UroGen Pharma Reports Third Quarter 2021 Financial Results and Recent Corporate Developments

November 15, 2021

- *Shift to single-arm Phase 3 study for UGN-102 in bladder cancer streamlines pivotal study to form basis of FDA submission*
- *Jelmyto® net product revenue of $11.4 million for the third quarter of 2021; New patient starts in September and October outpace July and August by nearly 60%*
- *Conference call and webcast to be held today at 10:00 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--Nov. 15, 2021-- UroGen Pharma Ltd. (Nasdaq: URGN), a biopharmaceutical company dedicated to building and commercializing novel solutions that treat urothelial and specialty cancers, today announced financial results for the third quarter ended September 30, 2021, and provided an overview of the Company's recent developments.

"The third quarter of 2021 was one of continued progress at UroGen, both clinically and commercially," said Liz Barrett, President and Chief Executive Officer of UroGen. "As we highlighted at our Spotlight Event last week, we will initiate a new single-arm Phase 3 study of UGN-102 in low-grade, intermediate-risk, non-muscle invasive bladder cancer. We believe this new design affords a higher probability of regulatory success for UGN-102 in patients with low-grade IR-NMIBC, while allowing us to remain on-track for a planned NDA submission in 2024." She added, "Additionally, as we approach the close of 2021, we are pleased with the strong demand we're seeing for Jelmyto. September and October marked our highest months ever for both new patient starts and patient enrollment forms. We look forward to a strong start to 2022, as we advance and grow our pipeline while furthering the adoption of Jelmyto with the goal of it becoming the standard of care for patients with low-grade UTUC."

Business Highlights:

Jelmyto (mitomycin) for pyelocalyceal solution:

- Achieved highest-ever Patient Enrollment Forms and New Patient Starts in September and October 2021.
- UroGen generated net product revenue of $11.4 million for the third quarter of 2021, representing over 200% growth over the third quarter of 2020 (first full quarter of launch), this compares to $13.0 million in the second quarter of 2021.
- As of November 1, 2021, 706 sites have been activated, which means they have completed their internal processes and have treated or are ready to treat patients. This represents a 73% increase since August 1, 2021.
- Sites that have treated more than one patient as of November 1, 2021, increased to 86, compared to 63 as of August 1, 2021: an increase of approximately 37%.

UGN-102 (mitomycin) for intravesical solution:

- Following discussions with the U.S. Food & Drug Administration (FDA), the Company announced plans to conduct a new single-arm Phase 3 study of UGN-102 for the treatment of low-grade, intermediate risk non-muscle invasive bladder cancer (LG-IR-NMIBC). The trial, which is expected to initiate in early 2022, will be similar in design to the Company's previous OPTIMA II study. Based on the results of the OPTIMA II study, the Company believes this new trial carries a high probability of demonstrating a significant benefit for patients.
- Based on the planned initiation of this new Phase 3 study of UGN-102, the Company has ceased enrollment in the ATLAS Phase 3 trial of UGN-102 in LG-IR-NMIBC. The Company plans to complete ongoing treatments and follow up for all patients currently enrolled in the therapy arm. Safety data from the ATLAS study is expected to be included in a planned regulatory submission for UGN-102.

UGN-301:

- Preclinical studies conducted to-date suggest that bladder cancer treated with a combination of TLR-7 agonist and an anti-CTLA4 antibody in RTGel, produces improved survival compared to treatment with other checkpoint inhibitors in RTGel, either alone or in combination with UGN-201.
- Non-human primate toxicity studies underway to facilitate the initiation of a multi-arm Phase 1 study of UGN-301 in combination with other agents.
- First-in-human study planned to start in the first half of 2022.

Geographic expansion:

- Initiated a named-patient access program for Jelmyto in France, Germany, Switzerland, Austria and the UK.

Third Quarter 2021 Financial Results:

Jelmyto Revenue: UroGen reported net product revenue of Jelmyto for the third quarter ended September 30, 2021 of $11.4 million. Net product revenue was $31.9 million for the first three quarters of 2021 compared to $3.8 million for the same period in 2020 due to the launch of Jelmyto in June

2020.

R&D Expense: Research and development expenses for the third quarter ended September 30, 2021 were $11.9 million, including non-cash share-based compensation expense of $1.0 million. This compares to $10.2 million, including non-cash share-based compensation expense of $1.5 million, for the same period in 2020. The increase of $1.7 million is primarily attributable to the launch of our Phase 3 ATLAS study for UGN-102 at the end of 2020, and development cost of UGN-301, partially offset by a decrease in R&D expense related to Jelmyto.

SG&A Expense: Selling, general and administrative expenses for the third quarter ended September 30, 2021 were $21.6 million, including non-cash share-based compensation expense of $4.5 million. This compares to $22.1 million, including non-cash share-based compensation expense of $5.2 million, for the same period in 2020. The $0.5 million decrease is primarily attributable to higher launch related commercial spend in 2020.

Financing on Prepaid Forward Obligation: UroGen reported financing expense related to the prepaid forward obligation to RTW Investments of $6.8 million for the third quarter ended September 30, 2021.

Net Loss: UroGen reported a net loss of $30.2 million, or basic and diluted net loss per ordinary share of $1.35, for the third quarter ended September 30, 2021. This compares to $28.8 million, or basic and diluted net loss per ordinary share of $1.31, for the same period in 2020.

Cash & Cash Equivalents: As of September 30, 2021, cash, cash equivalents and marketable securities totaled $110.3 million.

2021 Operating Expense and Revenue Guidance: The Company is reducing its anticipated full year 2021 operating expenses to the range of $137 million to $142 million, from $155 million to $165 million, the new guidance includes non-cash share-based compensation expense of $22 to $25 million, subject to market conditions. In addition, the Company is providing full year 2021 revenue guidance of $47 million to $51 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 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 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 1908609. 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)

	September 30, 2021	December 31, 2020
Cash and cash equivalents and marketable securities	$ 110,280	$ 103,911
Total assets	$ 134,603	$ 122,005
Total liabilities	$ 103,066	$ 25,650
Total shareholders' equity	$ 31,537	$ 96,355

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,	
	2021	2020	2021	2020
Revenue	$ 11,351	$ 3,461	$ 31,868	$ 3,833
Cost of revenue	1,244	309	3,568	357
Gross profit	10,107	3,152	28,300	3,476
Operating expense:				
Research and development expense	11,923	10,211	34,560	34,905
Selling, general and administrative expense	21,624	22,065	66,117	68,056
Total operating expense	33,547	32,276	100,677	102,961
Operating loss	(23,440)	(29,124)	(72,377)	(99,485)
Financing on prepaid forward obligation	(6,828)	-	(9,948)	-
Interest and other income, net	57	308	269	1,527
Loss before income taxes	$ (30,211)	$ (28,816)	$ (82,056)	$ (97,958)
Income tax expense	-	-	312	-
Net loss	$ (30,211)	$ (28,816)	$ (82,368)	$ (97,958)
Net loss per ordinary share basic and diluted	$ (1.35)	$ (1.31)	$ (3.69)	$ (4.52)
Weighted average shares outstanding, basic and diluted	22,380,598	22,058,343	22,318,589	21,657,712

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 non-muscle invasive bladder cancer. 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 ATLAS Trial

ATLAS was a global, open-label, randomized controlled Phase 3 trial designed to assess the efficacy and safety of UGN-102, with or without transurethral resection of bladder tumor (TURBT), versus TURBT alone in patients diagnosed with low-grade intermediate risk non-muscle invasive bladder cancer (LG-IR-NMIBC), defined as 1 or 2 of the following: new or recurrent multifocal bladder tumors, a solitary new or recurrent tumor >3 cm, or LG-IR-NMIBC recurrence in less than 12 months following a prior tumor diagnosis requiring endoscopic surgical resection or ablation.

Patients were randomized 1:1 to either UGN-102 or TURBT. Patients in the UGN-102 arm were treated with six weekly intravesical instillations of UGN-102. At the 3-month time point, patients were assessed for response. Patients who have demonstrated a complete response to either UGN-102 or TURBT, will continue for long-term follow-up for evidence of recurrence. Patients who demonstrate presence of persistent disease at 3-months, in either arm, will undergo a TURBT and then will also continue for long-term follow up for evidence of recurrence. The primary endpoint of the study was disease free survival. On November 10, 2021, the Company announced that, following discussions with the U.S. Food & Drug Administration, it has ceased enrollment in the ATLAS study and plans to initiate a new, single-arm Phase 3 study of UGN-102 in early 2022. All patients enrolled in the treatment arm of ATLAS will continue to receive treatment and undergo follow up.

Learn more about the ATLAS trial at www.clinicaltrials.gov (NCT04688931)

About UroGen Pharma Ltd.

UroGen is a biopharmaceutical company dedicated to building novel 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 belief that the design of our new planned Phase 3 clinical trial for UGN-102 affords a higher probability of regulatory success and that the trial carries a high probability of demonstrating a significant benefit for patients; the expected timing for enrollment with respect to the new Phase 3 clinical trial for UGN-102; plans with respect to the ATLAS Phase 3 clinical trial of UGN-102 and use and reporting of safety data; plans with respect to a regulatory submission for UGN-102; further adoption of Jelmyto; expected timing for clinical trials for UGN-301; and financial guidance for 2021. 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; 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; 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. 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 filed with the SEC on November 15, 2021, 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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