# UroGen Pharma Reports Fourth Quarter and Full Year 2019 Financial Results and Recent Corporate Developments

March 2, 2020

*Prescription Drug User Fee Act (PDUFA) Goal Date of April 18th for UGN-101 in Patients with Low-Grade Upper Tract Urothelial Cancer (LG UTUC)*

*Experienced Commercial Team Hired and Prepared for Planned UGN-101 Approval and Launch in Q2*

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

NEW YORK--(BUSINESS WIRE)--

UroGen Pharma Ltd. (Nasdaq:URGN) a biopharmaceutical company dedicated to building novel solutions that treat specialty cancers and urologic diseases because patients deserve better options, today announced financial results for the fourth quarter and full year ended December 31, 2019 and provided an overview of the Company's recent developments.

"At UroGen, we are eagerly awaiting potential approval of our lead product candidate, UGN-101, for the treatment of patients with low-grade upper tract urothelial cancer (LG UTUC). The significant progress on key clinical, regulatory and commercial milestones in 2019 places us in a position of strength as we prepare to deliver the first non-surgical therapy for the treatment of LG UTUC. Our experienced commercial team has been working tirelessly to ensure we are prepared for launch, and we look forward to providing these patients with a new treatment option," said Liz Barrett, President and Chief Executive Officer (CEO) of UroGen. "We are also advancing multiple pipeline candidates in development for areas of unmet need in both low-grade and high-grade bladder cancer, including UGN-102 and UGN-302, respectively. As our team pioneers new treatments to improve patient care in specialty cancers and urologic diseases, we look forward to maximizing patient and shareholder value through the exciting events on the horizon".

**2019 and Recent Highlights**

*UGN-101 (mitomycin gel) for instillation for Patients with LG UTUC Progress*

- Reported positive updated durability and complete response data from the pivotal Phase 3 OLYMPUS trial in September 2019. The data were consistent with previously reported results in May 2019 and January 2019.
- Completed a rolling New Drug Application (NDA) to the U.S. Food and Drug Administration (FDA).
- Received FDA filing acceptance and Priority Review of the NDA for UGN-101, with a PDUFA goal date of April 18, 2020. If approved, UGN-101 will be the first non-surgical, chemoablative treatment option for LG UTUC.

*UGN-101 Commercial Readiness*

- Hired an internal team with a track record of success in urology and oncology as well as a veteran sales force leadership team with deep uro-oncology relationships.
- Implemented innovative solutions around patient identification, reimbursement and seamless logistics to enhance physician adoption upon launch. Based on recent market research, 88% of urologists desire a new and differentiated treatment option for their patients.
    - Executed agreements with 3PL, a specialty distributor and a national partner to provide prepared admixture to urology clinics.

*Pipeline Expansion and Developments*

- *UGN-102 (mitomycin gel) for intravesical instillation for patients with low-grade intermediate risk non-muscle invasive bladder cancer (LG IR NMIBC)*
    - Completed enrollment ahead of schedule and reported positive interim results from the single-arm, open-label Phase 2b OPTIMA II trial of investigational UGN-102 for patients with LG IR NMIBC.
    - LG IR NMIBC is defined as those patients with one or two of the following criteria: multifocal disease, large tumors and rapid rates of recurrence. 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 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.
- *UGN-201 (TLR7/8 agonist) for patients with high-grade non muscle invasive bladder cancer (HG NMIBC)*
    - Shared nonclinical data of investigational UGN-201 (a TLR7/8 agonist) as a monotherapy and in combination with checkpoint inhibitors.
        - In murine models, UGN-201 in combination with local anti-CTLA-4 increased survival.

- Entered into an exclusive worldwide license agreement with Agenus Inc. to develop and commercialize zalifrelimab (AGEN1884, anti-CTLA-4 antibody) via intravesical delivery in combination with UGN-201 for the treatment of high-grade urinary tract cancers, initially targeting HG NMIBC. The combination of UGN-201 and zalifrelimab is referred to as investigational agent UGN-302.

*Corporate Achievements*

- Strengthened the Company's financial position with a follow-on offering of approximately $162 million in January 2019.

**2020 Anticipated Milestones and Product Development Plans**

*UGN-101*

- Publication of the final results of the primary endpoint from the OLYMPUS trial in patients with LG UTUC in 1H
- UGN-101 potential approval and launch in Q2

*UGN-102*

- Updated durability and complete response data from UGN-102 Phase 2b Study
- Initiation of pivotal Phase 3 Study in 2H

*UGN-302*

- Advancement to first in human clinical study following formulation and dose optimization

**Fourth Quarter and Full Year 2019 Financial Results; 2020 Guidance**

- As of December 31, 2019, cash, cash equivalents and marketable securities totaled $195.6 million, excluding restricted cash.
- Research and development expenses for the three months ended December 31, 2019 were $20.1 million, including non-cash share-based compensation expense of $1.9 million. Research and development expenses for the year ended December 31, 2019 were $49.3 million, including non-cash share-based compensation expense of $8.3 million. The research and development expenses for the three months and year ended December 31, 2019 included an in-process research and development charge of $10.0 million associated with the execution of the Agenus licensing agreement.
- General and administrative expenses for the three months ended December 31, 2019 were $19.7 million, including non-cash share-based compensation expense of $6.2 million. General and administrative expenses for the year ended December 31, 2019 were $60.2 million, including non-cash share-based compensation expense of $21.7 million.
- UroGen reported a net loss of $39.0 million, or basic and diluted net loss per ordinary share of $1.86, for the three months ended December 31, 2019. The Company reported a net loss of $105.1 million, or basic and diluted net loss per ordinary share of $5.12, for the year ended December 31, 2019.
- The Company anticipates operating expenses in the range of $145 to $155 million for 2020. Non-cash stock-based compensation expense for 2020 is expected to be in the range of $32 to $36 million subject to market conditions, and other non-operating income for 2020 is anticipated to be approximately $2.5 million.
- UroGen has 21.0 million ordinary shares outstanding.

**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 49393633. 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)

| | December 31, 2019 | December 31, 2018 |
|---|---|---|
| **Assets** | | |
| **CURRENT ASSETS:** | | |
| Cash and cash equivalents | $ 49,688 | $ 101,318 |
| Marketable securities | 97,389 | — |
| Restricted cash | 523 | 253 |
| Prepaid expenses and other current assets | 1,034 | 672 |
| **TOTAL CURRENT ASSETS** | 148,634 | 102,243 |
| **NON-CURRENT ASSETS:** | | |
| Property and equipment, net | 977 | 948 |

| | | |
|---|---|---|
| Restricted deposit | 223 | 51 |
| Right of use asset | 3,735 | — |
| Marketable securities | 48,555 | — |
| Other non-current assets | 264 | 317 |
| **TOTAL ASSETS** | $ 202,388 | $ 103,559 |
| | | |
| **Liabilities and Shareholders' equity** | | |
| | | |
| **CURRENT LIABILITIES:** | | |
| Accounts payable and accrued expenses | $ 11,186 | $ 8,540 |
| Employee related accrued expenses | 6,711 | 4,925 |
| Other current liabilities | 1,585 | — |
| **TOTAL CURRENT LIABILITIES** | 19,482 | 13,465 |
| | | |
| **NON-CURRENT LIABILITY:** | | |
| Long-term lease liability | 2,604 | — |
| **TOTAL LIABILITIES** | 22,086 | 13,465 |
| **TOTAL SHAREHOLDERS' EQUITY** | 180,302 | 90,094 |
| **TOTAL LIABILITIES AND SHAREHOLDERS' EQUITY** | $ 202,388 | $ 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)

| | Year ended December 31, | | Three months ended December 31, | |
|---|---|---|---|---|
| | **2019** | **2018** | **2019** | **2018** |
| **REVENUES** | $ 18 | $ 1,128 | $ — | $ — |
| **COST OF REVENUES** | — | 1,803 | — | — |
| **GROSS PROFIT (LOSS)** | 18 | (675) | — | — |
| **OPERATING EXPENSES:** | | | | |
| Research and development expenses | 49,297 | 36,934 | 20,094 | 11,465 |
| General and administrative expenses | 60,199 | 39,571 | 19,745 | 12,552 |
| **OPERATING LOSS** | (109,478) | (77,180) | (39,839) | (24,017) |
| **INTEREST AND OTHER INCOME, NET** | (4,332) | (1,648) | (866) | (425) |
| **LOSS BEFORE INCOME TAXES** | (105,146) | (75,532) | (38,973) | (23,592) |
| **INCOME TAX EXPENSE** | — | 125 | — | 125 |
| | | | | |
| **NET LOSS** | $ (105,146) | $ (75,657) | $ (38,973) | $ (23,717) |
| | | | | |
| **STATEMENT OF COMPREHENSIVE LOSS** | | | | |
| **NET LOSS** | $ (105,146) | $ (75,657) | $ (38,973) | $ (23,717) |
| **OTHER COMPREHENSIVE INCOME:** | | | | |
| **UNREALIZED GAIN (LOSS) ON MARKETABLE SECURITIES** | 276 | — | (27) | — |
| **COMPREHENSIVE LOSS** | $ (104,870) | $ (75,657) | $ (39,000) | $ (23,717) |
| | | | | |
| **NET LOSS PER ORDINARY SHARE, BASIC AND DILUTED** | $ (5.12) | $ (4.80) | $ (1.86) | $ (1.46) |
| **WEIGHTED AVERAGE SHARES OUTSTANDING, BASIC AND DILUTED** | 20,528,727 | 15,754,193 | 20,988,930 | 16,212,274 |

**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 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 Princeton, NJ with operations in New York, NY and Israel. Visit www.urogen.com to learn more or follow us on Twitter, @UroGenPharma.

**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 for UGN-101 to be approved and the timing thereof as a treatment option for LG UTUC; the expectation that UGN-101, if approved, will be the first drug approved for the treatment of LG UTUC; the expected readiness of UroGen for a potential commercial launch of UGN-101 in Q2 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 and the planned initiation of a Phase 3 pivotal study of UGN-102 in LG NMIBC; planned clinical development of UGN-201 for patients with HG NMIBC, either as monotherapy or in combination with other drugs; planned clinical development of

UGN-302 for intravesical treatment of urinary cancers following formulation and dose optimizations studies. These and other statements above are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of clinical trials, including the OLYMPUS Phase 3 trial 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-K filed with the SEC on March 2, 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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