UroGen Pharma Reports Highest Revenue Quarter and Significant Full Year 2022 Growth and Recent Corporate Developments

March 16, 2023

- *Reported JELMYTO® full year 2022 net product revenues of $64.4 million, an increase of 34% from full year 2021*
- *Completed enrollment of the ENVISION Phase 3 pivotal clinical trial for UGN-102 for LG-IR-NMIBC*
- *Topline data readout from ATLAS clinical study and Complete Response from ENVISION pivotal study expected to be reported mid-year 2023*

PRINCETON, N.J.--(BUSINESS WIRE)--Mar. 16, 2023-- **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, 2022, and overview of recent developments.

"I'm proud of our progress in 2022 and encouraged by our pipeline advancement and expanded access to JELMYTO as we work to fundamentally change the treatment paradigm of urologic cancers," said Liz Barrett, President and Chief Executive Officer of UroGen. "Last year was a marked year for our exciting investigational product, UGN-102, with the potential to transform the treatment of bladder cancer as the only non-surgical therapeutic for LG-IR-NMIBC. As we have shared many times, we believe UGN-102 could be a transformational product, and one that represents a significant opportunity to address the estimated 80,000 patients suffering from LG-IR-NMIBC in the U.S. each year. In 2022, we initiated and completed enrollment of ENVISION, our Phase 3 pivotal study of UGN-102, and more recently shared long-term follow up data from the previously completed OPTIMA II study, which showed a 24.4-month median time to recurrence in evaluable patients who completed the study. Topline data from the ATLAS and ENVISION trials are expected mid-year 2023, and assuming positive results, we remain on track to submit a New Drug Application (NDA) in 2024. Supported by a growing body of real-world outcomes data, we delivered our strongest ever JELMYTO net sales in Q4 helping propel ~34% full-year growth for fiscal 2022. Looking ahead, we remain confident in the long-term success of JELMYTO in low-grade upper tract urothelial cancer (LG-UTUC) and the significant market opportunity of UGN-102, if approved, to drive growth in the years ahead."

Business Highlights:

UGN-102 (mitomycin) for intravesical solution:

- Announced full enrollment in Phase 3 ENVISION pivotal study of UGN-102 for the treatment of LG-IR-NMIBC, which targeted enrollment of ~220 patients across 90 sites. Assuming positive findings, UroGen anticipates submitting an NDA for UGN-102 in 2024. Topline results from the primary endpoint evaluating the complete response rate at 3-months after first instillation are expected mid-year 2023.
- Topline data from the ATLAS trial, the predecessor to ENVISION, is expected mid-year 2023 and will evaluate complete response, duration of response and safety from ~280 patients that completed the trial.
- Shared new long-term follow up data from the Phase 2b OPTIMA II study at the Annual Society of Urologic Oncology (SUO) Meeting demonstrating a median DOR of 24.4 months (10.1 to 30.7 months) with UGN-102 in 15 of 25 evaluable patients.
- Announced preliminary results of a study to assess the feasibility of home instillation of UGN-102. In this study, UGN-102 was suitable to administer at home by a visiting nurse under the supervision of a treating physician and resulted in 75% (n=8) of patients achieving a complete response, defined as no detectable disease 3 months after starting treatment.

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

- JELMYTO® generated net product revenues of $18.1 million and $64.4 million for the fourth quarter and full year 2022, respectfully, compared to $16.2 million and $48.0 million for the same periods in 2021.
- Activated sites on March 1, 2023 were 983, compared to 930 on November 1, 2022, while repeat accounts on March 1, 2023 were 214, compared to 177 on November 1, 2022.
- Highlighted results from the first and largest post-commercial utilization review of JELMYTO published in Urologic Oncology: Seminars and Original Investigations. The study evaluated 132 patients from 15 high-volume academic and community centers and describes several uses of JELMYTO that differ from the pivotal OLYMPUS trial, including for the treatment of large tumors (>3cm in 15% of cases), high grade tumors (9% of cases), ureteral tumors (35% of cases), and as a valuable multimodal adjunct following complete endoscopic ablation. JELMYTO is not approved for the treatment of high-grade upper tract urothelial cancer.
- Shared new long-term follow up data from the OLYMPUS registration trial at the Annual Society of Urologic Oncology (SUO) Meeting demonstrating a median durability of response (DOR) of 28.9 months (14.6 to 47.6 months) with JELMYTO in 16 of 23 evaluable patients.
- Retrospective, multi-center study of 32 patients evaluating the safety of antegrade administration of JELMYTO was published in The British Journal of Urology International showing a favorable safety and tolerability profile for JELMYTO when administered via nephrostomy tube. At a median follow-up of 15 months following initiation of induction therapy, ureteral stenosis occurred in only three (9%) patients, with none having recurrent stenosis at a median 16-month follow-up.

Fourth Quarter and Full Year 2022 Financial Results:

JELMYTO Revenue: UroGen reported JELMYTO net product revenues for the fourth quarter 2022 of $18.1 million, compared to $16.2 million for the same period in 2021. Net product revenues were $64.4 million for the full year 2022, compared to $48.0 million for the full year 2021.

R&D Expense: Research and development expenses for the fourth quarter 2022 were $14.5 million, including non-cash share-based compensation expense of $0.6 million as compared to $13.1 million, including non-cash share-based compensation expense of $0.9 million, for the same period in 2021. Research and development expenses for the full year 2022 were $52.9 million, including non-cash share-based compensation expense of $2.6 million. This compares to $47.6 million, including non-cash share-based compensation expense of $4.0 million, for the full year 2021.

SG&A Expense: Selling, general and administrative expenses for the fourth quarter 2022 were $21.6 million, including non-cash share-based compensation expense of $1.8 million. This compares to $21.4 million, including non-cash share-based compensation expense of $4.5 million, for the same period in 2021. Selling, general and administrative expenses for the full year 2022 were $82.8 million, including non-cash share-based compensation expense of $8.0 million. This compares to $87.5 million, including non-cash share-based compensation expense of $19.1 million for the full year 2021.

Financing on Prepaid Forward Obligation: UroGen reported financing expense related to the prepaid forward obligation to RTW Investments of $5.1 million for the fourth quarter 2022. Financing expense related to the prepaid forward obligation to RTW Investments totaled $21.6 million for the full year 2022. The rate for 2023 will be 13% based on $64.4 million of global net product sales of JELMYTO in 2022.

Net Loss: UroGen reported a net loss of $28.2 million, or basic and diluted net loss per ordinary share of $1.22, for the fourth quarter 2022 as compared to $28.5 million, or basic and diluted net loss per ordinary share of $1.27, for the same period in 2021. UroGen reported a net loss of $109.2 million, or basic and diluted net loss per ordinary share of $4.79, for the full year 2022 versus $110.8 million, or basic and diluted net loss per ordinary share of $4.96, for the full year 2021. Financial results subsequently filed on Form 10-K for the year ended December 31, 2022 may include immaterial changes related to income tax expense, which is still under review due to ongoing assessment of taxes as it relates to the $100 million term loan from funds managed by Pharmakon Advisors.

Cash & Cash Equivalents: As of December 31, 2022, cash, cash equivalents and marketable securities totaled approximately $100 million.

2023 Revenue, Operating Expense and Financing Expense Guidance: UroGen anticipates full year 2023 net product revenues from JELMYTO to be in the range of $76 to $86 million. UroGen anticipates full year 2023 operating expenses in the range of $135 to $145 million, including non-cash share-based compensation expense of $6.0 to $11.0 million, subject to market conditions. UroGen anticipates full year 2023 financing expense related to the prepaid obligation to RTW Investments in the range of $21.0 to $26.0 million, of which approximately $9.8 to $11.1 million will be in cash. Interest only payments on the $100 million term loan facility with funds managed by Pharmakon Advisors will be made quarterly and continue to accrue at a rate of LIBOR (or a replacement benchmark following the cessation of LIBOR in the first half of this year) + 8.25%.

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)

	December 31, 2022 (Unaudited)	December 31, 2021
Cash and cash equivalents and marketable securities	$ 99,963	$ 89,814
Total assets	$ 136,239	$ 119,746
Total liabilities	$ 224,980	$ 111,333
Total shareholders' equity (deficit)	$ (88,741)	$ 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 December 31,		Year ended December 31,	
	2022	2021	2022	2021
Revenue	$ 18,092	$ 16,174	$ 64,357	$ 48,042
Cost of revenue	2,263	1,589	7,654	5,157
Gross profit	15,829	14,585	56,703	42,885
Operating expenses:				
Research and development expenses	14,477	13,082	52,906	47,642
Selling, general and administrative expenses	21,634	21,418	82,838	87,535
Total operating expenses	36,111	34,500	135,744	135,177
Operating loss	(20,282)	(19,915)	(79,041)	(92,292)
Financing on prepaid forward obligation	(5,081)	(7,343)	(21,559)	(17,291)

Interest expense on long-term debt	(3,223)	-	(8,438)	-
Interest and other income	406	(57)	1,010	212
Loss before income taxes	$ (28,180)	$ (27,315)	$ (108,028)	$ (109,371)
Income tax expense(1)	(69)	(1,137)	(1,135)	(1,449)
Net loss(1)	$ (28,249)	$ (28,452)	$ (109,163)	$ (110,820)
Statements of Comprehensive Loss				
Net loss(1)	$ (28,249)	$ (28,452)	$ (109,163)	$ (110,820)
Other comprehensive (loss)				
Unrealized (loss) on investments	48	(29)	(82)	(296)
Comprehensive Loss(1)	$ (28,201)	$ (28,481)	$ (109,245)	$ (111,116)
Net loss per ordinary share basic and diluted	$ (1.22)	$ (1.27)	$ (4.79)	$ (4.96)
Weighted average shares outstanding, basic and diluted	23,088,891	22,433,206	22,806,812	22,347,481

(1)Financial results included in UroGen's Annual Report on Form 10-K for the year ended December 31, 2022 may include immaterial changes related to income tax expense which is still under review due to the ongoing assessment of taxes as it related to the $100 million term loan with funds managed by Pharmakon Advisors.

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. Food and Drug Administration (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 completed target enrollment of 220 patients across 90 sites. 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 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: expected reporting of topline data readout from ATLAS clinical study and complete response from ENVISION pivotal study in mid-year 2023; UroGen's goal to fundamentally change the treatment paradigm of urologic cancers; UGN-102's potential to transform the treatment of bladder cancer as the only non-surgical therapeutic for LG-IR-NMIBC; the estimated patient population for UGN-102; the potential benefits and commercial potential for UGN-102; plans to submit an NDA to the FDA for UGN-102 and the expected timing thereof; confidence in the long-term success of JELMYTO in LG-UTUC and the significant market opportunity of UGN-102 to drive growth in the years ahead; the design, objectives and timing of the Phase 3 ENVISION trial; the expected benefits that may be demonstrated by the Phase 3 ENVISION trial; plans with respect to the treatment and follow up of patients previously enrolled in the Phase 3 ATLAS trial; plans with respect to a regulatory submission for UGN-102; further adoption of JELMYTO; financial guidance for 2023. RTGel's potential to improve the therapeutic profile 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: 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; the ability to obtain regulatory approval within the timeframe expected, or at all; 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) therefor 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; and UroGen's RTGel technology may not perform as expected and UroGen may not successfully develop and receive regulatory approval of any other product that incorporates RTGel technology . In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of UroGen's Form 10-Q for the period ended September 30, 2022, and other filings that UroGen makes with the SEC from time to time (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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