UroGen Pharma Reports Second Quarter 2018 Financial Results and Recent Corporate Developments

August 14, 2018

Plan to Initiate Q4 2018 Rolling New Drug Application (NDA) Submission to the U.S. Food and Drug Administration (FDA) for UGN-101, Ahead of Initial Projection of Q1 2019

Initiated Phase 2b Clinical Trial of UGN-102 (VesiGel™) for the Treatment of Low-Grade Non-Muscle Invasive Bladder Cancer (LG NMIBC)

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

RA'ANANA, Israel and NEW YORK, Aug. 14, 2018 (GLOBE NEWSWIRE) -- 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 the second quarter ended June 30, 2018 and provided an overview of the Company's recent developments.

"There aren't many opportunities in this industry to be first, but with UGN-101 (formerly referred to as MitoGel), we have the potential to have the first drug ever approved for low-grade upper tract urothelial cancer (LG UTUC). We believe that we will be one step closer to this major milestone in the field of uro-oncology with the planned rolling submission of a New Drug Application (NDA) for UGN-101 in the fourth quarter of this year," said Ron Bentsur, Chief Executive Officer of UroGen. "In the past six months, we have focused on full execution of UGN-101, from clinical development, to regulatory process, to preparing for potential commercialization. We are leveraging the momentum and learnings from our UGN-101 program to initiate our Phase 2b trial for UGN-102 (formerly referred to as VesiGel) which has the potential to treat a significantly larger population, patients diagnosed with low-grade non-muscle invasive bladder cancer (LG NMIBC). As we continue to advance our pipeline, we believe this is just the beginning of what's possible for UroGen and our RTGel™ platform."

Recent Highlights and Upcoming Milestones

- **UGN-101 Regulatory and Clinical Development:**
  - Announced positive findings from an interim analysis of the ongoing pivotal Phase 3 OLYMPUS clinical trial of UGN-101, an investigational mitomycin formulation for the non-surgical treatment of LG UTUC in May 2018.
    - Interim analysis showed a complete response (CR) rate of 59 percent (20 out of the interim analysis intent to treat population of 34 patients) who were evaluated for primary disease evaluation (PDE, or the primary endpoint).
    - 15 percent (five of 34 patients) achieved a partial response.
    - At the time of the interim analysis presentation, of the 20 patients who achieved a CR, 13 patients had reached three-month follow-up, and all remained in CR. Four of these 13 patients had reached six-month follow-up and one of the 13 patients had reached nine-month follow-up, and all remained in CR.
  - Top-line results from the OLYMPUS trial are expected in 2H 2018.
  - UroGen intends to initiate a rolling NDA submission for UGN-101 for the treatment of LG UTUC in Q4 2018 with a targeted completion by the end of Q1 2019.
  - Potential approval and commercial launch could potentially occur in 2019. The Company previously received Fast Track and Orphan Drug Designations for UGN-101.
  - If approved, UGN-101 would be the first approved therapy for LG UTUC.

- **UGN-102 (VesiGel) Clinical Development:**
  - Successful Investigational New Drug (IND) application for UGN-102 for the treatment of LG NMIBC in Q2 2018.
  - Initiated Phase 2b single-arm, open-label, multi-center trial designed to assess the efficacy and safety of UGN-102 as a potential first-line chemoablation agent in the treatment of patients with LG NMIBC at risk for recurrence.
    - 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).
    - In 2012, the annual incidence of urothelial bladder cancer was 80,000 in the United States with a prevalence of 700,000[1]. NMIBC accounts for approximately 80% of all new cases of bladder cancer diagnosed in the United States each year, with the majority of patients experiencing life-long, repetitive surgical treatment for cancer recurrence.

- **Advancing the Potential of the RTGel Platform:**
  - UGN-201 (Vesimune™): The Company continues to advance research for its novel imiquimod formulation for bladder instillation as a single agent and in combination with immune checkpoint inhibitors for the treatment of high-grade urothelial cancer. Pre-clinical models have demonstrated antitumor effects of UGN-201 as a single agent as well as in combination with novel immunomodulatory molecules via intravesical instillation in urothelial cancer. A clinical trial of UGN-201 remains on track for 1H 2019.
  - BotuGel™: Enrollment of patients by Allergan in the Phase 2 trial of RTGel in combination with BOTOX®[2] for the

treatment of overactive bladder is ongoing. 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 Supporting Commercialization Efforts:**
  - The addition of Peter P. Pfreundschuh as Chief Financial Officer aligns with the company's strategy as it prepares for continued growth and potential commercialization in 2019. Mr. Pfreundschuh brings over 25 years of leadership experience in the biotechnology and medical device sectors overseeing finance, business development and commercial operations.
  - The Company strengthened its Board of Directors with the appointment of Shawn Tomasello, a renowned industry expert with a track record of commercializing revolutionary, multi-billion dollar products in oncology. Most recently, she served as Chief Commercial Officer of Kite Pharma (subsequently Kite, a Gilead Company), where she led the commercialization of Yescarta®3 (axicabtagene ciloleucel), the first approved chimeric antigen receptor (CAR) T therapy for the treatment of adult patients with relapsed or refractory non-Hodgkin lymphoma. Previously, Ms. Tomasello served as Chief Commercial Officer at Pharmacyclics, Inc.

Second Quarter 2018 Financial Results

- As of June 30, 2018, cash and cash equivalents totaled $119.1 million.
- Research and development expenses for the six months ended June 30, 2018 were $15.9 million, including non-cash share-based compensation expense of $5.3 million. Research and development expenses for the three months ended June 30, 2018 were $8.3 million, including non-cash share-based compensation expense of $2.8 million.
- General and administrative expenses for the six months ended June 30, 2018 were $16.3 million, including non-cash share-based compensation expense of $7.0 million. General and administrative expenses for the three months ended June 30, 2018 were $10.2 million, including non-cash share-based compensation expense of $4.9 million.
- The Company reported a net loss of $31.4 million, or basic and diluted net loss per ordinary share of $2.02, for the six months ended June 30, 2018. The Company reported a net loss of $18.0 million, or basic and diluted net loss per ordinary share of $1.14, for the three months ended June 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.

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 47260983. 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)

	June 30, 2018	December 31, 2017
Assets		
CURRENT ASSETS:		
Cash and cash equivalents	$ 119,109	$ 36,999
Short-term investments	–	36,001
Restricted deposit	197	198
Accounts receivable	232	–
Inventory	142	316
Prepaid expenses and other current assets	1,024	958
TOTAL CURRENT ASSETS	120,704	74,472
NON-CURRENT ASSETS:		
Property and equipment, net	718	805
Restricted deposit	80	29
Other non-current assets	–	244
TOTAL ASSETS	$ 121,502	$ 75,550

Liabilities and Shareholders' equity		
CURRENT LIABILITIES:		
Accounts payable and accrued expenses	$ 5,655	$ 4,435
Employee related accrued expenses	2,211	1,950
Deferred revenues	86	650
TOTAL CURRENT LIABILITIES	7,952	7,035
TOTAL LIABILITIES	7,952	7,035
COMMITMENTS AND CONTINGENCIES		
SHAREHOLDERS' EQUITY:		
Ordinary shares, NIS 0.01 par value: 100,000,000 shares authorized at June 30, 2018 and December 31, 2017; 15,853,286 and 13,751,390 shares issued and outstanding at June 30, 2018 and December 31, 2017, respectively.	43	37
Additional paid-in capital	192,129	115,692
Accumulated deficit	(78,622)	(47,214)
TOTAL SHAREHOLDERS' EQUITY	113,550	68,515
TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY	$ 121,502	$ 75,550

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

	Six months ended June 30,		Three months ended June 30,	
	2018	**2017**	**2018**	**2017**
REVENUES	$ 845	$ 19	$ 364	$ –
COST OF REVENUES	748	18	318	–
GROSS PROFIT	97	1	46	–
OPERATING EXPENSES:				
Research and Development Expenses	15,895	6,315	8,273	3,651
General and Administrative Expenses	16,276	3,175	10,207	2,300
OPERATING LOSS	32,074	9,489	18,434	5,951
INTEREST AND OTHER (INCOME) EXPENSES, NET	(766)	127	(408)	248
REALIZED LOSS ON SALE OF SHORT-TERM INVESTMENT	100	–	–	–
NET LOSS	$ 31,408	$ 9,616	$ 18,026	$ 6,199
NET LOSS PER ORDINARY SHARE BASIC AND DILUTED	$ 2.02	$ 1.81	$ 1.14	$ 0.70
WEIGHTED AVERAGE NUMBER OF SHARES OUTSTANDING USED IN COMPUTATION OF BASIC AND DILUTED LOSS PER ORDINARY SHARE	15,528,826	5,755,714	15,784,393	9,204,405

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 (mitomycin gel for urothelial instillation), formerly known as MitoGel®, and UGN-102 (mitomycin gel for intravesical instillation), formerly known as VesiGel™, 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.

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 timing and results of clinical development and commercial prospects of the product candidates in UroGen's pipeline, including UGN-101 (formerly referred to as MitoGel®), UroGen establishing a leadership position in the field of uro-oncology and the ability to commercialize, which statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the potential approval of its first therapy; the ability to obtain and maintain regulatory approval; the scope, progress and expansion of developing and commercializing UroGen's product candidates; and UroGen's ability to attract or retain key management and personnel. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of our annual report for the year ended December 31, 2017 filed with the SEC on March 15, 2018 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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[1] https://seer.cancer.gov/statfacts/html/urinb.html

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

[3] Yescarta® is a proprietary trademark of Kite Pharma, Inc.

UroGen Pharma Ltd.