Fueling life sciences through transformative transactions

CALIFORNIA LIPAC PARTNERING OPPORTUNITY IN CHINA

ChinaBio 2018 Conference

March 2020
Forward Looking Statements

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

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 LIPAC, including with respect to the efficacy and safety of LIPAC’s product candidates; LIPAC’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 LIPAC’s product candidates; LIPAC’s ability to obtain and maintain intellectual property protection for its product candidates; LIPAC’s anticipated growth strategies; LIPAC’s expectations regarding competition; the anticipated trends and challenges in LIPAC’s business and the markets in which it operates; LIPAC’s ability to attract or retain key management and personnel; the size and growth of the potential markets for LIPAC’s product candidates and its ability to serve those markets; the rate and degree of market acceptance of LIPAC’s product candidates vis-à-vis alternative or existing therapies; LIPAC’s expectations regarding regulatory requirements; developments in applicable regulatory regimes; and the manner in which LIPAC intends to use its cash resources and the sufficiency thereof. Moreover, LIPAC operates in a very competitive and rapidly changing environment in which new risks emerge from time to time. It is not possible for LIPAC’s management to predict all risks, nor can LIPAC 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 LIPAC’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, LIPAC undertakes no obligation to update any such forward-looking statements after the date hereof to conform to actual results or changes in LIPAC’s expectations.
LIPAC Oncology Leadership Team

Dr. Ramachandran “TR” Thirucote, Ph.D.
Chief Executive Officer
- 30+ years of pharmaceutical experience
- Held positions at RoxRo, SRI, Agouron, and Thermedics

Will Robberts
President & Chief Financial Officer
- 20+ years of experience in finance, M&A and operations in pharma and other industries
- Held VP/C-level leadership positions at Thomson Reuters, KPMG, TesoRx Pharma, LiveNote, and SoftLine

Dr. Michael Oefelein, M.D., FACS
Chief Medical Officer
- 15+ years of experience in the pharmaceutical and medical device industries
- Held leadership positions at Allergan and Digirad

Past Commercial Success

Scientific Advisors

Dr. Shigeo Horie, M.D., Ph.D.
Scientific Advisor
- Chairman of the Department of Urology at Juntendo University in Tokyo
- Expert of Oncology, Men’s Health, Anti-Aging Medicine, and Genetics

Dr. Guru Betageri, Ph.D.
Scientific Advisor
- Inventor of proprietary proliposomal technology
- Professor of Pharmaceutical Sciences at Western University of Health Sciences
Introducing LIPAC Oncology

- LIPAC is a clinical stage pharmaceutical company employing LiPax™ (“LiPax”), its proprietary proliposomal intravesical paclitaxel drug delivery platform, to enhance and reformulate proven cancer drugs into more effective treatments.
- LiPax, a locally delivered formulation of the paclitaxel, is being developed for non-muscle invasive bladder cancer and other intracavitary cancer indications.
- LiPax has strong IP with formulation and patent coverage until 2037.

LiPax Pipeline

<table>
<thead>
<tr>
<th>Program</th>
<th>Preclinical</th>
<th>Ph 1</th>
<th>Ph 2</th>
<th>Ph 3</th>
</tr>
</thead>
<tbody>
<tr>
<td>TBC-1002 Non-Muscle Invasive Bladder Cancer (NMIBC)</td>
<td></td>
<td></td>
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<td></td>
</tr>
<tr>
<td>UTUC-1005 (Orphan) Upper Tract Urothelial Carcinoma (UTUC)</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>LEIPC-1007 Stage II/III Ovarian Cancer (OC)</td>
<td></td>
<td></td>
<td></td>
<td></td>
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<tr>
<td>LEIPC-1008 Intraperitoneal Carcinoma</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>LEITP-1009 Mesothelioma / Malignant Pleural Effusion</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>
I. Limitations in Bladder Cancer Treatment Today
Today, a NMIBC diagnosis is devastating and crippling as...

- Recurrence rates are high
- Systemic toxicities are prevalent and result in significant patient discomfort
- Dose limiting toxicities result in poorer patient outcomes
- Therapies cause intense urinary burning and pain
- Treatments are not optimized or formulated for NMIBC
- Leading drugs are in short supply

But, LIPAC is revolutionizing the treatment space with LiPax, a treatment...

- Without the pain
- Which delivers high concentrations of an extremely potent and proven chemotherapy
- Without severe systemic toxicities or side effects
- Formulated to persist and penetrate into the urothelium
- With targeted lethality which reduces recurrences and improves patient outcomes
- In development for additional underserved intracavitary cancer indications
NMIBC – A Large and Underserved Market

Stages of Bladder Cancer

Large Market Potential for Bladder Cancer

- **Bladder Cancer is the 6th most common cancer in the U.S. with ~700,000\(^2\) patients diagnosed**
  - 4th most common cancer in men and three-times more prevalent in men than in women\(^3\)
  - ~18,000 patients die each year in the U.S.\(^2\)
- 74\(^1\) of all bladder cancer is non-muscle invasive bladder cancer
  - NMIBC affects over 2 million\(^1,4\) patients globally and over 520,000\(^1,2\) patients in the U.S.
  - Highly recurrent with a 31% - 78\(^4\) five-year recurrence rate

High Financial and Emotional Burden of Existing Treatments

- Due to ongoing diagnostic and therapeutic requirements for the recurrent disease, as well as for disease progression, **bladder cancer is expected to remain the most expensive cancer to treat**\(^5\)
- **No new products in over two decades** and no approved products for intermediate-risk NMIBC
- Critical supply shortages, product rationing and price hikes of BCG and MMC limiting access

II. LIPAC – A Unique, Effective, and Risk-Mitigated Solution
LiPax Technology Overview

1. Formulation components self-assemble into liposomes upon addition of sterile water diluent

2. Self-assembly is driven by physiochemical properties of surfactant to maximize volume and minimize surface area

3. The API, paclitaxel, intercalates in the lipid rich region of the phospholipid

4. Random motion results in liposomes fusing with the urothelial wall

5. Paclitaxel is off-loaded into the target tissue and penetrates deep into the bladder wall

Liposomes can interact with cells by four different mechanisms. Potentially more than one mechanism can be operative at a time.

Overview of Liposome-Cell Interactions
Advantages of LiPax

LiPax overcomes significant challenges of current standards of care

**Advantages of LiPax**

- Penetration
- Persistence
- Closed System
- Effective Formulation
- POC Achieved
- Known and Proven Paclitaxel API

<table>
<thead>
<tr>
<th>LiPax</th>
<th>Current Treatments</th>
</tr>
</thead>
<tbody>
<tr>
<td><img src="image1" alt="Initial installation" /></td>
<td><img src="image2" alt="Initial installation" /></td>
</tr>
<tr>
<td><img src="image3" alt="First voiding after installation" /></td>
<td><img src="image4" alt="First voiding after installation" /></td>
</tr>
<tr>
<td><img src="image5" alt="Days after installation" /></td>
<td><img src="image6" alt="Days after installation" /></td>
</tr>
</tbody>
</table>

LiPax achieves persistence and penetration for a sustained period after treatment
## LiPax is a Novel Next Generation Formulation of Paclitaxel

LiPax formulation permits superior localized delivery of paclitaxel without systemic toxicity

<table>
<thead>
<tr>
<th>Generation</th>
<th>Formulation</th>
<th>Side Effects</th>
<th>Remarks</th>
</tr>
</thead>
<tbody>
<tr>
<td>1&lt;sup&gt;st&lt;/sup&gt; Generation – Taxol ($1.6B Revenue)</td>
<td>Significant Cremaphor formulation side effects</td>
<td></td>
<td></td>
</tr>
<tr>
<td>2&lt;sup&gt;nd&lt;/sup&gt; Generation – Abraxane™ ($1.2B+ Revenue)</td>
<td>Nano-particle formulation non-optimized solubility and off-loading kinetics</td>
<td></td>
<td>Approved in 2005 through the 505(b)(2) regulatory pathway</td>
</tr>
<tr>
<td>3&lt;sup&gt;rd&lt;/sup&gt; Generation – LiPax ($B+ Revenue Potential)</td>
<td>30x more active against T24 bladder cancer cells than unformulated paclitaxel&lt;sup&gt;1&lt;/sup&gt;</td>
<td>Low blood count, peripheral neuropathy, sepsis, breathing problems, etc.</td>
<td>Enhanced solubilization in acidic environment, Lipophilic and adheres to bladder wall, Optimized off-load kinetics and intratumoral concentration relative to Abraxane™, Enhanced tolerability, Known to regulatory authorities around the world with 505(b)(2) pathway agreed in the USA</td>
</tr>
</tbody>
</table>

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To date, there have been no reported adverse events due to the drug

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III. Clinical Overview & Regulatory Pathway
LiPax has shown enhanced activity against the bladder cancer cell line while maintaining no systemic exposure or toxicity

Proof of Formulation

Activity Against T24 Bladder Cancer Cell Line

~30x difference in IC$_{50}$

LiPax:
Concentration needed to kill 50% of cancer cells

Micelle formulation:
Concentration needed to kill 50% of cancer cells

LiPax shows 30x$^1$ enhanced activity against bladder cancer cell line vs micelle formulation of paclitaxel


$^1$
Proof of Better Penetration than the Standard of Care

LiPax was found significantly more effective in penetrating urothelial tissue to target tumors as compared to MMC and Abraxane™

**Human MMC Bladder Penetration Study**

- Bladders were removed from patients treated with intravesical MMC (N=24)
- MMC concentrations were measured at various thicknesses of the bladder wall

**Ex-Vivo Male Porcine Penetration Study**

<table>
<thead>
<tr>
<th>Cumulative thickness (µm) of the bladder sections</th>
<th>Amount of Paclitaxel in tissue (µg)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Shallow</td>
<td>LiPax</td>
</tr>
<tr>
<td>Deep</td>
<td>Abraxane™</td>
</tr>
<tr>
<td>500</td>
<td></td>
</tr>
<tr>
<td>1,500</td>
<td></td>
</tr>
<tr>
<td>2,500</td>
<td></td>
</tr>
<tr>
<td>3,500</td>
<td></td>
</tr>
<tr>
<td>4,500</td>
<td></td>
</tr>
<tr>
<td>Remainder of bladder</td>
<td></td>
</tr>
</tbody>
</table>

Methodology

- 7 patients with low detectable levels of MMC
- 17 patients with no detectable levels of MMC
Proof of Efficacy: Nude Mice Model

Summary

- LiPax achieved a 56% complete response rate with no detectable systemic exposure and toxicity
- LiPax demonstrated high lethality against bladder cancer cells
- No paclitaxel detected in tail vain of nude mouse indicating no systemic exposure
**NMIBC Clinical Program Status**

**PHASE 1:** Dose Escalation to Assess Maximum Tolerable Dose (MTD)

- **COHORT 1**
  - 3 Patients
  - Doses: 10, 25, 50, 75, 100, 150 mg

- **COHORT 2**
  - 3 Patients
  - Doses: 90, 180, 270, 360, 450, 540 mg

**PHASE 2:** Dose Response

- **MARKER LESION STUDY**
  - 10 Patients (12 weeks)
  - TURBT surgery followed by 6 weekly installations @ MTD
  - At week 12: Assess Marker Response Rate via Cystoscopy / Biopsy

**Current Status**

- Part 1 completed with promising results
- Phase 2 marker lesion study started in August 2019 and is expected to be completed by June 2020, and will serve as proof of efficacy
- As of February 26, 2020 ~60% enrollment
NMIBC Phase 1 Data Indicative of Significant Efficacy with LiPax Administration

Number of Months That Patients Have Remained Recurrence Free

<table>
<thead>
<tr>
<th>Subject</th>
<th>Months</th>
<th>Low-Risk Patients</th>
<th>Intermediate-Risk Patients</th>
</tr>
</thead>
<tbody>
<tr>
<td>Subject 1</td>
<td>~22 months</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Subject 2</td>
<td>~20 months</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Subject 3</td>
<td>~20 months</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Subject 4</td>
<td>~13 months</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Subject 5</td>
<td>~10 months</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Subject 6</td>
<td>~10 months</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Safety Data

- No dose limiting toxicities observed in 6 low-intermediate patients dosed with LiPax after TURBT bi-weekly for up to 6 doses
- No reduction in urinary HRQOL observed
- Undetectable paclitaxel systemic side effects despite high and dose proportional urinary levels of paclitaxel
- No transitional cell carcinoma recurrences observed to date

No patients have experienced recurrence of NMIBC since LiPax treatment while intermediate NMIBC patients recur 31%\textsuperscript{1} at one year and 50%\textsuperscript{1} at two years after TURBT and intravesical therapy

Marker Lesion Studies are Endorsed by the FDA and Have Proven Predictive Capability

- Marker lesion studies have proven to be an effective predictor of two-year patient outcomes as demonstrated by the Phase 2 study of apaziquone for patients with NMIBC
  - In the study, the CR of the marker lesion in 67% of patients was followed by a recurrence-free rate of 56.5% at 1-year follow up and 49.5% at 2-year follow up\(^1\)
- Study results will be available by June 2020

Favorable Regulatory Pathway

LiPax is targeting low- and intermediate-risk NMIBC, highly underserved indications with significant patient pools and clear regulatory paths forward

**NMIBC Patient Population by Risk Profile**

- **Low-risk** 50%
- **Intermediate-risk** 35%
- **High-risk** 15%

- BCG-refractory is ~40% of High-risk NMIBC (or 6% of all NMIBC)
- Most of the competition in NMIBC is targeting BCG-refractory due to potential fast-track approval via orphan designation but it is crowded and API shortages lead to slow enrollment

**Low / Intermediate-Risk Pathway Advantages**

- **Large Market Opportunity**: Majority of bladder cancer patients are low- and intermediate- risk
- **Lack of Therapies**: No drugs approved for low- and intermediate-risk NMIBC
- **Accelerated Approval**: Potential for breakthrough therapy designation from the FDA
- **Simplified Study Design**: Head-to-head study feasible
- **Faster Enrollment**: Supply shortages of BCG create study enrollment restrictions for BCG-refractory studies

**Phase 2b / 3 Trial Overview**

- **Timeframe**: 2020-2023
- **Study Design**: Head-to-head superiority / comparator trial (LiPax vs. Thiotepa)
- **Enrollment Size**: 350-450 patients
- **Endpoint**: 2-year RFS
  - Positive study = 10% or greater (versus > 30% CR in BCG refractory trials)

**Source**: 1. European Urology-Accredited Continuing Medical Education. 2. NMIBC – BCG Refractory Disease and Use of Interferon (Goonewardene, 2019).
BCG-Refractory NMIBC Landscape

BCG-Refractory NMIBC is Targeted Because of Potential Fast-Track Approval… However, There are Many Drawbacks

- **FDA Guidance and Simplified Study Design:** Single-arm Phase 2 design
  - Small number of patients (n~150) and a single registration study
- **Accelerated Approval:** Potential for Breakthrough Therapy designation from the FDA

**Small Market Opportunity:** Only ~6% NMIBC is BCG-refractory

**Slow Enrollment:** Supply shortages of BCG create study enrollment restrictions as patient population dependent on BCG drug supply

**Crowded Indication:** Many products are in development or near approval for BCG-refractory NMIBC

BCG-Refractory NMIBC Has a Small Patient Pool\(^1,2\)... … And is an Overcrowded Market with Limited Efficacy

- BCG-refractory standard of care is radical cystectomy with urinary diversion / bladder conservation
  - Gemcitabine / docetaxel is used off-label

32,000 Patients

16,000 Patients

8,000 Patients

Maximum peak sales for new BCG-refractory agents is under $200M in the U.S.

- ~6% of NMIBC patients are BCG-refractory
  - Only ~50% of patients are eligible for treatment as many refuse surgical treatment or are not medically fit for cystectomy
  - 3-month failure rate for most treatments is ~50%

**Keytruda\(^\text{TM}\)**

**Admin. Route:** Intravenous every 3 weeks

**Systemic AE:** Yes

**Immune AE:** Yes

**Serious AE:** Yes (Death)

**12 Month CR:**
- 40% Ta
- 19% CIS +/- Ta/T1
- 24% CIS +/- Ta/T1
- ~40% Ta

**MoA:** PD-1 inhibitor

---

**FerGene / Instilladrin\(^\text{TM}\)**

**Admin. Route:** Intravesical every 3 months

**Systemic AE:** No

**Immune AE:** No

**Serious AE:** No (Liver and renal failure)

**12 Month CR:**
- 44% Ta

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**Vicinium\(^\text{TM}\)**

**Admin. Route:** Intravesical; frequency pending on treatment phase

**Systemic AE:** Yes

**Immune AE:** No

**Serious AE:** Yes (Death)

**12 Month CR:**
- Not available

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**ALT-803**

**Admin. Route:** SQ or intravesical every 1-3 weeks

**Systemic AE:** Yes

**Immune AE:** Yes

**Serious AE:** Yes (Death)

**12 Month CR:**
- Not available

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**Opdivo\(^\text{TM}\)**

**Admin. Route:** Intravenous every 3 weeks

**Systemic AE:** Yes

**Immune AE:** Yes

**Serious AE:** Yes (Death)

**12 Month CR:**
- Not available

---

**MoA:**
- PD-1 inhibitor
- Interferon gene
- ABC: EpCAM-toxin
- IL-15 protein super agonist
- PD-L1 inhibitor

Sources: 1. European Urology-Accredited Continuing Medical Education. 2. NMIBC – BCG Refractory Disease and Use of Interferon (Goonewardene, 2019).
Quantitative survey of 100 urologists in the US supports LiPax as a frontline therapy for NMIBC

Market study suggests that US urologists recommend LiPax to replace 39% to 71% of therapies for all risk stages of NMIBC
IV. Competitive Landscape
The current post-TURBT drug treatment regimens are **lacking** and have **severe shortcomings** for both patients and healthcare providers.

<table>
<thead>
<tr>
<th></th>
<th>BCG* ($150 / dose)¹</th>
<th>Mitomycin C ($1,400 / dose)²</th>
<th>Thiotepa ($1,600 / dose)²</th>
<th>Valrubicin ($5,000 / dose)³</th>
<th>LiPax™</th>
</tr>
</thead>
<tbody>
<tr>
<td>Indicated for 1st Line NMIBC</td>
<td>✗</td>
<td>✗</td>
<td>✗</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>Low- and Intermediate-Risk Patients</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>No Fume Hood Required</td>
<td>✓</td>
<td>✗</td>
<td>✗</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>Adequate Supply</td>
<td>✗</td>
<td>✗</td>
<td>✓</td>
<td>✓</td>
<td>✓</td>
</tr>
<tr>
<td>No Dose Limiting Toxicities</td>
<td>✗</td>
<td>✗</td>
<td>✓</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>No Systemic Toxicities</td>
<td>✗</td>
<td>✗</td>
<td>✓</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>Demonstrated Deep Penetration Into</td>
<td>Unknown</td>
<td>✗</td>
<td>✓</td>
<td>✗</td>
<td>✓</td>
</tr>
<tr>
<td>the Bladder Wall</td>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*BCG is hard to procure due to limited supply.  
3. [https://www.accessdata.fda.gov/drugsatfda_docs/label/2011/020892s013lbl.pdf](https://www.accessdata.fda.gov/drugsatfda_docs/label/2011/020892s013lbl.pdf)
**LIPAC is an Attractive, De-Risked Investment Opportunity With Strong Competitive Advantages and Near-Term Value Milestones**

**Competitive Landscape Overview**

<table>
<thead>
<tr>
<th>Commercial Attributes</th>
<th>Clinical Attributes</th>
</tr>
</thead>
<tbody>
<tr>
<td>High</td>
<td>Low</td>
</tr>
<tr>
<td>RTGel™ (Pipeline)</td>
<td>MMC (Marketed)</td>
</tr>
<tr>
<td>LiPax</td>
<td>Thiotepa (Marketed)</td>
</tr>
<tr>
<td>RTGel™ (Pipeline)</td>
<td>Apaziquone (Pipeline)</td>
</tr>
</tbody>
</table>

**Main Competitor Analysis**

- **RTGel™**, LiPax’s main competitor in the low- and intermediate-risk NMIBC space, has significant clinical short comings
  - **Less Effective**: Unformulated MMC 40mg had higher CR rate than 40mg MMC RTGel™
  - **API Undesirable**: MMC has poor penetration leading to less effective patient outcomes and is not efficacious for metastatic bladder cancer
  - **Patient Discomfort**: Serious adverse events have been reported including rash, burning sensation, urgency in urination, and pain during urination

Notes: 1. Does not include BCG-refractory and high-risk treatments including Keytruda, Tecentriq, CAVATAK, ABI-009, VALSTAR, Vicinium, INSTILADRIN, CG-0070 and N-803. 2. BCG is approved for the treatment of high-grade papillary NMIBC.
## LiPax has Significant Advantages Over UroGen’s RTGel™

### UroGen’s RTGel™ is the main competitor to LiPax for low- to intermediate-risk NMIBC

<table>
<thead>
<tr>
<th></th>
<th>LiPax</th>
<th>RTGel™</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Active product ingredient</strong></td>
<td>Paclitaxel</td>
<td>Mitomycin C (MMC)</td>
</tr>
<tr>
<td><strong>API active against metastatic bladder cancer</strong></td>
<td>Yes, 40% response rate</td>
<td>No approved indication</td>
</tr>
<tr>
<td><strong>API stability in urine</strong></td>
<td>Stable</td>
<td>Degrades</td>
</tr>
<tr>
<td><strong>API $C_{urothelial} / C_{urine}$ partition coefficient</strong></td>
<td>0.5 (lipophilic)</td>
<td>0.02 (hydrophilic)</td>
</tr>
<tr>
<td><strong>Penetration of lamina propria</strong></td>
<td>Excellent</td>
<td>Poor</td>
</tr>
<tr>
<td><strong>Systemic exposure after intravesical instillation</strong></td>
<td>Not detected</td>
<td>Present</td>
</tr>
<tr>
<td><strong>Duration of intravesical persistence</strong></td>
<td>Up to 72 hours</td>
<td>Several hours</td>
</tr>
<tr>
<td><strong>Urinary bother / tolerability</strong></td>
<td>No change in patient reported outcome: OAB-q and IPSS; No dose limiting toxicity</td>
<td>Dysuria and frequency; dose limited toxicity at 0.2% MMC / RTGel™</td>
</tr>
</tbody>
</table>

**LiPax has demonstrated best-in-class potential in terms of formulation, penetration, and tolerability with responder rates to be established by 1Q 2020**

Concerns Surrounding UroGen’s Clinical Pipeline

UroGen has a Small Market Opportunity and Problematic Side Effects in UTUC Indication…

- Small universe of patients (~2,000 per year) significantly reduces commercial value
- API used in RTGel™, MMC, strictures ureter leading to severe patient side effects often requiring indwelling stent

With Deep Commercial and Clinical Concerns Around NMIBC Indications

- Lack of tumor removal is unappealing to physicians and results in multiple downstream consequences
- UroGen’s treatment plan contradicts medically accepted guidelines
- Superiority trial needed in order for urologists to change their standard of care resulting in increased costs, prolonged approval time, and decreased approval likelihood
V. Why Invest in LIPAC?
Significant Revenue Potential Driven by Large Unmet Need

Independent revenue forecast suggests ~$800M peak net revenue for NMIBC in the U.S.

LiPax Net Revenue ($M)$\textsuperscript{1}

- Total number of bladder cancer patients: 699,450$^2$
- 74%$^1$ of all bladder cancer is NMIBC
- Number of new NMIBC patients / year: 59,548$^{1,2}$
- Significant number of additional patients from multiple recurrence events
- Total NMIBC patients per year: 199,297

Forecast peak net revenue for NMIBC is projected to exceed $1.5 billion globally

Reference Point: Abraxane$^\text{TM}$ Net Revenue ($M)$\textsuperscript{3}

Significant Revenue Potential From 505(b)(2) Oncology Drug

Likelihood of Approval$^4$

Likelihood of approval is higher for non-new molecular entity (NME) drugs which often use the 505(b)(2) pathway to gain FDA approval

<table>
<thead>
<tr>
<th></th>
<th>Ph 1 to Approval</th>
<th>Ph 2 to Approval</th>
</tr>
</thead>
<tbody>
<tr>
<td>Oncology (all approval pathways)</td>
<td>5.1%</td>
<td>8.1%</td>
</tr>
<tr>
<td>All Indications (all approval pathways)</td>
<td>9.6%</td>
<td>15.3%</td>
</tr>
<tr>
<td>NME</td>
<td>6.2%</td>
<td>10.1%</td>
</tr>
<tr>
<td>Non-NME</td>
<td>22.6%</td>
<td>32.2%</td>
</tr>
</tbody>
</table>

Precedence for Public Market Support in NMIBC & UTUC Indications

UroGen has successfully raised $280M+ via the public markets to advance its clinical trials

<table>
<thead>
<tr>
<th>Event</th>
<th>Date</th>
<th>Details</th>
</tr>
</thead>
<tbody>
<tr>
<td>1</td>
<td>IPO</td>
<td>May-17</td>
</tr>
<tr>
<td>2</td>
<td>Jan-18 Public Offering</td>
<td>$64M raised</td>
</tr>
<tr>
<td>3</td>
<td>May-18 Market Cap peaked at</td>
<td>$1.1B</td>
</tr>
<tr>
<td>4</td>
<td>Oct-18 Commenced NMIBC Phase 2B Clinical Trial</td>
<td></td>
</tr>
<tr>
<td>5</td>
<td>Jan-19 Public Offering</td>
<td>$162M raised</td>
</tr>
<tr>
<td>6</td>
<td>Sep-19 Analyst Day</td>
<td></td>
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<tr>
<td>7</td>
<td>Dec-19 Announced FDA filing acceptance and priority review of NDA for UGN-101 for low-grade UTUC</td>
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</table>

Analyst Concerns Regarding UGN-102 (NMIBC)

“Recall, no ‘102 DOR data and little Ph 3 design info were provided at the Sep. 24th Analyst Day, sending the stock down 35%+ in the weeks following. Now, URGN is finalizing a H2H Ph 3 pivotal trial design of ‘102 vs TURBT w/ the 1EP of DOR at 12(+?) mos… We note that superiority would require a larger trial for sufficient statistical powering and reduced variability, potentially putting our assumed 2Q launch at risk. Importantly, we see added clinical risk and a smaller market for ‘102 than our pre-Analyst Day expectations. We now model peak, risk-unadjusted US sales of $205M vs $423M prev.”

LIPAC is the More Attractive Opportunity

✓ Utilizes more effective API with no supply issues or risk of price hikes
✓ Lipophilic properties lead to better bladder tissue penetration & persistence; no reported systemic toxicity and better patient outcomes
✓ Head-to-head study against Thiotepa aligns with and enhances standard of care

LIPAC is a Compelling Investment Opportunity with Multiple Significant Near-Term Value Milestones

## Summary Investment Highlights

<p>| | |</p>
<table>
<thead>
<tr>
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<tbody>
<tr>
<td><strong>1</strong></td>
<td>Differentiated Opportunity in Bladder Cancer with Clinical Success</td>
</tr>
<tr>
<td><strong>2</strong></td>
<td>Enhances the Standard of Care for Bladder Cancer</td>
</tr>
<tr>
<td><strong>3</strong></td>
<td>Large Unmet Need Drives Significant Revenue Potential</td>
</tr>
<tr>
<td><strong>4</strong></td>
<td>Strong Management Team with Extensive Industry Experience</td>
</tr>
<tr>
<td><strong>5</strong></td>
<td>Key Partnerships with Global Pharmaceutical Companies Offer Commercial Validation</td>
</tr>
</tbody>
</table>

## Key 2020 Value Inflection Points

<table>
<thead>
<tr>
<th>NMIBC</th>
<th>UTUC</th>
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<tbody>
<tr>
<td>Phase 2a marker lesion response data</td>
<td>Commence Phase 2b trial</td>
</tr>
<tr>
<td>Commence Phase 1/2a trial</td>
<td></td>
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</tbody>
</table>

<table>
<thead>
<tr>
<th>Ovarian Cancer</th>
<th>IPO &amp; International Licensing Deal</th>
</tr>
</thead>
<tbody>
<tr>
<td>File IND and commence Phase 1/2a trial</td>
<td></td>
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</table>

## Key 2021 Value Inflection Points

<table>
<thead>
<tr>
<th>NMIBC</th>
<th>UTUC</th>
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</thead>
<tbody>
<tr>
<td>Phase 2b dose response data</td>
<td></td>
</tr>
<tr>
<td>Phase 1/2a data</td>
<td></td>
</tr>
</tbody>
</table>

| International Licensing Deal |

## Key 2022 Value Inflection Points

<table>
<thead>
<tr>
<th>Ovarian Cancer</th>
<th>International Licensing Deal</th>
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</thead>
<tbody>
<tr>
<td>Phase 2 data</td>
<td></td>
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</tbody>
</table>

NMIBC NDA Filing in 2023
Appendix
## Recent Developments in the Bladder Cancer Space

<table>
<thead>
<tr>
<th>Recent Development</th>
<th>Commentary</th>
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<tbody>
<tr>
<td>▪ Ferring Pharmaceuticals announced spinout of FerGene</td>
<td>▪ Indicated for small patient pool</td>
</tr>
<tr>
<td>▪ FerGene to focus on development of nadofaragene firadenovec for high-grade, BCG-unresponsive NMIBC</td>
<td>▪ Potential for slow physician uptake as medical professionals have limited exposure to gene therapies and may be hesitant to prescribe over more familiar treatment methods</td>
</tr>
<tr>
<td>▪ Ferring and Blackstone Life Sciences announced $570+ million investment in FerGene</td>
<td></td>
</tr>
<tr>
<td>▪ ImmunityBio granted FDA Breakthrough Therapy Designation from the FDA for N-803 in combination with BCG for BCG-unresponsive NMIBC, CIS</td>
<td>▪ Indicated for small patient pool</td>
</tr>
<tr>
<td>▪ Sesen Bio initiated rolling submission of Biologics License Application to FDA for Vicinium for the treatment of BCG-unresponsive NMIBC</td>
<td>▪ Concerns around efficacy and side effects</td>
</tr>
<tr>
<td>▪ FDA granted accelerated approval to Seattle Genetics’ and Astellas’ PADCEV for people with locally advanced or metastatic urothelial cancer</td>
<td>▪ Approved ~3 months early</td>
</tr>
<tr>
<td>▪ FDA approved Merck’s Keytruda for BCG-unresponsive, high-risk NMIBC, CIS</td>
<td>▪ FDA panelists expressed concerns about efficacy, specifically the clinical meaningfulness of CR rates</td>
</tr>
<tr>
<td>▪ Roche’s Tecentriq failed a solo trial aimed at reducing the risk of cancer progressing or returning in patients with muscle-invasive urothelial cancer who had undergone surgery</td>
<td>▪ Safety concerns as Keytruda has severe side effects</td>
</tr>
<tr>
<td>▪ AstraZeneca announced that the Phase 3 trial for Imfinzi and Imfinzi plus tremelimumab in unresectable, Stage IV (metastatic) bladder cancer did not meet primary endpoints</td>
<td>▪ May alter Merck’s strategy with BCG production</td>
</tr>
<tr>
<td>▪ National Institute for Health and Care Excellence recommended against routine use of Keytruda for locally advanced or metastatic urothelial carcinoma</td>
<td>▪ In the phase 3 trial, Tecentriq did not show benefit when compared with simple observation at delaying the time to cancer recurrence or death, the study’s primary endpoint</td>
</tr>
</tbody>
</table>

Recent approvals and developments are in the overcrowded high-risk / BCG-unresponsive NMIBC space and do not address the large unmet need in low- / intermediate-risk NMIBC which LIPAC is targeting.

Sources: Company information and Wall Street research.
Upper Tract Urothelial Carcinoma

Overview

- Orphan disease with incidence of 5-10% of all urothelial carcinoma (5,000 – 10,000 cases / year)
- More aggressive than bladder cancer
- Standard of care is radical nephroureterectomy (RNU)
- No drugs are thought to be efficacious for the treatment of UTUC
- LiPax has demonstrated 345% greater drug concentration in porcine ureter study than MMC

Average Amount of Drug (µg) in Three Ureters per g of Tissue

Ex-Vivo study confirms LiPax has superior penetration into ureter vs. standard of care (MMC)

![Graph showing drug concentration comparison between Mitomycin C and LiPax.](image-url)
Stage II / III Ovarian Cancer

Ovarian cancer patients often have a poor prognosis with limited treatment options; LIPAC hopes to change that.

Overview

- Three RPCT have established that intraperitoneal instillation is superior to intravenous chemotherapy for patients with small volume residual disease after cytoreductive surgery.
- Barriers to current standards of care: (1) toxicity and (2) technical expertise with IP device.
- IP drug exposure much higher than can be delivered via intravenous/systemic route of administration:
  - >1000x for IP paclitaxel
  - >10-20 fold for cisplatin
- Overall survival advantage: 65.6 mo vs 49.7 mo, p=0.017

Concentration Required to Kill 50% of Cancer Cells

LiPax has demonstrated much greater potency against current chemotherapies for ovarian cancer:

<table>
<thead>
<tr>
<th>Drug</th>
<th>Mean IC50 (µg/ml) to OVCAR3-RFP</th>
</tr>
</thead>
<tbody>
<tr>
<td>LiPax</td>
<td>0.007449</td>
</tr>
<tr>
<td>Abraxane</td>
<td>0.033224</td>
</tr>
<tr>
<td>Doxorubicin HCl</td>
<td>0.190917</td>
</tr>
</tbody>
</table>
Overview

- Severe cancer with poor patient outcomes
- Current SOC is IV chemotherapy
  - Barrier
  - Toxicity
- Surgery time and technical expertise
- LiPax presents simpler installation via thoracotomy tube
- In vitro studies indicate that mesothelioma cells are very sensitive to LiPax
Manufacturing Process

Process Flow Chart

Simple homogenization
- Lipid hydration
- Addition of drug
- Dispersion

High pressure homogenization
- Drug incorporation into the lipid
- Size reduction

Lyophilization
- Remove and determine moisture content

Sieving
- Consistent particles

Filling, labelling and packaging
- The dried powder aliquots into containers such as vials, IV bags, etc.

Manufacturing Process Utilizes Common Industry-Standard Equipment