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

November 9, 2020

Top-line Results from UGN-102 OPTIMA II Phase 2b Trial Expected by Year End 2020; On Track to Initiate Phase 3 ATLAS Trial by Year End

Achieved Jelmyto® Net Product Revenue of $3.5 Million in First Full Quarter of Commercialization

Management Team Expanded to Drive Platform Expansion and Growth Strategies

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

PRINCETON, N.J.--(BUSINESS WIRE)--Nov. 9, 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 third quarter ended September 30, 2020 and provided an overview of the Company's recent developments.

"We are excited to see the continued interest and uptake of Jelmyto for patients with low-grade upper tract urothelial cancer in the first full quarter of launch, with a steady increase in new patients and providers as well as positive reimbursement trends. Additionally, we are pleased with the feedback we are receiving from urologists who see this treatment as a novel solution to avoid surgical intervention with a positive impact on patient outcomes," said Liz Barrett, President and Chief Executive Officer of UroGen. "With our second product candidate, UGN-102, soon to enter a pivotal trial and a recently enhanced management team, we look forward to continuing our mission of bringing novel solutions to patients with specialty cancers and urologic diseases."

Business Highlights and Upcoming Milestones:

Jelmyto (mitomycin) for pyelocalyceal solution:

- 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 has expanded prescriber usage, broadened awareness and increased coverage by commercial plans and Medicare.
- As of November 1, 2020, 165 sites have been activated, which means they have completed their internal processes and have treated or are ready to treat patients; 13 sites have treated more than one patient.
- The Company has received its C-Code, effective October 1, 2020, and continues to expect receipt of a J-Code in January 2021.
- Final durability data from the Phase 3 OLYMPUS pivotal trial evaluating Jelmyto (UGN-101) in LG-UTUC is expected to be presented at a medical meeting by the end of the year.

UGN-102:

- Top-line final data from the OPTIMA II Phase 2b trial evaluating UGN-102 in patients with low-grade intermediate risk non-muscle invasive bladder cancer (LG-IR-NMIBC) is expected by the end of the year.
- The Company remains on track to initiate ATLAS, a pivotal Phase 3 trial of UGN-102 by year end 2020. The trial will enroll approximately 600 patients and explore UGN-102 as a non-surgical treatment alternative compared to standard of care – transurethral resection of bladder tumor (TURBT) – in patients diagnosed with LG-IR-NMIBC. There are no drugs currently approved by the FDA as first-line treatment for LG-IR-NMIBC, a difficult-to-treat disease with approximately 80,000 addressable patients per year in the U.S. alone.

UGN-302 (UGN-201 (TLR 7/8) + UGN-301 (CTLA-4)):

- UroGen expects to commence the Phase I program for UGN-302 in the first half of 2021.
- The Company is initially targeting high-grade non-muscle invasive bladder cancer (HG-NMIBC) and has generated encouraging non-clinical data in a murine model suggesting treatment with UGN-201 and a CTLA-4 may result in improved survival and decreased tumor size.
- Intravesical delivery may have the potential to mitigate certain systemic side effects and adverse events associated with systemic CTLA-4 antibody administration.

Executive Team Updates:

- In September 2020, UroGen announced the appointment of three new executives to the Company's Executive Leadership Team:
  - Molly Henderson named Chief Financial Officer
  - Jason Smith named General Counsel and Chief Compliance Officer
  - Polly A. Murphy, DVM, Ph.D., named Chief Business Officer

- These new leaders bring extensive oncology and industry experience to UroGen, and the Company believes it is well positioned to drive future commercialization growth, pipeline development and platform expansion.

Third Quarter 2020 Financial Results:

Jelmyto Revenue: UroGen reported net product revenue of Jelmyto for the third quarter ended September 30, 2020 of $3.5 million.

R&D Expense: Research and development expenses for the third quarter ended September 30, 2020 were $10.2 million, including non-cash share-based compensation expense of $1.5 million. This compares to $9.5 million, including non-cash share-based compensation expense of $2.1 million, for the same period in 2019. Research and development expenses for the nine months ended September 30, 2020 were $34.9 million, including non-cash share-based compensation expense of $5.0 million. This compares to $29.2 million, including non-cash share-based compensation expense of $6.4 million, for the same period in 2019.

SG&A Expense: Selling, general and administrative expenses for the third quarter ended September 30, 2020 were $22.1 million, including non-cash share-based compensation expense of $5.2 million. This compares to $14.0 million, including non-cash share-based compensation expense of $5.2 million, for the same period in 2019. Selling, general and administrative expenses for the nine months ended September 30, 2020 were $68.1 million, including non-cash share-based compensation expense of $16.5 million. This compares to $40.5 million, including non-cash share-based compensation expense of $15.5 million, for the same period in 2019.

Net Loss: UroGen reported a net loss of $28.8 million, or basic and diluted net loss per ordinary share of $1.31, for the third quarter ended September 30, 2020. This compares to $22.3 million, or basic and diluted net loss per ordinary share of $1.06, for the same period in 2019. UroGen reported a net loss of $98.0 million, or basic and diluted net loss per ordinary share of $4.52, for the nine months ended September 30, 2020. This compares to $66.2 million, or basic and diluted net loss per ordinary share of $3.25, for the same period in 2019.

Cash & Cash Equivalents: As of September 30, 2020, cash, cash equivalents and marketable securities totaled $125.5 million, excluding restricted cash.

2020 Operating Expense Guidance: The Company provided an update to previously issued guidance for 2020. UroGen now expects 2020 total operating expense in the range of $138 to $143 million, including non-cash share-based compensation expense of $25 to $29 million, subject to market conditions. Other non-operating income for 2020 is anticipated to be approximately $2.0 million.

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 2146176. An archive of the webcast will be available for 30 days on the Company's website.

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

	September 30, 2020	December 31, 2019
Assets		
Current assets:		
Cash and cash equivalents	$ 24,565	$ 49,688
Marketable securities	95,058	97,389
Restricted cash	1,224	523
Accounts receivable	2,810	-
Inventory	1,346	-
Prepaid expenses and other current assets	4,335	1,034
Total current assets	129,338	148,634
Non-current assets		
Property and equipment, net	1,725	977
Restricted deposit	223	223
Right of use asset	2,652	3,735
Marketable securities	5,900	48,555
Other non-current assets	55	264
TOTAL ASSETS	$ 139,893	$ 202,388
Liabilities and Shareholders' equity		
Current liabilities:		
Accounts payable and accrued expenses	$ 8,492	$ 11,186
Employee related accrued expenses	7,791	6,711
Other current liabilities	1,335	1,585
Total current liabilities	17,618	19,482
Non-current liabilities		

Long-term lease liability	1,748	2,604
TOTAL LIABILITIES	19,366	22,086
TOTAL SHAREHOLDERS' EQUITY	120,527	180,302
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 139,893	$ 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 September 30,		Nine months ended September 30,	
	2020	2019	2020	2019
Revenues	$ 3,461	$ —	$ 3,833	$ 18
Cost of revenues	309	—	357	—
Gross profit	3,152	—	3,476	18
Operating expenses:				
Research and development expenses	10,211	9,481	34,905	29,203
Selling, general and administrative expenses	22,065	13,972	68,056	40,454
Operating loss	(29,124)	(23,453)	(99,485)	(69,639)
Interest and other income, net	308	1,201	1,527	3,466
NET LOSS	$ (28,816)	$ (22,252)	$ (97,958)	$ (66,173)
STATEMENTS OF COMPREHENSIVE LOSS				
Net loss	$ (28,816)	$ (22,252)	$ (97,958)	$ (66,173)
Other comprehensive income				
Unrealized (loss) gain on marketable securities	(268)	22	216	303
COMPREHENSIVE LOSS	$ (29,084)	$ (22,230)	$ (97,742)	$ (65,870)
Net loss per ordinary share basic and diluted	$ (1.31)	$ (1.06)	$ (4.52)	$ (3.25)
Weighted average shares outstanding, basic and diluted	22,058,343	20,916,780	21,657,712	20,373,070

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.

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 intends to begin a pivotal study to further investigate UGN-102 in the treatment of this condition by year end 2020.

About the Phase 2b OPTIMA II Trial

OPTIMA II (OPTimized Instillation of Mitomycin for Bladder Cancer Treatment) is an open-label, single-arm, multi-center Phase 2b clinical trial of investigational agent UGN-102 (mitomycin) for intravesical solution to evaluate its safety and efficacy in patients with low-grade non-muscle invasive bladder (LG NMIBC) cancer at intermediate risk of recurrence. Intermediate risk is defined as one or two of the following: multiple tumors, solitary tumor >3 cm, or recurrence (≥ 1 occurrence of LG NMIBC within one year of the current diagnosis).

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 product, Jelmyto (mitomycin) for pyelocalyceal solution, and investigational 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

While it is not possible at this time to estimate the impact that COVID-19 could have on our business, the COVID-19 pandemic and mitigation measures have had and may continue to have an adverse impact on global economic conditions, which could have an adverse effect on our business and financial condition, including impairing our ability to raise capital when needed. The continued spread of COVID-19 and the measures taken by various governments could disrupt the supply chain of material needed for our product candidates and our approved product, Jelmyto, interrupt healthcare services, delay coverage decisions from Medicare and third party payors, delay ongoing and planned clinical trials involving our product candidates and have a material adverse effect on our business, financial condition and results of operations. 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 UroGen's proprietary RTGel technology platform to improve therapeutic profiles of existing drugs; UroGen's plans to secure coverage, coding and reimbursement for *Jelmyto*; the potential of UGN-102 for LG NMIBC; the potential initiation of the Phase 3 ATLAS study of UGN-102 in LG-NMIBC by the end of 2020; the potential timing of top-line results from the UGN-102 OPTIMA II Phase 2b Trial; the expected presentation of Phase 3 OLYMPUS final durability data by the end of 2020; the potential initiation of the Phase I program for UGN-302 in the first half of 2021; the potential for UGN-302 to mitigate certain systemic side effects and adverse events associated with systemic CTLA-4 antibody administration; and financial guidance for 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 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 August 10, 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.

View source version on businesswire.com: https://www.businesswire.com/news/home/20201109005290/en/

INVESTOR CONTACT:
Sara Blum Sherman
Head of Investor Relations
sara.sherman@urogen.com
609-467-4975

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

Source: UroGen Pharma Ltd.