UroGen Pharma Reports First Quarter 2020 Financial Results and Recent Corporate Developments

May 7, 2020

Received U.S. FDA Approval for Jelmyto™, the First and Only Non-Surgical Treatment for Patients with Low-Grade Upper Tract Urothelial Cancer (LG-UTUC)

Jelmyto Launch on Track for June 1, 2020

Reported UGN-102 Phase 2b Trial Interim Data with 65% Complete Response at Three Months and 85% Durability at Nine Months in Low-Grade Non-Muscle Invasive Bladder Cancer

Conference Call and Webcast to be held Today at 8:30 AM ET

PRINCETON, N.J.--(BUSINESS WIRE)--May 7, 2020-- 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 first quarter ended March 31, 2020 and provided an overview of the Company's recent developments.

"The first quarter of 2020 was marked by flawless execution by our team as we prepared for the landmark FDA approval of Jelmyto, the first and only non-surgical treatment for patients with low-grade upper tract urothelial cancer. It has been our mission to pioneer novel treatments for patients in areas of unmet need, and our team has developed innovative solutions to engage key stakeholders so we can bring this effective, kidney-sparing therapy to patients who have been waiting for new treatment options," said Liz Barrett, President and Chief Executive Officer of UroGen. "Our momentum continues as we advance our portfolio of product candidates, highlighted by our recent UGN-102 data readout for the treatment of patients with low-grade intermediate-risk non-muscle invasive bladder cancer, a disease associated with high rates of recurrence and the need for repetitive surgical intervention. The recently published positive interim UGN-102 data, combined with pivotal data for Jelmyto, further supports our confidence in the broad potential of our pipeline. We remain on track to initiate a pivotal Phase 3 study later this year."

Recent Highlights and Upcoming Milestones

Jelmyto (mitomycin) for pyelocalyceal solution, formerly known as UGN-101, for adult patients with low-grade upper tract urothelial cancer (LG-UTUC)

- UroGen announced the U.S. FDA granted approval of Jelmyto for the treatment of patients with LG-UTUC on April 15, 2020. Jelmyto is the first and only FDA approved non-surgical treatment option for patients with LG-UTUC.
- The most commonly reported adverse events (≥ 20%) were ureteric obstruction, flank pain, urinary tract infection, hematuria, renal dysfunction, fatigue, nausea, abdominal pain, dysuria, and vomiting. No treatment-related deaths occurred.
- *The Lancet Oncology* published pivotal results from the Phase 3 OLYMPUS trial, reporting that 59% of LG-UTUC patients treated with Jelmyto achieved a Complete Response (CR). Durability at 12-months (based on interim data) was estimated to be 84% by Kaplan-Meier analysis.
- At the 12-month time point for assessment of durability, 19 patients remained in CR, seven had experienced recurrence of disease, nine patients continued to be followed for the 12-month duration of response, and median duration of response was not reached as of the FDA-approval date.

Jelmyto Commercial Readiness

- The Company expects to commence the official commercial launch of Jelmyto on June 1, 2020.
- To maximize engagement with health care professionals and key stakeholders in this current environment, the Company has developed innovative solutions, including a virtual platform, to allow for an effective launch.
- The team has completed the application for submission of the C-code, and submission of the J-code application to CMS is in process. The company continues to expect a C-code will be secured by October and a J-code by the end of the year.

UGN-102 (mitomycin) for intravesical solution for patients with low-grade intermediate risk non-muscle invasive bladder cancer (LG-IR-NMIBC)

- UroGen announced a positive interim data analysis of UGN-102 in patients with LG-IR-NMIBC, featured in a late-breaking abstract published in the April Supplement to *The Journal of Urology.*
- The Phase 2b OPTIMA II trial demonstrated a CR rate at three months following onset of treatment of 65% (41/63 patients). In this subset of patients, 31/32 patients (97%) and 17/20 patients (85%) remained free of disease at six- and nine-months follow-up, respectively.
- The most commonly reported adverse events seen to date (≥ 10%) were reported as mild to moderate and include dysuria, hematuria, urinary frequency, fatigue, urgency and urinary tract infection.
- The detailed results presentation from the Phase 2b OPTIMA II trial will be available online via the American Urological Association (AUA) in mid-May 2020.
- The Company remains on track to initiate a pivotal Phase 3 trial in 2H 2020.
- There are no drugs currently approved by the FDA as first-line treatment for LG-IR-NMIBC. UGN-102 has the potential to

provide a non-surgical treatment alternative for approximately 80,000 patients diagnosed with LG-IR-NMIBC in the United States alone.

First Quarter 2020 Financial Results; 2020 Guidance

- As of March 31, 2020, cash, cash equivalents and marketable securities totaled $159.2 million, excluding restricted cash.
- Research and development expenses for the three months ended March 31, 2020 were $16.6 million, including non-cash share-based compensation expense of $1.9 million, and a one-time payment of $6.6 million to unwind our obligation to the IIA.
- Selling, general and administrative expenses for the three months ended March 31, 2020 were $22.0 million, including non-cash share-based compensation expense of $5.7 million.
- UroGen reported a net loss of $37.8 million, or basic and diluted net loss per ordinary share of $1.79, for the three months ended March 31, 2020.
- The 2020 financial guidance set forth during the Company's full year and fourth quarter 2019 quarterly call on March 2, 2020 remains the same based on current business goals and anticipated activities.

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 4575088. An archive of the webcast will be available for two weeks on the Company's website.

UROGEN PHARMA LTD.
CONDENSED CONSOLIDATED BALANCE SHEETS
(U.S. dollars in thousands, except share and per share data)
(Unaudited)

	March 31, 2020	December 31, 2019
Assets		
CURRENT ASSETS:		
Cash and cash equivalents	$ 26,468	$ 49,688
Marketable Securities	92,623	97,389
Restricted cash	1,223	523
Prepaid expenses and other current assets	1,552	1,034
TOTAL CURRENT ASSETS	121,866	148,634
NON-CURRENT ASSETS:		
Property and equipment, net	991	977
Restricted deposit	223	223
Right of use asset	3,433	3,735
Marketable Securities	40,137	48,555
Other non-current assets	279	264
TOTAL ASSETS	$ 166,929	$ 202,388
Liabilities and Shareholders' equity		
CURRENT LIABILITIES:		
Accounts payable and accrued expenses	$ 8,500	$ 11,186
Employee related accrued expenses	4,075	6,711
Other current liabilities	1,632	1,585
TOTAL CURRENT LIABILITIES	14,207	19,482
NON-CURRENT LIABILITIES:		
Long-term lease liability	2,268	2,604
TOTAL LIABILITIES	16,475	22,086
TOTAL SHAREHOLDERS' EQUITY	150,454	180,302
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 166,929	$ 202,388

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,	
	2020	2019
REVENUES	$ —	$ —
COST OF REVENUES	—	—
GROSS PROFIT	—	—
OPERATING EXPENSES:		
Research and development expenses	16,588	9,726
Selling, general and administrative expenses	21,973	12,707
OPERATING LOSS	(38,561)	(22,433)
INTEREST AND OTHER INCOME, NET	768	989
NET LOSS	$ (37,793)	$ (21,444)
STATEMENT OF COMPREHENSIVE LOSS		
NET LOSS	$ (37,793)	$ (21,444)
OTHER COMPREHENSIVE INCOME:		
UNREALIZED GAIN ON MARKETABLE SECURITIES	35	—
COMPREHENSIVE LOSS	$ (37,758)	$ (21,444)
NET LOSS PER ORDINARY SHARE, BASIC AND DILUTED	$ 1.79	$ 1.11
WEIGHTED AVERAGE SHARES OUTSTANDING, BASIC AND DILUTED	21,158,161	19,340,082

About the Phase 3 OLYMPUS Trial

OLYMPUS (Optimized DeLivery of Mitomycin for Primary UTUC Study) is an open-label, single-arm Phase 3 clinical trial of UGN-101, *Jelmyto* (mitomycin) for pyelocalyceal solution, to evaluate the safety, tolerability and tumor ablative effect of *Jelmyto* in patients with low-grade UTUC. Seventy-one patients were treated at clinical sites across the United States and Israel. Study participants were treated with six weekly instillations of *Jelmyto* administered via a standard catheter. Four to six weeks following the last instillation, patients underwent a Primary Disease Evaluation (PDE) to determine Complete Response (CR), the primary endpoint of the study. PDE involved a ureteroscopy and wash cytology, a standard microscopic test of cells obtained from the urine to detect cancer and for cause biopsy. Patients who achieved a CR at the PDE timepoint were then followed for up to 12 months to determine the durability of response with *Jelmyto*.

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.

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.

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: side pain, urinary tract infection, blood in your urine, kidney problems, tiredness, nausea, stomach pain, trouble with urination, vomiting, low red blood cell count, frequent urination, itching, chills, and fever.

Please see JELMYTO Full Prescribing Information, including the Patient Information at www.jelmyto.com.

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 approved *Jelmyto™* (mitomycin) for pyelocalyceal solution, and pipeline treatment UGN-102 (mitomycin) for intravesical solution are designed to ablate tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade upper tract urothelial cancer and low-grade non-muscle invasive bladder cancer, respectively. UroGen is headquartered in Princeton, NJ with operations in Israel. Visit www.urogen.com to learn more or follow us on Twitter, @UroGenPharma.

COVID-19 Pandemic Potential Impact

UroGen continues to gather information in this very fluid and rapidly-evolving environment regarding the potential impact of the COVID-19 pandemic on our Company, however, we are not currently able to quantify or predict with any certainty the overall scope of impact on UroGen, or any resulting delays in the availability of Jelmyto™ (mitomycin) for pyelocalyceal solution. Our primary focus is on the health and well-being of patients, caregivers, and UroGen employees at this critical juncture.

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 potential of *Jelmyto*™to transform the treatment of LG-UTUC; the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs; the planned commercial launch of Jelmyto on June 1, 2020, including UroGen's virtual launch plans; UroGen's plans to secure coding and reimbursement for Jelmyto; the potential of UGN-102 for LG NMIBC; the initiation of a Phase 3 study of UGN-102 in LG-NMIBC in 2H 2020; and financial guidance for the remainder of 2020. 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 achieving commercial readiness for the launch of a new product; 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 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 May 7, 2020, 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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