UroGen Pharma Reports Third Quarter 2018 Financial Results and Completed UGN-101 OLYMPUS Trial Enrollment

November 12, 2018

Following a Recent Pre-New Drug Application (NDA) Meeting with the FDA for UGN-101, the Company Has Concluded Patient Enrollment in the OLYMPUS Phase 3 Trial for Low-Grade Upper Tract Urothelial Cancer (LG UTUC)

Breakthrough Therapy Designation (BTD) Previously Granted by the FDA for UGN-101 for the Treatment of LG UTUC

Company On Track to Initiate UGN-101 Rolling NDA Submission to the FDA in Q4 2018 with Completed Submission in Q2 2019 and Potential Approval in 2019

Conference Call Today at 8:30 a.m. Eastern Time

RA'ANANA, Israel & NEW YORK--(BUSINESS WIRE)--Nov. 12, 2018-- UroGen Pharma Ltd. (Nasdaq:URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in the field of urology, with a focus on uro-oncology, today announced financial results for third quarter ended September 30, 2018 and that it has completed enrollment of the UGN-101 OLYMPUS Phase 3 Trial in patients with low-grade upper tract urothelial cancer (LG UTUC) following a recent pre-New Drug Application (NDA) meeting held with the U.S. Food and Drug Administration (FDA).

At the pre-NDA meeting, the company presented updated data from this open-label, single arm Phase 3 study. The meeting resulted in agreement on the required information for the NDA submission and suitability for NDA submission of primary endpoint (Complete Response) data for the approximately 65 patients enrolled to date.

UroGen plans to present the topline data in January 2019, with the full dataset expected at a medical meeting in the second quarter of 2019.

"We are pleased by the FDA's support for UGN-101 and recognition of the potential clinical benefit that UGN-101 presents for LG UTUC as a potentially organ-sparing, non-invasive therapy for this disease," said Ron Bentsur, Chief Executive Officer of UroGen. "We are excited about the progress that we continue to make across our clinical pipeline and look forward to collaborating with the FDA to bring this potentially transformative therapy to patients with LG UTUC."

Breakthrough Therapy Designation (BTD) was granted by the FDA for UGN1-101 for the treatment of LG UTUC in October 2018. UroGen remains on track to initiate the UGN-101 Rolling NDA Submission to the FDA in Q4 2018 and complete the submission in Q2 2019, with potential approval expected in 2019.

Additional Highlights and Upcoming Milestones

- **UGN-102 Clinical Development:**
  - UroGen is enrolling patients as part of its Phase 2b single-arm, open-label, multi-center trial designed to assess the efficacy and safety of UGN-102 (mitomycin gel) for intravesical instillation as a potential first-line chemoablation agent in the treatment of patients with LG Non-Muscle Invasive Bladder Cancer (NMIBC) at risk for recurrence.
  - Initial data from the trial is expected in 1H 2019.
  - Similar to LG UTUC, there are currently no drugs approved by the FDA as first-line treatment for NMIBC, and only three drugs have been approved by the FDA, all as adjuvant treatments, following TURBT (transurethral resection of bladder tumor).
  - UGN-102 represents a very substantial opportunity in UroGen's pipeline with the potential to initially address up to approximately 85,000 patients for whom TURBT is no longer effective.[1]
- **Advancing the Potential of the RTGel Platform:**
  - Allergan continues to enroll patients in its Phase 2 trial of BotuGel, UroGen's RTGel in combination with BOTOX® [2], for the treatment of overactive bladder. This clinical trial, if successful, has the potential to demonstrate the broad applicability of the RTGel platform beyond uro-oncology. Phase 2 data is expected in 2019.
- **Corporate Developments:**
  - UroGen strengthened its leadership team with the appointment of Jones "Woody" Bryan, Ph.D., as Senior Vice President, Business Development. Dr. Bryan is a seasoned industry veteran who brings over 25 years of industry experience. He is focused on the integration of corporate strategy and business development to assess potential partnerships, both inbound and outbound, and bolster UroGen's product portfolio.

Third Quarter 2018 Financial Results

- As of September 30, 2018, cash and cash equivalents totaled $109.5 million.

- Research and development expenses for the nine months ended September 30, 2018 were $25.5 million, including non-cash share-based compensation expense of $9.1 million. Research and development expenses for the three months ended September 30, 2018 were $9.6 million, including non-cash share-based compensation expense of $3.8 million.
- General and administrative expenses for the nine months ended September 30, 2018 were $27.0 million, including non-cash share-based compensation expense of $12.7 million. General and administrative expenses for the three months ended September 30, 2018 were $10.7 million, including non-cash share-based compensation expense of $5.7 million.
- The Company reported a net loss of $51.9 million, or basic and diluted net loss per ordinary share of $3.30, for the nine months ended September 30, 2018. The Company reported a net loss of $20.5 million, or basic and diluted net loss per ordinary share of $1.28, for the three months ended September 30, 2018.

Conference Call & Webcast Information

Members of UroGen's management team will host a live conference call and webcast today at 8:30 a.m. Eastern Time to review the Company's financial results and provide a general business update. Due to observance of the Veteran's Day holiday in the United States on November 12, 2018, UroGen's corresponding 6-K will be filed with the Securities and Exchange Commission (SEC) before market on November 13, 2018.

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 47697839. 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, 2018	December 31, 2017
Assets		
CURRENT ASSETS:		
Cash and cash equivalents	$ 109,483	$ 36,999
Short-term investments	—	36,001
Restricted deposit	197	198
Accounts receivable	112	—
Inventory	—	316
Prepaid expenses and other current assets	1,679	958
TOTAL CURRENT ASSETS	111,471	74,472
NON-CURRENT ASSETS:		
Property and equipment, net	992	805
Restricted deposit	81	29
Other non-current assets	14	244
TOTAL ASSETS	$ 112,558	$ 75,550
Liabilities and Shareholders' equity		
LIABILITIES:		
Accounts payable and accrued expenses	$ 6,291	$ 4,435
Employee related accrued expenses	2,684	1,950
Deferred revenues	—	650
TOTAL LIABILITIES	8,975	7,035
TOTAL SHAREHOLDERS' EQUITY	103,583	68,515
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 112,558	$ 75,550

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

	Nine months ended September 30,		Three months ended September 30,	
	2018	2017	2018	2017
REVENUES	$ 1,128	$ 7,831	$ 283	$ 7,812
COST OF REVENUES	1,803	313	1,055	295

GROSS PROFIT	(675)	7,518	(772)	7,517
OPERATING EXPENSES:				
Research and development expenses	25,469	11,936	9,574	5,621
General and administrative expenses	27,019	5,374	10,743	2,199
OPERATING LOSS	53,163	9,792	21,089	303
INTEREST AND OTHER (INCOME) EXPENSES, NET	(1,323)	122	(556)	(5)
REALIZED LOSS ON SALE OF SHORT-TERM INVESTMENT	100	—	—	—
NET LOSS	$ 51,940	$ 9,914	$ 20,533	$ 298
NET LOSS PER ORDINARY SHARE, BASIC AND DILUTED	$ 3.30	$ 1.31	$ 1.28	$ 0.02
WEIGHTED AVERAGE SHARES OUTSTANDING, BASIC AND DILUTED	15,721,445	8,223,124	16,092,583	13,051,117

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™, 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 product candidates, UGN-101 and UGN-102, are designed to potentially remove tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade upper tract urothelial carcinoma and bladder cancer, respectively. UroGen is headquartered in Ra'anana Israel with U.S. headquarters in New York, NY and an office in Los Angeles, CA.

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 with respect to the effect of Breakthrough Therapy Designation on the potential regulatory approval of UGN-101, timing and results of clinical development and commercial prospects of UGN-101, and submission of a rolling NDA for UGN-101, the timing and results of clinical development of UGN-102, and the timing and clinical application of RTGel including with respect to BotuGel, which 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 complications thereof; the ability to obtain and maintain regulatory approval; the labeling for any approved product; the scope, progress and expansion of developing and commercializing UroGen Pharma'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 Pharma'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 Pharma's Form 20-F filed with the SEC on March 15, 2018 and other filings that UroGen Pharma 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 Pharma'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 Pharma as of the date of this release.

[1] Cutress, 2012; NIH SEER Stat; UroGen Market Research

[2] BOTOX® is a proprietary trademark of Allergan Pharmaceuticals

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