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

August 11, 2022

- Reported *Jelmyto*® net product revenue of $16.6 million, a 22% increase from the first quarter of 2022, and 28% increase YoY; reaffirmed 2022 full-year revenue guidance of $70-$80 million
- Enrollment of UGN-102 Phase 3 single-arm, ENVISION pivotal trial in subjects with low-grade intermediate risk non-muscle invasive bladder cancer (LG-IR-NBIMC) is ongoing and on track; enrollment completion anticipated before end of 2022
- Progressed first in human multi-arm Phase 1 clinical trial of UGN-301 in high-grade non-muscle invasive bladder cancer (HG-NMIBC)
- Conference call and webcast to be held today at 10:00 AM ET

PRINCETON, N.J.--(BUSINESS WIRE)--Aug. 11, 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 second quarter ended June 30, 2022 and provided an overview of recent developments.

"Positive momentum established last quarter has accelerated into the second quarter of 2022 and we remain confidently on track to meet our anticipated guidance, both financial and in terms of clinical development," said Liz Barrett, President, and Chief Executive Officer of UroGen. "*Jelmyto* adoption continues to grow and real-world evidence supports the use of our medicine for LG-UTUC patients. The use of in-office nephrostomy tube administration has grown and data on the potential benefits of this alternate mode of administration was presented at the American Urological Association meeting in May. Enrollment in the Phase 3 ENVISION pivotal trial of UGN-102 in low-grade, intermediate-risk NMIBC is on pace for completion by year-end, while our Phase 1 clinical study of UGN-301 in high-grade NMIBC is well underway. With intent to provide reassurance in this challenging capital market environment, we are continuously assessing the strategic and efficient deployment of capital across our operations and have made a modest reduction to our operating expense forecast. We remain focused towards our goal of reaching cash flow break-even in 2025 while not compromising the success of *Jelmyto* and UGN-102 as well as our long-term growth potential. We will remain flexible and opportunistic and continuously evaluate new and current levers in the event we find a need to conserve or access additional working capital."

Business Highlights:

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

- Generated net product revenues of $16.6 million for the second quarter of 2022, representing a 22% increase over the first quarter of 2022, and a 28% increase over the same period in 2021.
- Activated sites on August 1 were 893, compared to 857 on May 1, 2022 while repeat accounts on August 1 were 144, compared to 114 on May 1, 2022.
- Nephrostomy tube administration of *Jelmyto* has increased from approximately 20% to approximately 40% of instillations over the past 3 months, benefits of which include avoiding the necessity of medical equipment as well as conferring more flexibility with scheduling and instillation.
- Empirical data of *Jelmyto* instillation via Nephrostomy were presented at the American Urological Association meeting in May by Dr. Kyle Rose demonstrating that 13 of 26 patients examined exhibited a complete response, while another 12 patients had a partial response. Importantly, ureteral stenosis occurred in 4 (15%) patients, compared to 31 (44%) patients in the pivotal OLYMPUS trial, where all participants were administered *Jelmyto* via retrograde administration. There were no severe adverse events reported and no patients had impaired renal function.

UGN-102 (mitomycin) for intravesical solution:

- Enrollment ongoing and active in the single-arm Phase 3 ENVISION pivotal trial of UGN-102 for the treatment of LG-IR-NMIBC. Completion of enrollment continues to be anticipated by the end of 2022.
- ENVISION is similar in design to the previously completed Phase 2b OPTIMA II study which demonstrated a complete response (CR) rate of 65% and probability of remaining in CR 12 months after therapy of 72.5% by Kaplan Meier analysis. UroGen continues to anticipate submitting a New Drug Application (NDA) to the FDA for UGN-102 in 2024.

UGN-301 (zalifrelimab) for intravesical solution:

- UroGen's first-in-human, novel, multi-arm Phase 1 clinical trial of UGN-301, the Company's anti-CTLA4 antibody, in HG-NMIBC is ongoing. 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.

Second Quarter 2022 Financial Results:

Jelmyto Revenue: UroGen reported net product revenues of *Jelmyto* for the second quarter 2022 of $16.6 million, compared to $13.0 million in the

second quarter of 2021.

R&D Expense: Research and development expenses for the second quarter 2022 were $12.6 million, including non-cash share-based compensation expense of $0.7 million as compared to $12.1 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 second quarter 2022 were $20.8 million, including non-cash share-based compensation expense of $2.2 million. This compares to $22.3 million, including non-cash share-based compensation expense of $5.0 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 $5.8 million for the second quarter 2022, compared to $3.1 million for the same period in 2021. The rate applied to cash payments incurred in 2022 is 13% based on $48 million of global net product sales of *Jelmyto* in 2021.

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 $2.2 million for the second quarter of 2022. As the transaction closed in March 2022, there was no such expense in the second quarter of 2021.

Net Loss: UroGen reported a net loss of $26.7 million, or basic and diluted net loss per ordinary share of $1.18, for the second quarter 2022 as compared to $26.2 million, or basic and diluted net loss per ordinary share of $1.17 for the same period in 2021.

Cash & Cash Equivalents: As of June 30, 2022, cash, cash equivalents and marketable securities totaled $112.4 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.

2022 Revenue, Operating Expense and RTW Expense Guidance: The Company reiterates anticipated full year 2022 net product revenues from *Jelmyto* to be in the range of $70 to $80 million. The Company has reduced its anticipated full year 2022 operating expenses to be in the range of $130 to $140 million, including non-cash share-based compensation expense of $10 to $16 million, subject to market conditions. The Company reiterates anticipated full year 2022 non-cash financing expense related to the prepaid obligation to RTW Investments in the range of $22 to $26 million, of which approximately $9.1 to $10.4 million will be paid 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 the Company's financial results and provide a general business update. The webcast can be accessed from the Investors section of the UroGen's website at https://investors.urogen.com/.

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

	June 30, 2022 (Unaudited)	December 31, 2021
Cash and cash equivalents and marketable securities	$ 112,411	$ 89,814
Total assets	$ 146,141	$ 119,746
Total liabilities	$ 187,054	$ 111,333
Total shareholders' equity (deficit)	$ (40,913)	$ 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 June 30,		Six months ended June 30,	
	2022	2021	2022	2021
Revenue	$ 16,604	$ 13,032	$ 30,168	$ 20,517
Cost of revenue	1,846	1,427	3,371	2,324
Gross profit	14,758	11,605	26,797	18,193
Operating expenses:				
Research and development expenses	12,640	12,124	25,336	22,637
Selling, general and administrative expenses	20,833	22,304	42,133	44,493
Total operating expenses	33,473	34,428	67,469	67,130
Operating loss	(18,715)	(22,823)	(40,672)	(48,937)
Financing on prepaid forward obligation	(5,833)	(3,120)	(11,659)	(3,120)
Interest expense on long-term debt	(2,239)	-	(2,521)	-
Interest and other income	128	33	126	212
Loss before income taxes	$ (26,659)	$ (25,910)	$ (54,726)	$ (51,845)
Income tax expense	(32)	(312)	(357)	(312)
Net loss	$ (26,691)	$ (26,222)	$ (55,083)	$ (52,157)
Net loss per ordinary share basic and diluted	$ (1.18)	$ (1.17)	$ (2.43)	$ (2.34)
Weighted average shares outstanding, basic and diluted	22,703,572	22,331,119	22,667,825	22,287,037

About Jelmyto®

Jelmyto (mitomycin) for myelocaliceal 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 LG IR NMIBC

Out of the 80,000 estimated cases of bladder cancer per year in the U.S., approximately 35,000 are low-grade NMIBC patients comprised of both low-risk (approximately 15,000) and intermediate risk (approximately 20,000). These patients face a future of recurrence and additional surgeries. Recurrence in low-grade intermediate-risk NMIBC is pervasive and often underestimated. In patients who recur, approximately 68 percent will experience two or more recurrence episodes throughout the course of their disease, a high and frequent rate in contrast to other non-metastatic cancers. Currently, the only effective primary treatment available is a surgical procedure known as transurethral resection of bladder tumor, or TURBT. Every time TURBT is performed it imposes more burden and serious risks on patients. Approximately 25 percent of patients are not appropriate for TURBT, whether due to physical factors such as age and comorbidities or an unwillingness to undergo surgery.

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: our 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; plans with respect to a regulatory submission for UGN-102 and the timing thereof; the expected benefits of the FDA's expansion of the in-use period for *Jelmyto's* admixture; the ongoing Phase 1 clinical study for UGN-301 and the design, objectives and timing thereof; financial and clinical development guidance for 2022 and our expectations regarding our ability to meet such guidance; our goal and ability to reach cash flow break-even in 2025; our long-term growth potential; the potential of RTGel to improve the 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: clinical trial enrollment challenges that may impact the expected timing of our ongoing and planned clinical trials, including challenges related to the ongoing COVID-19 pandemic and the Russia-Ukraine war; 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; potential safety and other complications from any approved products; 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 ability to satisfy required customary bring down conditions and deliverables for the second tranche of the term loan facility with Pharmakon; 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 May 10, 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 or will be 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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