UroGen Pharma Reports Fourth Quarter and Full Year 2020 Financial Results and Recent Corporate Developments

March 18, 2021

- *Achieved Jelmyto® net product revenue of $8.0 million for the fourth quarter of 2020; $11.8 million in first seven months of commercialization*
- *Initiated ATLAS Phase III trial for UGN-102 and actively enrolling patients with low-grade intermediate risk NMIBC*
- *Expanded immuno-oncology pipeline with strategic research collaborations*
- *Strategic funding provides additional capital to support continued Jelmyto launch and UGN-102 program*
- *Conference call and webcast to be held today at 8:30 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--Mar. 18, 2021-- UroGen Pharma Ltd. (Nasdaq: URGN), a biopharmaceutical company dedicated to building and commercializing novel solutions that treat specialty cancers and urologic diseases, today announced financial results for the fourth quarter and full year ended December 31, 2020 and provided an overview of the Company's recent developments.

"2020 was a pivotal year for UroGen with the launch of our first product marking our transition from a clinical to a commercial stage company. Reflecting on our first two full quarters of our Jelmyto launch, we observed strong interest and early uptake from physicians, both in the community and hospital setting," said Liz Barrett, President and Chief Executive Officer of UroGen. "Beyond Jelmyto, we are pleased to have initiated the Phase 3 ATLAS trial for UGN-102 and expect to provide enrollment updates later this year. Additionally, we are looking forward to further realizing the potential of the RTGel™ platform through strategic collaborations, such as our recent immuno-oncology research agreements with leading academic centers."

Business Highlights and Upcoming Milestones:

Jelmyto (mitomycin) for pyelocalyceal solution:

- Achieved net product revenue of $8.0 million for the fourth quarter of 2020. Since the June 1, 2020 launch of Jelmyto®, the first and only FDA approved non-surgical treatment option for adult patients with low-grade upper tract urothelial cancer (LG-UTUC), the Company reported $11.8 million in net product revenue.
- As of March 1, 2021, 250 sites have been activated, which means they have completed their internal processes and have treated or are ready to treat patients; 31 sites have treated more than one patient.
- Effective January 1, 2021, the Company received a permanent and product-specific J-code for Jelmyto from the Centers for Medicare and Medicaid Services (CMS), replacing the previously issued temporary C-code and standardizing and facilitating reimbursement in the hospital outpatient, ambulatory surgery center and physician office settings of care.
- UroGen presented final durability data from the Phase 3 OLYMPUS pivotal trial evaluating Jelmyto in LG-UTUC at the Annual Meeting of the Society of Urologic Oncology. Of the 58% (41/71) of patients who achieved a complete response (CR), 12-month durability of response was estimated at 81.8% by Kaplan-Meier analysis.

UGN-102 (mitomycin) for intravesical solution:

- Initiated ATLAS, the Phase 3 trial of UGN-102 in the fourth quarter of 2020. The ATLAS trial expects to enroll approximately 630 patients and is exploring UGN-102 as a primary non-surgical treatment compared to standard of care – transurethral resection of bladder tumor (TURBT) – in patients diagnosed with low-grade intermediate risk non-muscle invasive bladder cancer (LG-IR-NMIBC).
- Reported top-line final data from the OPTIMA II Phase 2b trial evaluating UGN-102 in patients with LG-IR-NMIBC, showing that 65% of patients receiving UGN-102 (41/63) achieved a CR three months after the start of therapy. In this subset of patients, duration of response at 12 months following treatment initiation (nine months post CR) was estimated by Kaplan-Meier analysis to be 72.5%. The Company expects to present the full data at an upcoming medical meeting and publish in a peer-reviewed journal.

UGN-302 [UGN-201 (imiquimod) TLR 7/8 + UGN-301 (zalifrelimab) CTLA-4]:

- Announced a strategic three-year collaboration agreement with The University of Texas MD Anderson Cancer Center to advance UGN-302, a combination intravesical immunotherapy, which is delivered directly into the bladder, for the treatment of high-grade non-muscle invasive bladder cancer (HG-NMIBC).
- UroGen and MD Anderson expect to progress the UGN-302 program in the first half of 2021, which includes potential non-clinical studies for UGN-301 and UGN-201, as well as potential clinical studies for UGN-201.
- UroGen continues to pursue additional collaborations and partnerships with leading academic institutions, biotech and pharma.

Strategic Funding:

- UroGen and RTW announced a transaction totaling $75 million in funding for UroGen to support the launch of Jelmyto and the development of UGN-102.
- RTW will provide UroGen with an upfront cash payment of $75 million and will receive tiered future payments based on global annual net product sales of Jelmyto and UGN-102, if approved.

Fourth Quarter and Full Year 2020 Financial Results:

Jelmyto Revenue: UroGen reported net product sales of Jelmyto for the fourth quarter ended December 31, 2020 of $8.0 million. Net product sales of Jelmyto for the year ended December 31, 2020 were $11.8 million. Jelmyto was launched on June 1, 2020.

R&D Expense: Research and development expenses for the fourth quarter ended December 31, 2020 were $12.4 million, including non-cash share-based compensation expense of $1.4 million. This compares to $20.1 million, including non-cash share-based compensation expense of $1.9 million, for the same period in 2019. Research and development expenses for the year ended December 31, 2020 were $47.3 million, including non-cash share-based compensation expense of $6.4 million. This compares to $49.3 million, including non-cash share-based compensation expense of $8.3 million, for the full year 2019.

SG&A Expense: Selling, general and administrative expenses for the fourth quarter ended December 31, 2020 were $22.2 million, including non-cash share-based compensation expense of $5.1 million. This compares to $19.7 million, including non-cash share-based compensation expense of $6.2 million, for the same period in 2019. Selling, general and administrative expenses for the year ended December 31, 2020 were $90.2 million, including non-cash share-based compensation expense of $21.6 million. This compares to $60.2 million, including non-cash share-based compensation expense of $21.7 million, for the full year 2019.

Net Loss: UroGen reported a net loss of $30.5 million, or basic and diluted net loss per ordinary share of $1.38, for the fourth quarter ended December 31, 2020. This compares to $39.0 million, or basic and diluted net loss per ordinary share of $1.86, for the same period in 2019. UroGen reported a net loss of $128.5 million, or basic and diluted net loss per ordinary share of $5.90, for the year ended December 31, 2020. This compares to $105.1 million, or basic and diluted net loss per ordinary share of $5.12, for the full year 2019.

Cash & Cash Equivalents: As of December 31, 2020, cash, cash equivalents and marketable securities totaled $103.9 million.

2021 Operating Expense Guidance: The Company anticipates operating expenses in the range of $155 to $170 million, including non-cash share-based compensation expense of $24 to $28 million, subject to market conditions.

Conference Call & Webcast Information:

Members of UroGen's management team will host a live conference call and webcast today at 8:30 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 9043018. 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)

	December 31, 2020	December 31, 2019
Cash and cash equivalents and marketable securities	$ 103,911	$ 195,632
Total assets	$ 122,005	$ 202,388
Total liabilities	$ 25,650	$ 22,086
Total shareholders' equity	$ 96,355	$ 180,302

UROGEN PHARMA LTD.
CONDENSED CONSOLIDATED STATEMENTS OF OPERATIONS AND COMPREHENSIVE LOSS
(U.S. dollars in thousands, except share and per share data)

	Three months ended December 31,		Year ended December 31,	
	2020	2019	2020	2019
Revenues, net	$ 7,966	$ —	$ 11,799	$ 18
Cost of revenues	652	—	1,009	—
Gross profit	7,314	—	10,790	18
Operating expenses:				
Research and development expenses	12,405	20,094	47,310	49,297
Selling, general and administrative expenses	22,163	19,745	90,219	60,199
Total operating expenses	34,568	39,839	137,529	109,496

Operating loss	(27,254)	(39,839)	(126,739)	(109,478)
Interest and other income, net	102	866	1,629	4,332
Loss before income taxes	(27,152)	(38,973)	(125,110)	(105,146)
Income tax expense	3,374	—	3,374	—
Net loss	$ (30,526)	$ (38,973)	$ (128,484)	$ (105,146)
Net loss per ordinary share basic and diluted	$ (1.38)	$ (1.86)	$ (5.90)	$ (5.12)
Weighted average shares outstanding, basic and diluted	22,146,581	20,988,930	21,780,826	20,528,727

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.

JELMYTO® and UroGen® are registered trademarks of UroGen Pharma, Ltd.

©2021 UroGen Pharma, Inc. All rights reserved.

JEL-PT-ISI-002

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 2b 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 reported topline interim results from the Phase 2b OPTIMA II trial in May 2020 and initiated a Phase 3 study to further investigate UGN-102 in the treatment of this condition in December 2020.

About the Phase 3 ATLAS Trial

ATLAS is an 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. The trial is anticipated to enroll approximately 630 patients in over 100 clinical sites in the U.S., Europe and Israel.

Patients will be randomized 1:1 to either UGN-102 or TURBT. Patients in the UGN-102 arm will be treated with six weekly intravesical instillations of UGN-102. At the 3-month time point, patients will be 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 is disease free survival.

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 specialty cancers and urologic diseases 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. UroGen's first commercial product, and investigational treatment UGN-102 (mitomycin) for intravesical solution for patients with low-grade non-muscle invasive bladder cancer, 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: the expected timing of enrollment updates with respect to the Phase 3 ATLAS trial for UGN-102; UroGen's ability to realize the potential of the RTGel™ platform through strategic collaborations; the expected timing of presentation of, and publication of, the full data set from the OPTIMA II Phase 2b trial for UGN-102; the potential initiation of the Phase I program for UGN-302 in the first half of 2021; the potential initiation of nonclinical studies of checkpoint inhibitors combined with RTGel in GBM; the ability of UroGen to enter into additional collaborations and partnerships; the potential of UroGen's proprietary RTGel technology platform to improve therapeutic profiles of existing drugs; and financial guidance for 2021. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: 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-K filed with the SEC on March 18, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20210318005462/en/

INVESTOR CONTACTS:
Sara Blum Sherman
Head of Investor Relations
investors@urogen.com
609-467-4975

Lee Roth
lroth@burnsmc.com
212-213-0006

MEDIA CONTACT:
Eric Van Zanten
Senior Director, Communications
Eric.VanZanten@urogen.com
610-529-6219

Source: UroGen Pharma Ltd.