Transforming Therapies in Uro-Oncology

Jefferies Healthcare Conference
June 4, 2019
Forward-Looking Statements

This presentation and the accompanying oral presentation by UroGen Pharma Ltd. ("UroGen") contains forward-looking statements. All statements contained herein other than statements of historical fact constitute forward-looking statements, including statements regarding UroGen’s anticipated results of operations and financial position, business strategy and operating plans and UroGen’s expectations for future operations.

This press release contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, including, without limitation: plans to conduct an early stage feasibility evaluation; the potential of UGN-101 for LG UTUC; the timing for completion of the rolling NDA for UGN-101; the anticipated timing for full Phase 3 data from the OLYMPUS trial; 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 timing for completion of pre-commercial activities and infrastructure build-out in anticipation of a potential commercial launch of UGN-101; the expected readiness of UroGen for a potential commercial launch of UGN-101 and the strength and timing of the potential commercial launch of UGN-101; the expected reimbursement landscape for UGN-101; the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs; the anticipated timing for initial data from the OPTIMA II trial; the opportunity and potential of UGN-102 for LG NMIBC; the likelihood of regulatory timing and approvals for UGN-201; and UroGen’s 2019 guidance. These statements 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 the OPTIMA II Phase 2b 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; risks that UroGen’s net loss for 2019 may differ materially from the anticipated range previously provided by UroGen and affirmed in this press release due to changes in UroGen’s operating plans and/or due to estimates that may prove to be incorrect; 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 May 9, 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.
**UroGen: Delivering Value to Patients in Uro-Oncology and Beyond**

- **RTGel™** reverse thermal hydrogel technology platform
- Novel, Innovative treatment for LG UTUC & LG NMIBC
- Strong foundation and balance sheet
- Expanding beyond RTGel with UGN-201 and active partnership discussions

**LG UTUC**: Low-grade upper tract urothelial cancer
**LG NMIBC**: Low-grade non-muscle invasive bladder cancer
Significant Market Opportunity in Urologic Cancers

UroGen: Platform to Product Candidates

Upper Tract Urothelial Cancer (UTUC)

- **UGN-101 (LG)**
  - Annual US Addressable Market: ~6-8k

Bladder Cancer

- **UGN-102 (LG)**
  - Annual US Addressable Market: ~80k

- **UGN-201 (HG)**
  - Annual US Addressable Market: ~2k+
How the RTGel Technology is Designed to Work

RTGel\(^{(1)}\): Liquid at low temperature (LT) and converts into gel form at body temperature (BT) following intravesical instillation

1. Storage
   Room temperature
   Gel form

2. Pre-Instillation
   Low temperature
   Gel to Liquid form

3. During Instillation
   Low temperature
   Liquid form

4. Post-Instillation
   Body temperature
   Liquid to Gel form

5. In Bladder/Upper Tract
   Body temperature
   Gel form

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\(^{(1)}\) RTGel: Reverse Thermal Gelation Hydrogel.
LG UTUC Market Overview
What is LG UTUC and Who are These Patients?

- Urothelial carcinoma (UC) is the 9th most common cancer globally.\(^1\)
- UC is the most costly cancer in the US health care system on a per-patient basis.\(^1\)
- Low grade upper tract (kidney and ureter) urothelial carcinoma (LG UTUC) is a rare malignant tumor of the cells lining the urinary tract. \(^2\)
- Most commonly presents in the elderly who also suffer from comorbid conditions such as hypertension, diabetes, obesity and the metabolic syndrome.\(^2\)
- Current standard of care for LG UTUC typically involves repetitive endoscopic tumor removal and/or removal of the kidney and ureter.


\(^2\) Browne BM, et al. 2018
Current Journey Drives Recurrent Patients to Kidney Removal

*Many patients treated with repeated ureteroscopy until RNU
A Significant Unmet Need in LG UTUC

78% Nephroureterectomies Performed in Patients with LG UTUC

Impact of RNU:
• Hospitalization & general anesthesia
• Quality of life, lifestyle, and activity reduction
• Risk of CKD
An Increasing Proportion of Urologists Wish They Had a Treatment that Would Delay Radical Surgical Intervention for LG UTUC

I wish I had a treatment that would **delay radical surgical intervention** for low-grade UTUC

<table>
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<tr>
<td>3%</td>
<td>9%</td>
<td>20%</td>
<td>68%</td>
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UroGen Market Research
UGN-101 Has the Potential to Revolutionize Treatment in LG UTUC

Innovation versus Radical Intervention
UGN-101 (mitomycin gel) for instillation
UGN-101: A Case of Complete Remission without Surgery

Patient with LG UTUC

Previously underwent repeated endoscopic surgery

Candidate for kidney removal

Before UGN-101

Post UGN-101

UGN-101 is an investigational product candidate not approved by the FDA
UroGen Delivers Updated Complete Response (CR) and Durability Data from the UGN-101 Phase 3 OLYMPUS Trial

*Complete Response (CR) Rate Consistent at 59 Percent*

*Six-month Durability Strong with 89 Percent of Evaluable Patients Remaining in CR*

*Full Phase 3 Data Anticipated for 2H 2019*

NEW YORK--(BUSINESS WIRE)--UroGen Pharma Ltd. (Nasdaq: URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in the field of urology, today announced findings from a secondary analysis from the pivotal Phase 3 OLYMPUS trial which showed that UGN-101 (mitomycin gel) for instillation, an investigational mitomycin formulation, demonstrated a 59 percent complete response rate in a subset of patients with endoscopically unresectable low-grade upper tract urothelial cancer (UTUC). Findings were presented by Seth Paul Lerner, M.D., FACS, Professor of Urology at Baylor College of Medicine, in an oral presentation during the plenary session at the 114th American Urological Association (AUA) Annual Meeting in Chicago.

May 05, 2019 04:25 PM Eastern Daylight Time
UGN-101: 59% CR Rate from OLYMPUS Study Significantly Higher than Protocol Defined Target Response Rate

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<th>RESPONSE RATE*</th>
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<tr>
<td>Overall (n=71)</td>
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<tr>
<td>CR Rate</td>
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<tr>
<td>6 Month CR Durability**</td>
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**Forty-one patients entered follow-up. At the time of the analysis, 66 percent (27/41) of patients have completed a six-month evaluation.

- Data consistent with January 2019 Data Update and Interim Analysis Presented at AUA in May 2018
- Majority of adverse events were reported as mild or moderate. Events occurring at a rate of greater or equal to 15% included: ureteric stenosis, hematuria, urinary tract infection, flank pain, nausea, hydronephrosis, vomiting, dysuria and abdominal pain.

*As of data announcement at AUA on May 5, 2019
UGN-101: Potential to be 1st Drug Ever Approved in LG UTUC

- FDA Orphan Drug Designation – September 2014
- FDA Fast Track Designation – August 2017
- FDA Breakthrough Therapy Designation – October 2018
- Rolling NDA Submission Targeted Completion
- OLYMPUS Full Data Readout
- Potential Approval and Launch
Preventing for Commercialization of UGN-101 Upon Approval

Current commercial plans optimized for UGN-101 and efficiently set the stage for UGN-102
Prepping the Market for the Launch of UGN-101

UroGen Corporate
Positions UroGen as an emerging leader in urology and uro-oncology

Innovative Solutions
Raises awareness for the benefits of RTGel and its potential therapeutic applications

Kidney Preservation
Defines and advances the clinical rationale for kidney preservation as a driver of treatment selection in UTUC
Accelerating Educational Initiatives to Impact Awareness and Adoption

- **UTUC.com**: Designed to address a void in the urology space by educating patients about UTUC and available treatment options
  - Make patients aware of additional treatment options before RNU and encourage them to talk to their urologists about taking a kidney-sparing approach

- **Medical Science Liaisons**: 7 MSLs hired and deployed to engage in education, scientific exchange and clinical support
  - >100 one-on-one KOL interactions at AUA 2019
Multifaceted, Targeted Approach to Support Uro-Oncology Practices

- **Targeted approach** lays groundwork for understanding of therapy, patient management and reimbursement
- Smart targeting – **right customers** - **right patients** - **right time**

33% of Urology Practices Treat 90% of Patient Population
Engaging in a Proactive Market Access Strategy

Distribution

Coverage and Reimbursement

UroGen Support

Defining Cost Burden to System for LG UTUC via HEOR Study

UGN-101 Has Potential to:

- **Reduce** risks of surgery and anesthesia
- **Reduce** high cost of surgery
- **Reduce** post-operative complications & downtime
- **Reduce** need for dialysis, possible kidney transplant, chronic kidney disease-related morbidity
Broad Payer Reimbursement is Anticipated With Minimal Management

- We anticipate UGN-101 will be primarily reimbursed under medical benefit (Commercial/Medicaid) and Medicare Part B. Two reimbursement opportunities:
  - Drug Costs ("buy and bill")
  - Procedural and ancillary fees associated with product administration
- Commonly used CPT are an anticipated fit for UGN 101 Instillation
  - Cystoscopy
  - Retrograde pyelogram
  - Fluoroscopy
- Coverage expected; Commercial and Medicaid plans unlikely to restrict access beyond Prior Authorization

As mean age of diagnosis for UTUC is around 70 years old, majority of patients are covered under Medicare

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<th>Medicaid</th>
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<tr>
<td>54%</td>
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UGN-102 (mitomycin gel) for intravesical instillation
UGN-102: Potential to be the First Primary Non-Surgical Chemoablative Therapy for BC

Rationale for Innovating NMIBC Treatment

- Potential to replace the standard of care (TURBT)
  - Large patient population
  - Relapse rates are high
  - Limited treatment options
- Drugs currently used only as adjuvant after surgery
- Last drug approved >15 years ago
- Moves care from OR to office
- Potential to decrease cost and morbidity of contemporary therapy

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<table>
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<th>INTERMEDIATE RISK LG NMIBC</th>
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<tr>
<td>~80,000</td>
<td>(10-20% of total LG NMIBC population)</td>
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SEER Data – US
UGN-102 is an investigational product not approved by the FDA
UGN-102: OPTIMA Trial Primary Chemoablation for Intermediate Risk LG NMIBC

- **Design**: Open-label, single-arm, Phase 2b trial
- **Patient Group**: “Intermediate Risk” LG NMIBC
- **Number of Patients**: 60 patients
- **Treatment Regimen**: Six weekly instillations of UGN-102
- **Primary Efficacy Endpoint**: CR at three months
  - Durability at 12-months also key
- **Planned Regulatory Pathway**: 505(b)(2)
UGN-201 (imiquimod)
UGN-201: Leveraging the Platform in Immunotherapy

- Intriguing immunomodulatory asset
- Encouraging Phase 1b data in CIS suggests preliminary efficacy signal
- Pre-clinical Models Have Demonstrated Antitumor Effects of UGN-201 for High-Grade Disease
- Evaluating pathways to advance program to clinical trials
Allergan Collaboration: Moving RTGel Beyond Oncology to OAB

Current BOTOX® Overactive Bladder (OAB) Sales:
~ $500 Million

- Extends power of the platform with a new type of molecule
- Exclusive license agreement with Allergan for non-invasive approach to OAB
- Potential to replace multiple injections of BOTOX into the bladder with a single instillation
- Up to $225 million ($25 million already received) and tiered royalties on net sales

BOTOX® is a registered trademark of Allergan
UroGen Pharma Announces Early Stage Feasibility Agreement with Janssen

*Focused Agreement is on Therapeutic Area of Mutual Interest*

April 23, 2019 08:00 AM Eastern Daylight Time

NEW YORK--(BUSINESS WIRE)--UroGen Pharma Ltd. (Nasdaq:URGN), a clinical-stage biopharmaceutical company developing treatments to address unmet needs in the field of uro-oncology, today announced that it has entered into an agreement with Janssen Research & Development, LLC (Janssen) to conduct an early-stage feasibility evaluation in a therapeutic area of mutual interest. UroGen and Janssen will each conduct certain activities under the terms of the agreement.
Strong Momentum Heading into 2H 2019

**2H 2019**
- UGN-101 NDA Submission
- UGN-101 Full Data Readout
- UGN-102 Initial CR Data
- UGN-102 OPTIMA II Enrollment Completion
- UGN-201 Clinical Pathway Determined
- Salesforce hired, trained and prepared for UGN-101 launch
- Allergan Completion of Phase 2 trial of RTGel with BOTOX

**2020**
- Potential Approval and Launch of UGN-101
- UGN-102 Update and Next Steps
- Initiation of UGN-201 Clinical Trial
- Allergan Next Steps of RTGel with BOTOX Program
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