Building a Uro-Oncology Franchise
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These forward-looking statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of preclinical studies and clinical trials conducted by or on behalf of UroGen, including with respect to the efficacy and safety of UroGen’s product candidates; UroGen’s ability to obtain and maintain regulatory approval of its product candidates, and the labeling for any approved products; the scope, progress, expansion and costs of developing and commercializing UroGen’s product candidates; UroGen’s ability to obtain and maintain intellectual property protection for its product candidates; UroGen’s anticipated growth strategies; UroGen’s expectations regarding competition; the anticipated trends and challenges in UroGen’s business and the markets in which it operates; UroGen’s ability to attract or retain key management and personnel; the size and growth of the potential markets for UroGen’s product candidates and its ability to serve those markets; the rate and degree of market acceptance of UroGen’s product candidates vis-à-vis alternative or existing therapies; UroGen’s expectations regarding regulatory requirements; developments in applicable regulatory regimes; and the manner in which UroGen intends to use its cash resources and the sufficiency thereof. Moreover, UroGen operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. It is not possible for UroGen’s management to predict all risks, nor can UroGen assess the impact of all factors on its business or the extent to which any such factor or combination of factors may cause actual results to differ materially from those contained herein. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed herein may not occur, and UroGen’s actual results could differ materially and adversely from those anticipated or implied by the forward-looking statements contained herein. Except at required by law, UroGen undertakes no obligation to update any such forward-looking statements after the date hereof to conform to actual results or changes in UroGen’s expectations.
**Need**: Lack of FDA-Approved Drugs in Urinary Tract Diseases

**Proposed Solution**: Transform Local Therapies in Urology

- **UTUC**: Upper Tract Urothelial Carcinoma
  - No drugs approved by FDA

- **NMIBC**: Non-Muscle Invasive Bladder Cancer
  - No drugs approved in over 15 years by FDA

- **Kidney Cancer**
  - Several drugs approved by FDA

- **Prostate Cancer**
  - Several drugs approved by FDA
Urinary Tract Physiology Limits Drug Exposure

- Constant urine creation
- Bladder and upper tract movement
- Voiding

Drug washed out before it has a chance to work properly
Urinary Tract Anatomy Limits Surgical Therapy

- Renal pelvis anatomy makes it hard to see and reach all tumors
- Not all bladder tumors are easily seen, making complete resection difficult
Current Standard of Care for Low-Grade UTUC and NMIBC

**Low-Grade UTUC Current Treatment**
- High rates of recurrences
- Resection of visible & accessible tumors only
- Multifocal & inaccessible tumors

**Low-Grade NMIBC Current Treatment**
- High rates of recurrence
- Resection of visible tumors
- Adjuvant chemo follow up

**Repeated cycle of surgical therapy in urology**
Our Goal is to Transform Local Therapies in Uro-Oncology, Making Drug Therapy a First-Line Treatment Option

- Tumor surgical procedures often have limited success due to the inability to properly identify, reach and resect all tumors; Patients avoid associated potential complications
- An effective chemoablation agent can potentially provide better eradication of tumors irrespective of the detectability and location of the tumors
Transforming Local Therapies in Urology

Designed to expand the therapeutic uses of locally delivered drugs to the urinary tract by novel sustained release formulations

- **Mitomycin C for Low-Grade UTUC**
  - Adjuvant therapy post surgical removal of tumors

- **Botox for Overactive Bladder**
  - Multiple injections into bladder

- **MitoGel for Low-Grade UTUC**
  - Chemoablation of tumors instead of surgery
  - Unmet medical need
  - IND accepted, commenced pivotal, open-label, single-arm Phase 3 trial in 1Q17
  - Orphan Drug Designation

- **BotuGel for Overactive Bladder**
  - Single instillation into the bladder
  - Exclusive license deal with Allergan
  - Up to $225 million ($17.5 million upfront and $207.5 million milestones) and tiered royalties on net sales
  - Based on preclinical studies
  - Validates UroGen platform for a non uro-oncological indication
# Experienced Management Team in Drug Development and Urology

<table>
<thead>
<tr>
<th>Name</th>
<th>Roles and Experience</th>
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<tbody>
<tr>
<td><strong>Arie Belldegrun, M.D.</strong></td>
<td>Chairman, Kite Pharma: Chairman, CEO, President, Founder; UCLA: Professor of Urology, Director, Institute of Urologic Oncology; Cougar Biotechnology: Founder, Vice Chairman-BoD, Chairman-SAB; Agensys: Chairman-BoD, Founder</td>
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<tr>
<td><strong>Ron Bentsur</strong></td>
<td>Chairman, Keryx Biopharmaceuticals: CEO, Director, CFO; XTL Biopharmaceuticals: CEO, Director; ING Barings Furman Selz Investment Banking</td>
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<tr>
<td><strong>Gil Hakim</strong></td>
<td>President, Israeli Operation, Keryx Biopharmaceuticals: CEO, Director, CFO; Omrix Biopharmaceuticals; J&amp;J (Biosense-Webster)</td>
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<tr>
<td><strong>Mark Schoenberg, M.D.</strong></td>
<td>Medical Director, University Professor and Chairman of Urology Department at Montefiore Medical Center for Albert Einstein School of Medicine; Former Director of Urologic Oncology at Johns Hopkins</td>
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## Accomplished Board of Directors

<table>
<thead>
<tr>
<th>Name</th>
<th>Positions and Roles</th>
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<tbody>
<tr>
<td>Arie Belledegrun, M.D.</td>
<td>Kite Pharma: Chairman, CEO, President, Founder</td>
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<td></td>
<td>UCLA: Professor of Urology, Director, Institute of Urologic Oncology</td>
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<tr>
<td></td>
<td>Cougar Biotechnology: Founder, Vice Chairman-BoD, Chairman-SAB</td>
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<tr>
<td></td>
<td>Agensys: Chairman-BoD, Founder</td>
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<tr>
<td>Kate Falberg</td>
<td>Jazz Pharmaceuticals PLC: EVP and CFO</td>
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<td></td>
<td>Amgen Inc.: SVP, Finance and Strategy and CFO, VP, Chief Accounting Officer, and VP, Treasurer</td>
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<td></td>
<td>Director: BioMarin Pharmaceutical, Aimmune Therapeutics, aTyr Pharma, The Trade Desk, Meditation, Axovant</td>
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<tr>
<td>Stuart Holden, M.D.</td>
<td>Chairman of ProQuest Investments’ Scientific Advisory Board</td>
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<td>UCLA: Professor of Urology, Spielberg Family Chair in Urologic Oncology</td>
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<tr>
<td></td>
<td>Associate Director of the UCLA Institute of Urologic Oncology</td>
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<td>Chaim Hurvitz</td>
<td>CEO of CH Health</td>
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<td>Director: Teva; Aposense, NeuroRx, Emerald Medical Applications Corp</td>
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<td>Chairman: Polypid, Galmed Pharmaceuticals</td>
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<td>Ran Nussbaum</td>
<td>Co-founder of The Pontifax Group</td>
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<td></td>
<td>Director: Kite Pharma, cCAM Biotherapeutics, Eloxx, Nutrinia, Quiet Therapeutics</td>
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<td>Chairman: Keros, OCON Medical, NasVax</td>
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<td></td>
<td>CEO: Biomedix and Spearhead</td>
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<tr>
<td>Pini Orbach, PhD</td>
<td>Head of Pharma and Life Science at Arkin Holdings</td>
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<td>CEO: NanoDoc Technology</td>
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<td>Director: HealOR, Metallo-Therapy, FusiMab, Quiet Therapeutics</td>
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<td>ING Barings Furman Selz Investment Banking</td>
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<tr>
<td>Fred Cohen, M.D.</td>
<td>TPG: Senior Advisor and former Partner</td>
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<td>Founder, TPG Biotechnology</td>
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<td>UCSF</td>
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## Product Pipeline

<table>
<thead>
<tr>
<th>Preclinical</th>
<th>Phase 1</th>
<th>Phase 2</th>
<th>Phase 3</th>
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<tbody>
<tr>
<td>MitoGel: LG (1) UTUC (Phase 3)</td>
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<tr>
<td>VesiGel: LG Bladder Cancer (Phase 2a)</td>
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<tr>
<td>Vesimune: Bladder Cancer CIS (2) (Phase 1b)</td>
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<tr>
<td>BotuGel: OAB (3)</td>
<td>Exclusive license agreement</td>
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### Next Steps

- Continue Ph 3
- Planned Ph 2b in 1H18
- Planned Ph 2 in 2H18 combination therapies
- Planned Ph 2 in 2H17 by Allergan

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(1) LG: Low Grade. (2) CIS: Carcinoma in Situ. (3) OAB: Overactive Bladder.
Uro-Oncology: Significant Market Opportunity

**BLADDER**
- U.S. Pr. = 580,000
- U.S. In. = 80,000

**UPPER TRACT**
- U.S. Pr. = 45,000
- U.S. In. = 7,500

- LG NMI 32%
- MI 60%
- HG NMI 8%

LG=Low Grade; HG=High Grade; MI=Muscle Invasive; NMI=Non Muscle Invasive; Pr.=Prevalence; In.=Annual Incidence
Our Innovation Applied to Urology

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

1. Room temperature
2. Cooling
   - Low temperature
   - Liquid form
3. During instillation:
   - Low temperature
   - Liquid form
4. In the bladder:
   - Body temperature
   - Gel form

\(^{(1)}\) RTGel: Reverse Thermal Gelation Hydrogel.
Our Innovation Applied to Urology

- Drug administered as liquid and solidifies at target
- Longer drug dwell time to extend contact with tissue
- Novel formulation to enable higher drug solubility
- Can overcome physio-anatomical obstacles of the urinary tract
- Maintains drug’s mode of action
MitoGel for LG UTUC

<table>
<thead>
<tr>
<th>Novel RTGel / MMC sustained release formulation</th>
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<tbody>
<tr>
<td>Orphan Drug Designation for UTUC</td>
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<tr>
<td>Potential first-line chemoablation treatment of low-grade UTUC</td>
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</table>
MitoGel: Clinical Proof of Concept in UTUC

UTUC Compassionate Use Program

• Localized treatment of hard to resect tumors; extensive tumor burden; some with solitary kidney
• Conducted in the United States, Europe and Israel
• >130 instillations performed
• Observed to be generally well-tolerated and feasible to administer

<table>
<thead>
<tr>
<th>Patients with UTUC treated with MitoGel</th>
<th>22</th>
</tr>
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<tbody>
<tr>
<td>Biopsy confirmed low-grade</td>
<td>18</td>
</tr>
<tr>
<td>Completed six weekly instillations</td>
<td>14</td>
</tr>
<tr>
<td>Evaluable</td>
<td>13</td>
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</tbody>
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Response to MitoGel at PDE\(^{(1)}\)

<table>
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<tr>
<th>Complete response</th>
<th>8</th>
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<tbody>
<tr>
<td>Partial response</td>
<td>5</td>
</tr>
</tbody>
</table>

• 100% response to MitoGel therapy at PDE time point
• Based on clinical results to date, chemoablation with MitoGel appears safe and feasible in UTUC
MitoGel: Pivotal Trial Design and Expected Timing

<table>
<thead>
<tr>
<th>Trial Starts</th>
<th>Recruiting and Treating Patients</th>
<th>End Recruitment, Primary Endpoint</th>
<th>NDA Filing&lt;sup&gt;(1)&lt;/sup&gt;</th>
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<tbody>
<tr>
<td>2017</td>
<td></td>
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<td>2018</td>
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**Design:**
- Single pivotal, open-label, single-arm, Phase 3 trial

**Patient Group:**
- Patients with low-grade UTUC

**Number of Patients:**
- ~70 patients

**Treatment Regimen:**
- Six weekly instillations of MitoGel

**Primary Efficacy Endpoint:**
- CR<sup>(2)</sup> at ~4 weeks after last instillation
- Patients with CR will be:
  - Followed for durability
  - Treated with MitoGel monthly for up to 12 months of maintenance therapy

**Regulatory Pathway:**
- 505(b)(2)

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<sup>(1)</sup> Only if clinical trial is successfully completed; <sup>(2)</sup> CR: Complete Response
Potential first-line chemoaulation treatment of low-grade NMIBC

Novel RTGel / high dose MMC sustained release formulation

Potential alternative to TURBT
Chemoablation with VesiGel: Proof of Concept

**Phase 2a**

- **VesiGel 0.06% (40mg):** 45.0% (20 pts)
- **VesiGel 0.12% (80mg):** 86.4% (22 pts)
- **VesiGel 0.2% (120mg):** 83.3% (12 pts)

**Dose Escalation (DE)**

- **VesiGel 0.12% (80mg):** 86.4%
- **VesiGel 0.2% (120mg):** 83.3%

**Patient Group:**
- Patients with low-grade NMIBC

**Number of Evaluable Patients:**
- Phase 1: 15 (not shown)
- Phase 2a: 65
- DE: 12 with LG

**Treatment Regimen:**
- Six weekly instillations
- No TURBT

**Primary Efficacy Endpoint:**
- CR at ~4 weeks after last instillation

- **86%** complete response with VesiGel 0.12% (80mg)
- Using higher doses may not increase efficacy
VesiGel: Potential Alternative to TURBT

Eligibility for TURBT:
- Tumor size: > 1cm
- Multifocal: > 3 tumors

Shortcomings of TURBT:
- Nonvisible lesions
- Incomplete resection
- Hospitalization
- Anesthesia

VesiGel 0.12% vs. Active Control (MMC 0.1%):
- Higher CR rates in TURBT eligible group

VesiGel can allow for increased coverage of the bladder tissue, designed to overcome the shortcomings of TURBT surgery.
VesiGel: Durability of Response in Clinical Program

% of patients treated with VesiGel with complete response (CR) over 12 months

- ~80% durability at 12 months
- No additional treatments were given during this period
Vesimune for CIS Bladder Cancer

| Novel Imiquimod formulation for bladder instillation |
| Orphan Drug Designation for CIS bladder cancer |
| Potential local immunotherapy for the treatment of CIS bladder cancer |
Vesimune: Preliminary Proof-of-Concept Data

Patient Group:
- Patients with NMIBC

Number of Evaluable Patients:
- DE: 23 (not shown)
- Phase 1: 10 (under U.S. IND)

Treatment Regimen:
- Six weekly instillations
- No TURBT to CIS patients

Primary Efficacy Endpoint:
- CR at 5-7 weeks post last instillation

- Novel local immunotherapy drug for high-grade NMIBC
- Potential synergism with immune checkpoint inhibitors
Data Presentations and Medical Meetings

Results of UroGen’s studies and trials presented at international urology meetings and published in peer reviewed journals
## Broad Potential Applications Supporting Further Growth

### Uro-Oncology

<table>
<thead>
<tr>
<th>Low-Grade</th>
<th>High-Grade</th>
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<tbody>
<tr>
<td><strong>VesiGel</strong></td>
<td><strong>Vesimune</strong></td>
</tr>
<tr>
<td>Ph 2 pending</td>
<td>Ph 1b complete</td>
</tr>
<tr>
<td><strong>MitoGel</strong></td>
<td>Orphan</td>
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<tr>
<td>Ph 3</td>
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### Other Urology

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<tr>
<th>BotuGel</th>
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<tr>
<td>Overactive Bladder</td>
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<tr>
<td>Ph 2 pending</td>
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</tbody>
</table>

### Potential Uses

- **Gastrointestinal Tract**
- **Female Reproductive System**
- **Urology**
- **BotuGel**
- Allergan License Agreement

### Preclinical Candidates

- High-Grade Urothelial Carcinoma
  - Ph 2 pending

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**Notes:**
- Low-Grade Uro-Oncology
- High-Grade Uro-Oncology
- Orphan
- Ph 1b complete
Broad IP Coverage in Five Interrelated Disciplines

- **Pharmaceutical Composition** (leading drug candidates protected)
- **Delivery System for Bladder and Upper Urinary Tract**
- **Methods of Treatment**
- **Combination Therapy**
- **Manufacturing Process**

**60+ patents and patent applications**

- 11 granted in United States
- 15 granted in other jurisdictions
Our Strategy

- To become a leader in the treatment of urothelial cancers and other urological indications
- Achieve first ever FDA drug approval for the treatment of UTUC (Orphan Drug Designation granted)
- Change treatment paradigm for low-grade bladder cancer
- Enable treatment of high-grade urothelial carcinoma with immunotherapy drug, custom made for urology
Thank you