



# Transforming Therapies in Uro-Oncology

Corporate Presentation  
June 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

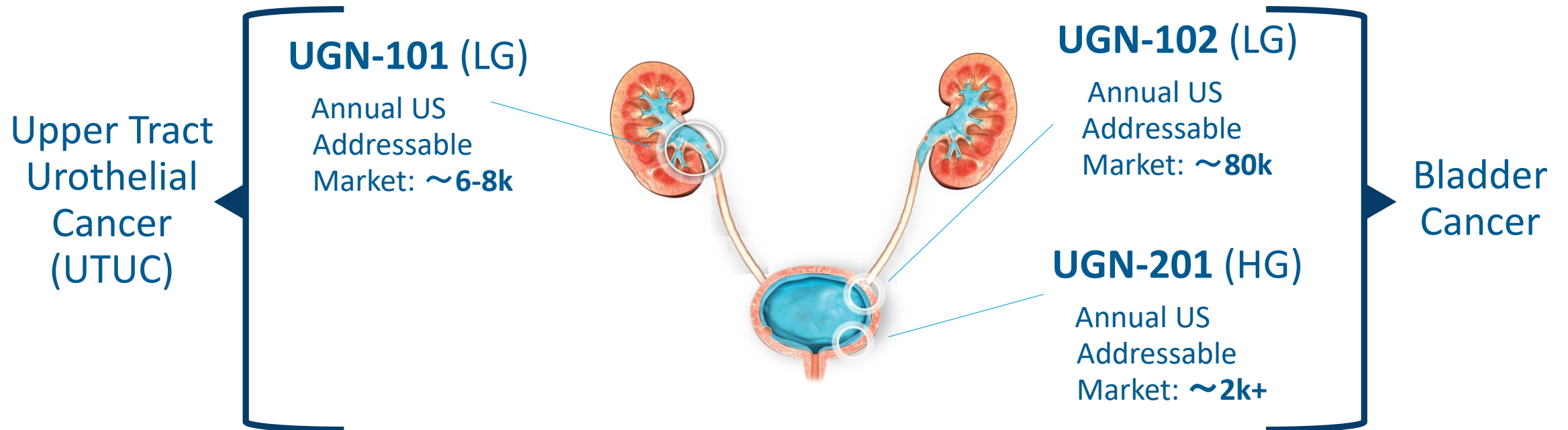


Strong foundation and balance sheet

Expanding beyond RTGel with UGN-201 and active partnership discussions

# Significant Market Opportunity in Urologic Cancers

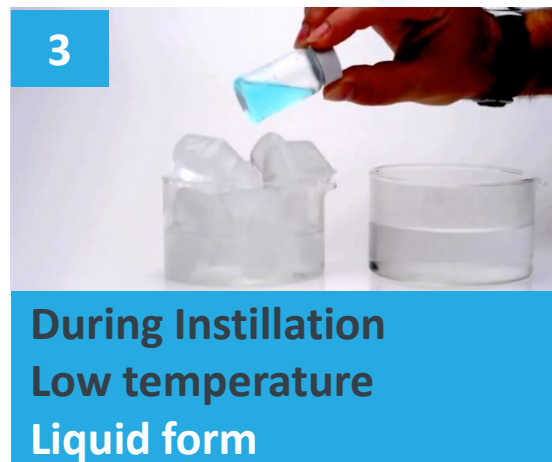
## UroGen: Platform to Product Candidates



## Reverse Thermal Hydrogel Technology

# How the RTGel Technology is Designed to Work

*RTGel<sup>(1)</sup>: Liquid at low temperature (LT) and converts into gel form at body temperature (BT) following intravesical instillation*



<sup>(1)</sup> RTGel: Reverse Thermal Gelation Hydrogel.

# LG UTUC Market Overview

# What is LG UTUC and Who are These Patients?

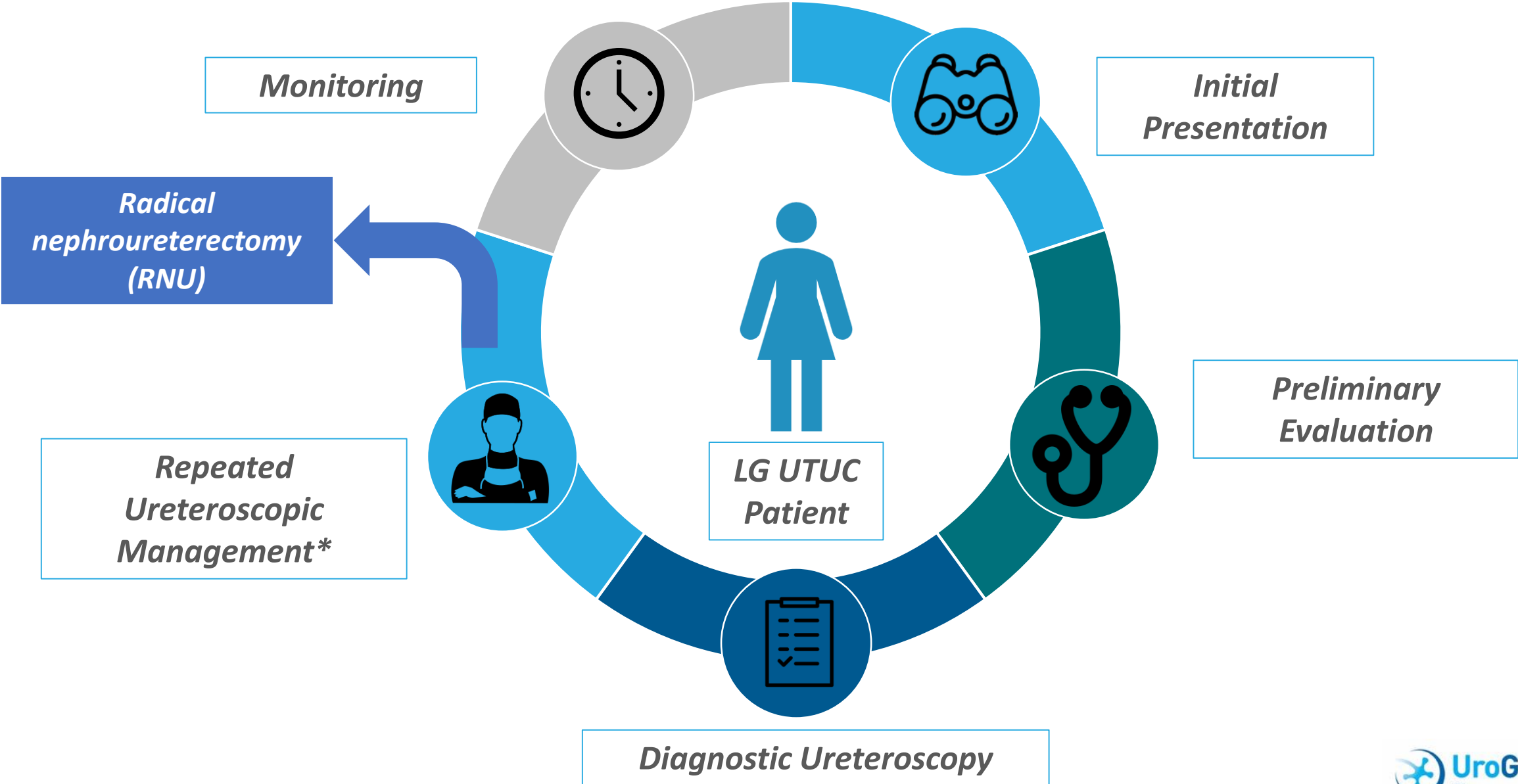
- Urothelial carcinoma (UC) is the 9<sup>th</sup> most common cancer globally.<sup>1</sup>
- UC is the most costly cancer in the US health care system on a per-patient basis.<sup>1</sup>
- Low grade upper tract (kidney and ureter) urothelial carcinoma (LG UTUC) is a rare malignant tumor of the cells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



<sup>1</sup> <http://www.hematologyandoncology.net/archives/february-2016/upper-tract-urothelial-carcinoma-special-considerations/>

<sup>2</sup> 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%

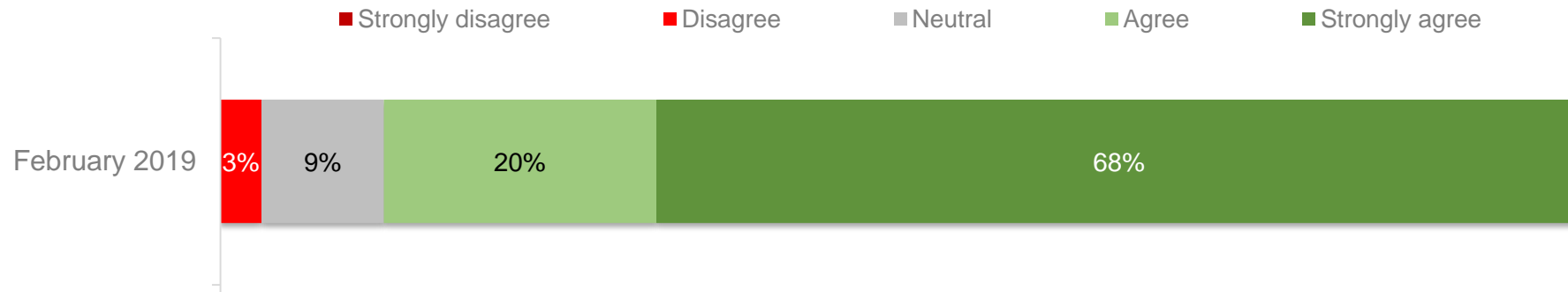
Nephroureterectomies Performed  
in Patients with LG UTUC

## Impact of RNU:

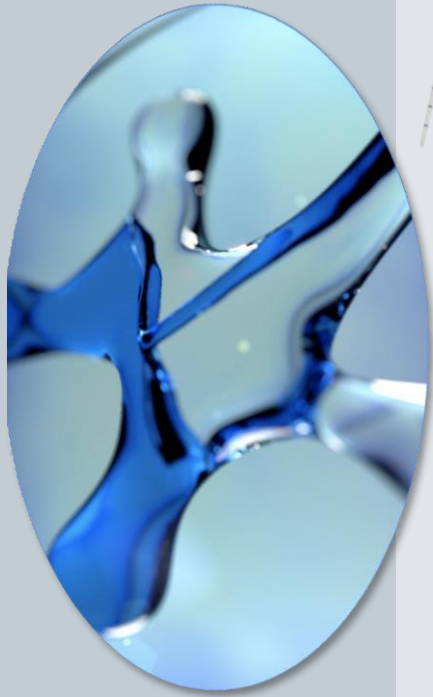
- Hospitalization & general anesthesia
- Quality of life, lifestyle, and activity reduction
- Risk of CKD

# 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



# UGN-101 Has the Potential to Revolutionize Treatment in LG UTUC



***Innovation versus Radical Intervention***

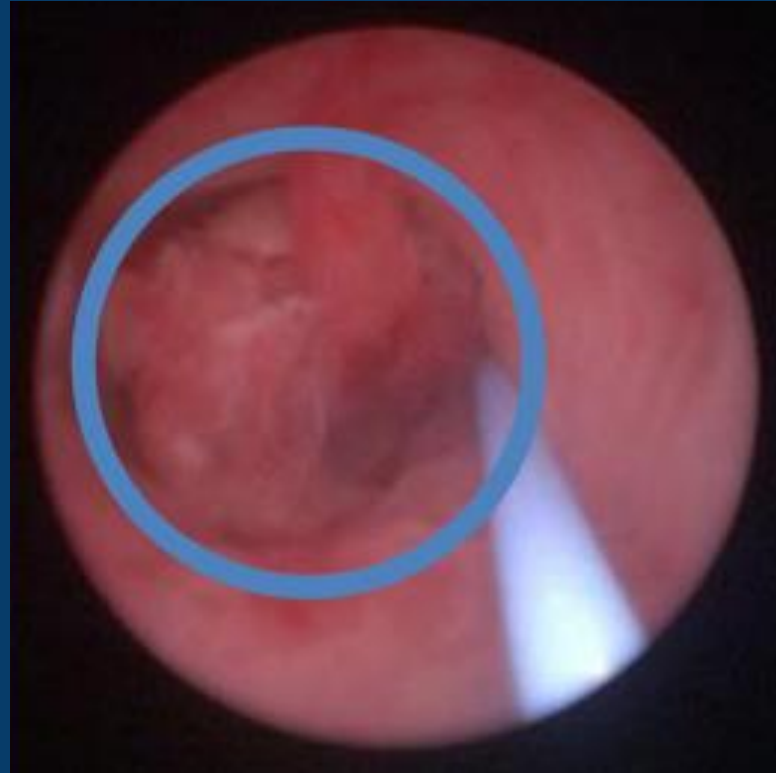
# UGN-101 (mitomycin gel) for instillation

# UGN-101: A Case of Complete Remission without Surgery

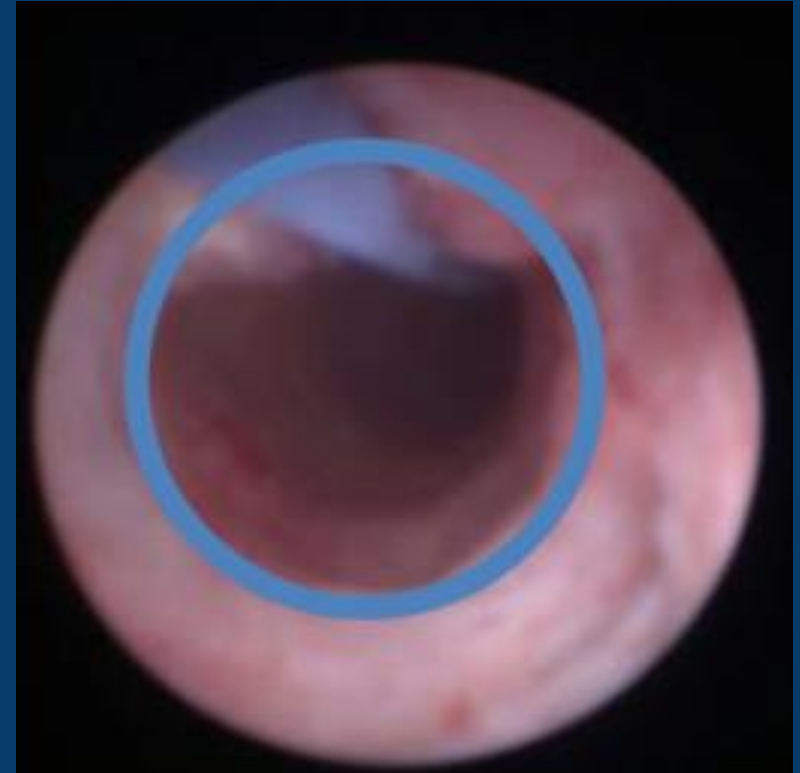
Patient with LG UTUC

Previously underwent repeated endoscopic surgery

Candidate for kidney removal



Before UGN-101



Post UGN-101

# **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***

May 05, 2019 04:25 PM 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 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 114<sup>th</sup> American Urological Association (AUA) Annual Meeting in Chicago.

# UGN-101: 59% CR Rate from OLYMPUS Study Significantly Higher than Protocol Defined Target Response Rate

	RESPONSE RATE*	
	Overall (n=71)	Endoscopically Unresectable Tumors 48% (34/71)
CR Rate	59% (42/71)	59% (20/34)
6 Month CR Durability**	89% (24/27)	85% (17/20)

*\*\*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.

# 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
- 2H 2019 Rolling NDA Submission Targeted Completion
- 2H 2019 OLYMPUS Full Data Readout
- 1H 2020 Potential Approval and Launch



# Preparing 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

**INTRODUCING UROGEN PHARMA**

UroGen is a clinical-stage biopharmaceutical company focused on developing innovative solutions to address unmet needs in the fields of urology and uro-oncology.

urogen.com

**BOOTH #3840**

**UroGen Pharma**

**TRANSFORMING LOCAL THERAPIES IN UROLOGY**

UroGen is a clinical-stage biopharmaceutical company focused on developing innovative solutions to address unmet needs in the fields of urology and uro-oncology.

**An Industry-Leading Uro-Oncology Pipeline**

Phase	Number of Candidates
Phase I	1
Phase II	2
Phase III	1
Preclinical	1

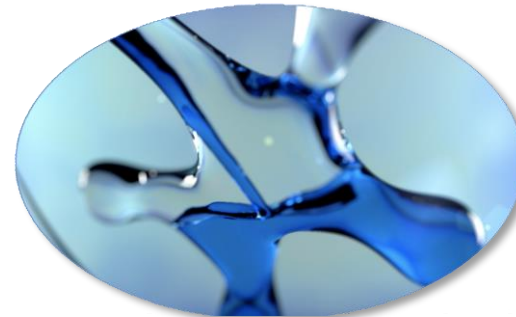
**UroGen Pharma**

**TRANSFORMING LOCAL THERAPIES IN UROLOGY**

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**UroGen Pharma**

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



**THE RIGHT TOOL FOR TOUGH JOBS**

UroGen is a clinical-stage biopharmaceutical company focused on developing innovative solutions to address unmet needs in the fields of urology and uro-oncology.

**UroGen Pharma**

**Kidney Preservation**  
Defines and advances the clinical rationale for kidney preservation as a driver of treatment selection in UTUC

**Kidney preservation in upper tract urothelial carcinoma (UTUC): Are we doing all we can?**

**Clinical perspectives on kidney preservation and its place in modern UTUC treatment**

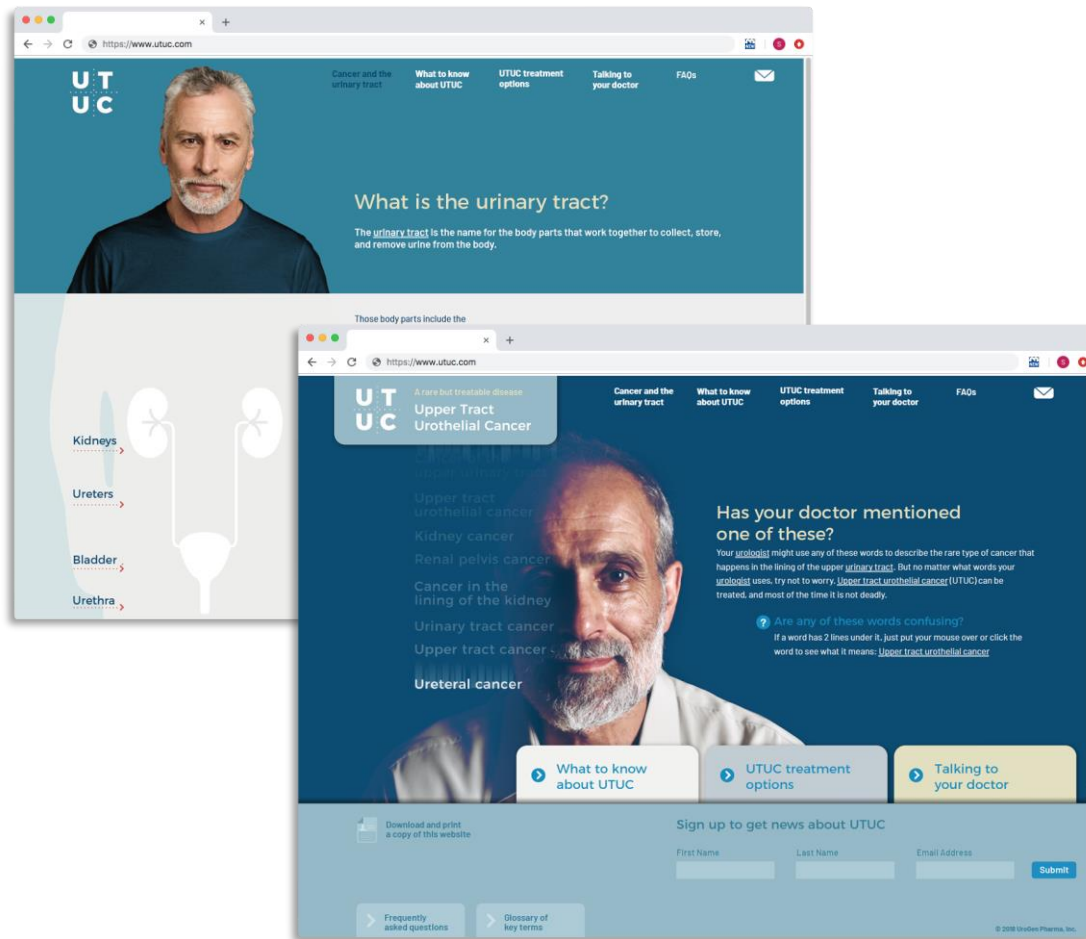
**UTUC Intactileum**

**Kidney preservation in upper tract urothelial carcinoma (UTUC): Are we doing all we can?**

The case for prioritizing kidney-sparing approaches in low-grade UTUC treatment

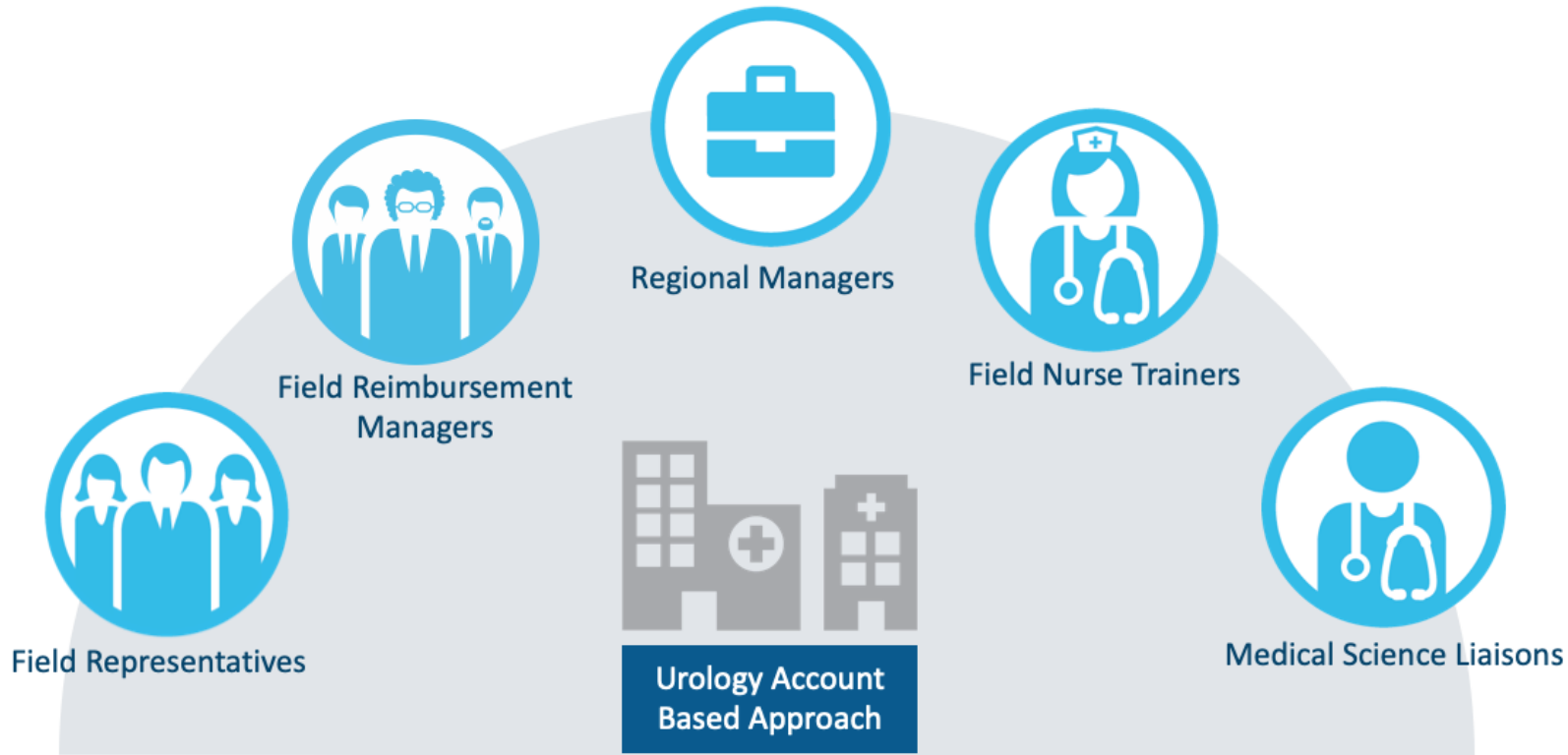
**UTUC Intactileum**

# 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 MSJs 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**



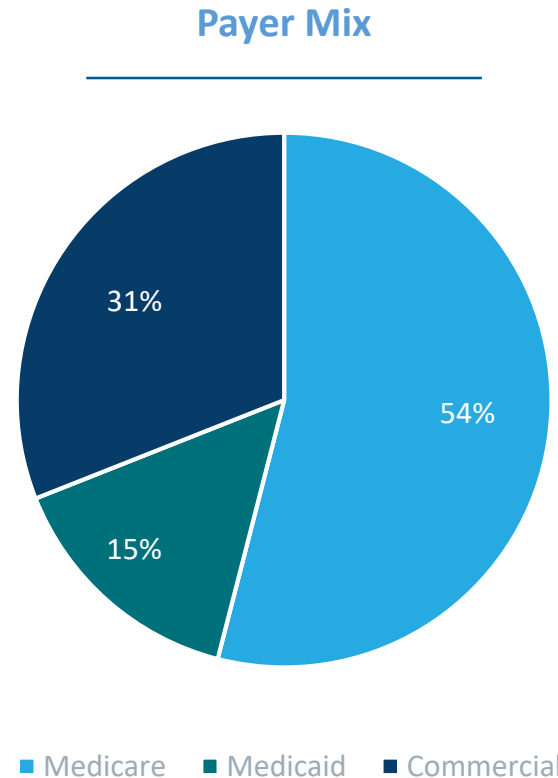
**UroGen Support**

**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



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

- 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

# 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

### BLADDER CANCER

**700,000** prevalence

**81,000** incidence

### LG NON-MUSCLE INVASIVE BLADDER CANCER

**343,000** prevalence

**40,000** incidence

### INTERMEDIATE RISK LG NMIBC

**~80,000** (10-20% of total LG NMIBC population)



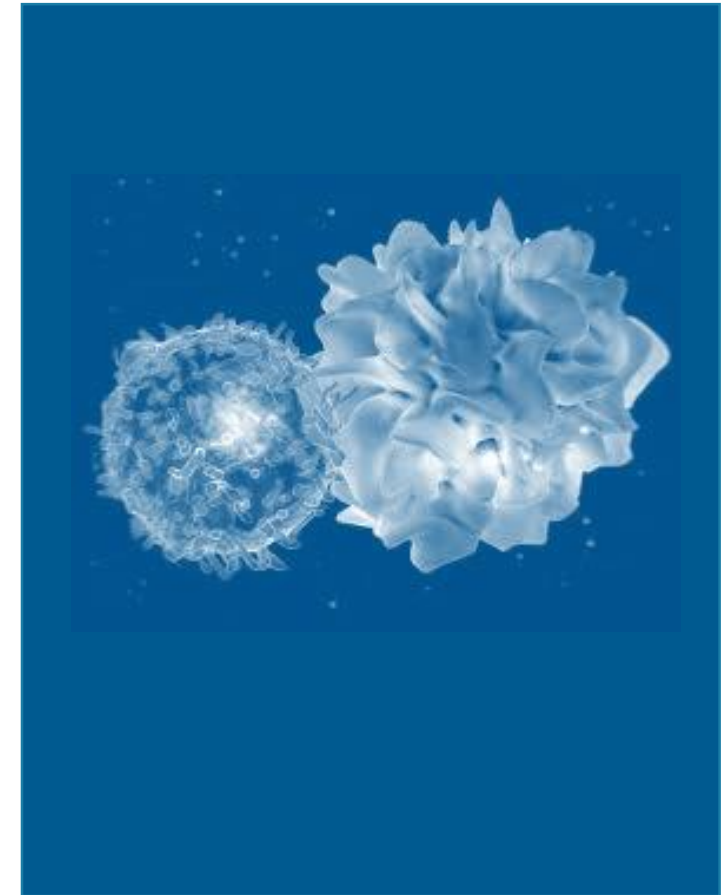
# UGN-102: OPTIMA Trial Primary Chemoablation for Intermediate Risk LG NMIBC

- ✓ UGN-102 Trial Initiation – August 2018
  - 2H 2019 Initial CR Data
  - 2H 2019 Complete Enrollment
  - 2020 Evaluate CR and Durability
- **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



# UroGen and Janssen: Early Stage Feasibility Evaluation



## **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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