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

May 10, 2022

- *Jelmyto® net product revenue increased 81% over Q1 2021 to $13.6 million*
- *Enrollment of UGN-102 Phase 3 single-arm, ENVISION pivotal trial is ongoing; completion of enrollment expected by end of 2022*
- *Initiated novel, multi-arm Phase 1 clinical trial of UGN-301 in high-grade non-muscle invasive bladder cancer (NMIBC)*
- *Conference call and webcast to be held today at 10:00 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--May 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 first quarter ended March 31, 2022, and provided an overview of recent developments.

"The first quarter of 2022 saw further commercial, clinical and operational progress as we continued to grow patient access to *Jelmyto*, advance our clinical-stage candidates and significantly strengthened our financial position in support of our business," said Liz Barrett, President, and Chief Executive Officer of UroGen. "Thus far in 2022, we have begun enrolling patients in the Phase 3 ENVISION pivotal trial of UGN-102 in low-grade, intermediate-risk NMIBC and initiated a first-in-human, multi-arm Phase 1 clinical study of UGN-301 in high-grade NMIBC. Our accomplishments throughout 2021 and early 2022, coupled with our strengthened balance sheet, position us well to continue to execute on our strategic initiatives as we look to accelerate growth and advance our mission of transforming the treatment paradigm for patients with urothelial cancers."

Business Highlights:

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

- Generated net product revenue of $13.6 million for the first quarter of 2022, representing 81% growth over the first quarter of 2021.
- Continued phased launch of uTRACT patient registry intended to evaluate real-world outcomes of LG-UTUC patients treated with *Jelmyto*, provide insight into long-term treatment benefits, and evaluate its use in clinical practice in the U.S.

UGN-102 (mitomycin) for intravesical solution:

- UroGen began dosing patients in the single-arm Phase 3 ENVISION pivotal trial of UGN-102 for the treatment of low-grade, intermediate-risk NMIBC. The study will enroll approximately 220 patients across 90 sites. Enrollment is expected to be completed 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. Assuming positive findings, UroGen anticipates submitting a New Drug Application (NDA) for UGN-102 in 2024.

UGN-301 (zalifrelimab) for intravesical solution:

- UroGen initiated a 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.
- UGN-301 is in development through a strategic collaboration with The University of Texas MD Anderson Cancer Center and represents the Company's expansion into immunotherapy. The Phase 1 program intends to build upon encouraging nonclinical data showing that intravesical administration of anti-CTLA4 and a TLR agonist leveraging RTGel™ has the potential to improve mortality in the setting of high-grade NMIBC.

First Quarter 2022 Financial Results:

Jelmyto Revenue: UroGen reported net product revenue of *Jelmyto* for the first quarter 2022 of $13.6 million, compared to $7.5 million in the first quarter of 2021.

R&D Expense: Research and development expenses for the first quarter 2022 were $12.7 million, including non-cash share-based compensation expense of $0.7 million as compared to $10.5 million, including non-cash share-based compensation expense of $1.1 million, for the same period in 2021.

SG&A Expense: Selling, general and administrative expenses for the first quarter 2022 were $21.3 million, including non-cash share-based compensation expense of $2.2 million. This compares to $22.2 million, including non-cash share-based compensation expense of $5.1 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 first quarter 2022. The rate applied to cash payments incurred in 2022 is 13% based on $48 million of global net product sales of Jelmyto in 2021.

Net Loss: UroGen reported a net loss of $28.4 million, or basic and diluted net loss per ordinary share of $1.25, for the first quarter 2022 as compared to $25.9 million, or basic and diluted net loss per ordinary share of $1.17, for the same period in 2021.

Cash & Cash Equivalents: As of March 31, 2022, cash, cash equivalents and marketable securities totaled $137.1 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 reiterates anticipated full year 2022 operating expenses in the range of $140 to $160 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 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 commencement of the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call (855) 765-5685 (U.S.) or (615) 247-5916 (International) to listen to the live conference call. The conference ID number for the live call will be 6687048. An archive of the webcast will be available for two weeks on the Company's website.

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

	March 31, 2022	December 31, 2021
Cash and cash equivalents and marketable securities	$ 137,118	$ 89,814
Total assets	$ 165,715	$ 119,746
Total liabilities	$ 182,789	$ 111,333
Total shareholders' equity	$ (17,074)	$ 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 March 31,	
	2022	2021
Revenue	$ 13,564	$ 7,485
Cost of revenue	1,525	897
Gross profit	12,039	6,588
Operating expense:		
Research and development expenses	12,696	10,513
Selling, general and administrative expenses	21,300	22,189
Total operating expense	33,996	32,702
Operating loss	(21,957)	(26,114)
Financing on prepaid forward obligation	(5,826)	-
Interest expense on long-term debt	(282)	-
Other income (expense)	(2)	179
Loss before income taxes	$ (28,067)	$ (25,935)
Income tax expense	(325)	-
Net loss	$ (28,392)	$ (25,935)
Net loss per ordinary share basic and diluted	$ (1.25)	$ (1.17)
Weighted average shares outstanding, basic and diluted	22,631,509	22,242,375

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 RTGel™ 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 RTGel™ 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 ability to execute on our strategic initiatives and continue to execute on our strategic initiatives as we look to accelerate growth and advance our mission of transforming the treatment paradigm for uro-oncology patients; the number of patients expected to be enrolled and the timing for completion of enrollment with respect to our ongoing Phase 3 ENVISION trial; our belief that the design of the Phase 3 ENVISION trial increases our probability of regulatory success; plans with respect to a regulatory submission for UGN-102 and the timing thereof; the ongoing Phase 1 clinical study for UGN-301 and the design, objectives and timing thereof; the availability of the second tranche term loan under the term loan facility; and financial guidance for 2022. 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 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 Form 10-K filed with the SEC on March 21, 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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