UroGen Pharma Reports Third Quarter 2019 Financial Results and Recent Corporate Developments

November 12, 2019

Completed Submission of New Drug Application (NDA) to U.S. FDA for UGN-101 in Patients with Low-Grade Upper Tract Urothelial Cancer (LG UTUC)

Exclusive Worldwide License Agreement with Agenus to Advance Treatment of High-Grade Urinary Tract Cancers in Combination with UGN-201

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

NEW YORK--(BUSINESS WIRE)--Nov. 12, 2019-- UroGen Pharma Ltd. (Nasdaq:URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in uro-oncology, today announced financial results for the third quarter ended September 30, 2019 and provided an overview of the Company's recent developments.

"On behalf of the UroGen team, we are very pleased to announce that we have completed the New Drug Application (NDA) submission for UGN-101 to the FDA. This is a very important milestone for UroGen and for patients with low-grade UTUC as UGN-101 has the potential to bring the first primary chemoablation drug to these patients. I am proud of the UroGen team and investigators who have helped to make this key milestone a reality," said Liz Barrett, President and Chief Executive Officer of UroGen. "Beyond UGN-101, we have taken important steps to build a company that can deliver long-term sustainable growth through our focus on high unmet need areas utilizing both local delivery and novel medicines. We've demonstrated that relentless commitment with our recently announced positive complete response data for investigational agent UGN-102 in patients with intermediate risk LG NMIBC, and our exclusive collaboration with Agenus to advance therapies for high-grade urinary tract cancers. We look forward to a strong future for UroGen."

Recent Highlights and Upcoming Milestones

- **UGN-101** (mitomycin gel) for instillation for patients with low-grade upper tract urothelial cancer (LG UTUC):
  - The Company completed its NDA submission to the FDA, and the FDA has a 60-day filing review period to determine whether the NDA submission is complete. The FDA previously granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to UGN-101 for the treatment of LG UTUC. UGN-101 is eligible for Priority Review and the Company is planning for anticipated approval and launch in 1H 2020. UGN-101 plans for launch readiness are on track for January 2020.
  - The NDA submission is supported by positive data from the pivotal Phase 3 OLYMPUS clinical trial.
  - UroGen also initiated a retreatment protocol as an extension to OLYMPUS to evaluate the efficacy and safety of retreatment with UGN-101 in patients from the trial who initially achieved a CR and were subsequently found to have a documented recurrence of LG UTUC during follow-up.

- **UGN-102** (mitomycin gel) in development for intravesical instillation for patients with intermediate risk low-grade non-muscle invasive bladder cancer (LG NMIBC):
  - UroGen recently presented interim results from the single-arm, open-label Phase 2b OPTIMA II trial of investigational UGN-102 for patients with intermediate risk LG NMIBC. LG NMIBC is defined as those with one or two of the following criteria: multifocal disease, large tumors and rapid rates of recurrence.
    - In an interim analysis, 63% (20/32) of patients from the Phase 2b OPTIMA II trial achieved a CR. Patients will continue to be followed with 12-month durability to be reported at a later date.
    - The most common treatment emergent adverse events (TEAEs) included dysuria, pollakiuria, fatigue, hematuria and urinary tract infection. The majority were characterized as mild or moderate in severity and transient.
  - The trial completed enrollment of 63 patients ahead of schedule at clinical sites across the U.S. and Israel. The Company intends to initiate a pivotal Phase 3 trial in 2020 following a planned discussion with the FDA in 1Q 2020.
  - This is a patient population whereby the current standard of care, transurethral resection of bladder tumor, or TURBT, is used repeatedly to address chronic recurrence of disease. These patients experience what can be viewed as a form of surgical failure and many undergo multiple surgical procedures during life to "manage" bladder cancer recurrences.
  - There are no drugs currently approved by the FDA as first-line treatment for intermediate risk LG NMIBC. UGN-102 has the potential to provide a non-surgical alternative for the treatment of chronic relapse for approximately 80,000 patients characterized as intermediate risk.

- **Pipeline Advancement:**
  - **UGN-201** (TLR 7/8 agonist) for patients with high-grade NMIBC**:**
    - UroGen is exploring the utility of UGN-201 in the context of novel combinatorial immunotherapy for NMIBC. Nonclinical data suggests an efficacy signal when UGN-201 is administered locally with anti-CTLA4 antibody in a murine model of high-grade bladder cancer. The Company plans to optimize combinations and advance

into human studies.

- Exclusive Worldwide License Agreement with **Agenus Inc.** to Advance Treatment of High-Grade Urinary Cancers
  - UroGen entered into an exclusive worldwide license agreement with Agenus to develop and commercialize zalifrelimab (AGEN1884, anti-CTLA-4 antibody) in combination with UGN-201 for the treatment of urinary tract cancers via intravesical delivery. Zalifrelimab (AGEN1884, anti-CTLA-4 antibody) is currently being evaluated by Agenus as a monotherapy in PD-1 refractory patients.
  - The initial indication for development will be high-grade NMIBC.
  - UroGen will be responsible for all development and commercialization activities.

Third Quarter 2019 Financial Results; 2019 Guidance

- As of September 30, 2019, cash, cash equivalents and marketable securities totaled $221.7 million.
- Research and development expenses for the three months ended September 30, 2019 were $9.5 million, including non-cash share-based compensation expense of $2.1 million. Research and development expenses for the nine months ended September 30, 2019 were $29.2 million, including non-cash share-based compensation expense of $6.4 million.
- General and administrative expenses for the three months ended September 30, 2019 were $14.0 million, including non-cash share-based compensation expense of $5.2 million. General and administrative expenses for the nine months ended September 30, 2019 were $40.5 million, including non-cash share-based compensation expense of $15.5 million.
- The Company reported a net loss of $22.3 million, or basic and diluted net loss per ordinary share of $1.06, for the three months ended September 30, 2019. The Company reported a net loss of $66.2 million, or basic and diluted net loss per ordinary share of $3.25, for the nine months ended September 30, 2019.
- Including the recently announced Agenus deal, the Company is still on track to end the year with a net loss for the year in the range of $100 to $110 million, which is expected to include non-cash stock-based compensation expense in the range of $28 to $30 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 (888) 771-4371 (U.S.) or (847) 585-4405 (International) to listen to the live conference call. The conference ID number for the live call will be 49159339. 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)

	September 30, 2019	December 31, 2018
Assets		
CURRENT ASSETS:		
Cash and cash equivalents	$ 66,147	$ 101,318
Marketable Securities	92,046	—
Restricted deposit	523	253
Prepaid expenses and other current assets	1,160	672
TOTAL CURRENT ASSETS	159,876	102,243
NON-CURRENT ASSETS:		
Property and equipment, net	893	948
Restricted deposit	55	51
Marketable Securities	63,506	—
Other non-current assets	2,423	317
TOTAL ASSETS	$ 226,753	$ 103,559
Liabilities and Shareholders' equity		
CURRENT LIABILITIES:		
Accounts payable and accrued expenses	$ 7,880	$ 8,540
Employee related accrued expenses	5,082	4,925
Other current liabilities	1,126	—
TOTAL CURRENT LIABILITIES	14,088	13,465
NON-CURRENT LIABILITIES:		
Long-term lease liability	1,749	—
TOTAL NON-CURRENT LIABILITIES	1,749	—
TOTAL LIABILITIES	15,837	13,465
TOTAL SHAREHOLDERS' EQUITY	210,916	90,094

TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 226,753	$ 103,559

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,	
	2019	**2018**	**2019**	**2018**
REVENUES	$ —	$ 283	18	$ 1,128
COST OF REVENUES	—	1,055	—	1,803
GROSS (LOSS) PROFIT	—	(772)	18	(675)
OPERATING EXPENSES:				
Research and development expenses	9,481	9,574	29,203	25,469
General and administrative expenses	13,972	10,743	40,454	27,019
OPERATING LOSS	(23,453)	(21,089)	(69,639)	(53,163)
FINANCE INCOME, NET	1,201	556	3,466	1,323
REALIZED LOSS ON SALE OF SHORT-TERM INVESTMENT	—	—	—	(100)
NET LOSS	$ (22,252)	$ (20,533)	$ (66,173)	$ (51,940)
NET LOSS PER ORDINARY SHARE, BASIC AND DILUTED	$ (1.06)	$ (1.28)	$ (3.25)	$ (3.30)
WEIGHTED AVERAGE SHARES OUTSTANDING, BASIC AND DILUTED	20,916,780	16,092,583	20,373,070	15,721,445
STATEMENT OF COMPREHENSIVE LOSS				
NET LOSS	$ (22,252)	$ (20,533)	$ (66,173)	$ (51,940)
OTHER COMPREHENSIVE INCOME:				
UNREALIZED GAIN ON MARKETABLE SECURITIES	22	—	303	—
COMPREHENSIVE LOSS	$ (22,230)	$ (20,533)	$ (65,870)	$ (51,940)

About UroGen Pharma Ltd.

UroGen Pharma Ltd. (Nasdaq:URGN) is a clinical-stage biopharmaceutical company developing advanced non-surgical treatments to address unmet needs in the field of urology, with a focus on uro-oncology. 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 lead investigational candidates, UGN-101 (mitomycin gel) for instillation, and UGN-102 (mitomycin gel) for intravesical instillation, 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 New York, NY with operations in Los Angeles, CA and Israel.

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 UGN-101 for LG UTUC; the potential approval of UGN-101 and the timing thereof; the expectation that UGN-101, if approved, will be the first drug approved for the non-surgical treatment of LG UTUC; the expected readiness of UroGen for a potential commercial launch of UGN-101 in 1H 2020; the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs; the opportunity and potential of UGN-102 for LG NMIBC; the planned initiation of a Phase 3 pivotal study of UGN-102 in LG NMIBC following discussions with FDA; plans for clinical development of UGN-201 and initiation of human studies; and plans to develop and commercialize zalifrelimab (AGEN1884, anti-CTLA-4 antibody) in combination with UGN-201 for the treatment of urinary tract cancers via intravesical delivery, including plans for initial development in high-grade NMIBC. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the design, timing and success of clinical trials, including the OLYMPUS Phase 3 trial, the OPTIMA II Phase 2b trial, Phase 3 studies for UGN-102, anticipated studies for UGN-201, and anticipated studies of zalifrelimab (AGEN1884, anti-CTLA-4 antibody) in combination with UGN-201 for the treatment of urinary tract cancers via intravesical delivery, 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; and UroGen's ability to attract or retain key management, members of the board of directors and personnel. 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 12, 2019, 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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