strong balance sheet.”

Business Highlights:

***Jelmyto* (mitomycin) for pyelocalyceal solution in low-grade Upper Tract Urothelial Cancer (LG-UTUC):**

- Reported net product revenue of JELMYTO for the third quarter 2022 of $16.1 million, compared to $11.4 million in the third quarter of 2021, representing an increase of 41% from the same period last year.
- Activated sites and repeat accounts as of November 1 were 930 and 177, respectively as compared to 893 and 144 on August 1, 2022.
- FDA authorized an extension of the in-use period for JELMYTO admixture from 8 hours to 96 hours following reconstitution. This extension expands access to JELMYTO and gives urologists greater flexibility in choosing when to reconstitute and schedule instillations, including early morning instillation which we estimate is preferred by nearly all prescribing HCPs.
- A multi-center retrospective analysis published in the British Journal of Urology International by Kyle Rose, M.D. Society of Urologic Oncology Fellow, Moffitt Cancer Center, and colleagues showed that 17 of 32 (59%) patients who received JELMYTO via antegrade administration had no evidence of disease at the primary disease evaluation and did not recur at a median follow up of 13 months. Importantly, ureteral stenosis occurred in just 3 (9%) of treated patients. This retrospective analysis concluded that antegrade administration of JELMYTO demonstrated a favorable safety profile, including a low rate of ureteral stenosis and can be administered without general anesthesia.

UGN-102 (mitomycin) for intravesical solution:

- Full enrollment of approximately 220 patients across 90 sites in the single-arm Phase 3 ENVISION pivotal trial of UGN-102, for the treatment of low-grade, intermediate-risk NMIBC is expected to be completed as soon as the end of November 2022.
- Hosted thought-leader webinar on UGN-102 and non-muscle invasive bladder cancers on October 18, 2022, which underscored the magnitude of the patient population and high unmet need in LG-IR-NMIBC.

UGN-301 (zalifrelimab) for intravesical solution:

- UroGen continues to enroll subjects for its first-in-human, novel, multi-arm Phase 1 clinical trial of UGN-301, the Company’s anti-CTLA4- antibody, in high-grade NMIBC.
- This Phase 1 clinical trial will utilize a Master Protocol to evaluate the safety and tolerability of UGN-301 as monotherapy and in combination with other immunomodulators, including UGN-201, the Company’s proprietary toll-like receptor 7

(TLR7) agonist, as well as other potential chemo and/or immune therapies in patients with NMIBC.

Board of Directors Appointments

- Announced the appointment of two new independent members to the Board of Directors: Dr. Leana S. Wen, an emergency medicine physician and public health policy expert, and Dan Wildman, a seasoned executive with more than forty years of Medical Device and Pharmaceutical industry experience developing and commercializing meaningful innovations that changed the standard of care, including Auris Heath's (a division of Ethicon Inc.) Monarch robotic surgery system

Third Quarter 2022 Financial Results:

Jelmyto Revenue: UroGen reported net product revenue of *JELMYTO* for the third quarter 2022 of $16.1 million, compared to $11.4 million in the third quarter of 2021, representing an increase of 41% from the same period last year.

R&D Expense: Research and development expenses for the third quarter 2022 were $13.1 million, including non-cash share-based compensation expense of $0.6 million as compared to $11.9 million, including non-cash share-based compensation expense of $1.0 million, for the same period in 2021.

SG&A Expense: Selling, general and administrative expenses for the third quarter 2022 were $19.1 million, including non-cash share-based compensation expense of $1.8 million. This compares to $21.6 million, including non-cash share-based compensation expense of $4.5 million, for the same period in 2021.

Financing on Prepaid Forward Obligation: UroGen reported non-cash financing expense related to the prepaid forward obligation to RTW Investments of $4.8 million for the third quarter 2022.

Interest Expense on Long-Term Debt: Interest expense related to the up to $100 million term loan facility with funds managed by Pharmakon Advisors was $2.7 million for the third quarter of 2022. As the transaction closed in March 2022, there was no such expense in the third quarter of 2021.

Net Loss: UroGen reported a net loss of $25.8 million, or basic and diluted net loss per ordinary share of $1.13, for the third quarter 2022 as compared to $30.2 million, or basic and diluted net loss per ordinary share of $1.35, for the same period in 2021.

Cash & Cash Equivalents: As of September 30, 2022, cash, cash equivalents and marketable securities totaled $95.9 million. This includes the first $75 million tranche of the up to $100 million term loan facility with funds managed by Pharmakon Advisors, which closed in March 2022.

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 the Company'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.

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

	September 30, 2022 (Unaudited)	December 31, 2021
Cash and cash equivalents and marketable securities	$ 95,911	$ 89,814
Total assets	$ 128,473	$ 119,746
Total liabilities	$ 191,759	$ 111,333
Total shareholders' equity (deficit)	$ (63,286)	$ 8,413

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,	
	2022	2021	2022	2021
Revenue	$ 16,097	$ 11,351	$ 46,265	$ 31,868
Cost of revenue	2,020	1,244	5,391	3,568
Gross profit	14,077	10,107	40,874	28,300
Operating expenses:				
Research and development expenses	13,093	11,923	38,429	34,560
Selling, general and administrative expenses	19,071	21,624	61,204	66,117
Total operating expenses	32,164	33,527	99,633	100,677
Operating loss	(18,087)	(23,440)	(58,759)	(72,377)
Financing on prepaid forward obligation	(4,819)	(6,828)	(16,478)	(9,948)
Interest expense on long-term debt	(2,694)	-	(5,215)	-
Interest and other income	478	57	604	269
Loss before income taxes	$ (25,122)	$ (30,211)	$ (79,848)	$ (82,056)
Income tax expense	(709)	-	(1,066)	(312)
Net loss	$ (25,831)	$ (30,211)	$ (80,914)	$ (82,368)

Net loss per ordinary share basic and diluted	$ (1.13)	$ (1.35)	$ (3.56)	$ (3.69)
Weighted average shares outstanding, basic and diluted	22,798,263	22,380,598	22,711,686	22,318,589

About *Jelmyto*®

Jelmyto (mitomycin) for pyelocalyceal solution, is a drug formulation of mitomycin indicated for the treatment of adult patients with low-grade upper tract urothelial cancer (LG-UTUC). Utilizing the RTGel™ technology platform, UroGen's proprietary sustained release, hydrogel-based formulation, Jelmyto is designed to enable longer exposure of urinary tract tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means. Jelmyto is delivered to patients using standard ureteral catheters or nephrostomy tube. The U.S. FDA previously granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to Jelmyto for the treatment of LG-UTUC. On April 15, 2020, the FDA approved Jelmyto, making it the first drug approved for the treatment of LG-UTUC in adult patients.

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 low-grade intermediate risk NMIBC. Utilizing the RTGelTM Technology Platform, UroGen's proprietary 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. The Company presented results from the Phase 2b OPTIMA II trial in September 2021.

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) as primary chemoablative therapy in patients with low-grade, intermediate-risk NMIBC. The Phase 3 ENVISION trial is expected to enroll approximately 220 patients across 90 sites and study participants will receive six once-weekly intravesical instillations of UGN-102. The planned primary endpoint will evaluate the complete response rate at three months after the first installation, and the key secondary endpoint will evaluate durability over time in patients who achieve complete response at the three-month assessment. Based on discussions with the FDA, and enrollment expected by the end of 2022, assuming positive 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 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 RTGelTM 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 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 implications of the extension of the in-use period for JELMYTO for all stakeholders, including greater flexibility for customers in choosing when to mix and schedule instillations; RTGel's potential to improve the therapeutic profiles of existing drugs; UroGen's sustained release technology making local delivery potentially more effective as compared to other treatment options; the ongoing Phase 3 ENVISION trial for UGN-102, the design and objectives thereof, the number of patients to be enrolled and the timing for completion of enrollment; the design, potential benefits and commercial potential for UGN-102, if approved; plans with respect to a regulatory submission for UGN-102 and the timing thereof; the potential of UGN-102 to significantly broaden the utility of UroGen's RTGel technology; and the ongoing Phase 1 clinical study for UGN-301 and the design, enrollment, and timing thereof, including the evaluation of the safety and tolerability of UGN-301 as monotherapy and in combination with other immunomodulators, including UGN-201. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: clinical trial enrollment challenges that may impact the expected timing of our planned clinical trials, including challenges related to the ongoing COVID-19 pandemic and the Russia-Ukraine conflict; the timing and success of clinical trials and potential safety and other complications thereof; 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 ongoing COVID-19 pandemic; the labeling for any approved product; 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; the availability of the second tranche term loan under the term loan facility with Pharmakon and the ability to satisfy required customary bring-down conditions and deliverables for the second tranche; UroGen's ability to attract or retain key management, members of the board of directors and personnel; and any negative effects on UroGen's business, commercialization and product development plans caused by or associated with COVID-19 or geopolitical events. 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 August 11, 2022, 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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