at a glance™

Recent Events: ***UroGen completed a successful IPO in May 2017, raising approximately $63 million net at $13 per share to fund three drug candidates for treating urological cancers, a vastly underserved market. Its lead program, MitoGel, is in an open label, single arm pivotal Phase 3 trial, with top-line date due 2Q18. An expected NDA filing 2H18 under the 505(b)(2) pathway could lead to a market launch in 2019. Its second late-stage program will enter Phase 2b trials 1H18. Look for a steady flow of clinical data during the next 12-18 months.***

Key Considerations

- **UroGen's board of directors is chaired by Arie Belldegrun, MD, a noted surgical urologist who is a founder and CEO of Nasdaq-traded KITE Pharma, recently acquired by Gilead for $11.8 billion.**
- **The company's operating management is led by CEO Ron Bentsur, who was previously CEO of Keryx, which he guided to a $2 billion-plus market cap specialty pharma company as it secured FDA marketing approval of Auryxia® through a successful 505 (b)(2) pathway filing.**
- **UroGen is developing potentially game-changing technology--a drug-based therapy designed to replace surgery as first-line treatments in at least three of the most difficult forms of urological cancers.**
- **Surgery is currently first-line in these indications because current drugs do not have long enough contact with the tumors to be effective – they get washed away too quickly by the flow and voiding of urine surrounding the tumors.**
- **UroGen's solution: the generic chemo agent mitomycin C formulated with a proprietary temperature-sensitive gel, a reverse thermal gel called RTGel.**
- **RTGel is liquid at cold temperatures, allowing instillation with a catheter to the tumor sites.**
- **But at body temperature the warmed liquid turns into a sticky gel, adhering to the tumor. The gel dissolves slowly, giving mitomycin C more 'dwell time' to penetrate the tumor -- up to eight hours, in fact, vs. 5-30 minutes in the standard saline water formulation.**
- **A Phase 3 pivotal trial of UroGen's lead candidate, MitoGel, began 2Q17 and was granted Fast Tract designation by the FDA in 3Q17. Phase 3 top-line data is expected 2Q18.**

UroGen Pharma Ltd.

(Nasdaq: URGN)

Recent Price:	**$31.73**
Approx. Shares O/S:	**13 Million**
Approx. Mkt Cap:	**$412 Million**
Institutions Own:	**37 Percent**

Analyst Coverage/Targets
Cowan (Out-Perform), Jefferies ($27), Oppenheimer ($32), Raymond James ($25)

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- **The company's second candidate, VesiGel, is for treating low-grade non-muscle invasive bladder cancer, or NMIBC. A U.S. Phase 2b is scheduled to start 1H18.**
- **UroGen's third current program is an immuno-oncology therapy in Phase 1 to treat high-grade muscle-invasive bladder cancer.**
- **Partnership opportunities may be plentiful. RTGel is a versatile platform technology and it could have wide utility in areas currently not core to UroGen.**
- **In 4Q16, UroGen licensed RTGel to Allergan to be used in combination with Botox for the treatment of overactive bladder. UroGen has received $25M from Allergan since signing and the deal could bring in an additional $200 million in milestones and royalties. Allergan filled an IND application in July 2017 to begin U.S. clinical trials.**

About UroGen

Nasdaq-listed UroGen is headquartered in Ra'anana, Israel and New York City and has clinical and financial operations in the U.S. Its focus is urological cancers, specifically cancers of the kidney, ureters, and bladder where there is an opportunity to replace standard of care surgeries with first-line drug-based solutions.

The company raised approximately $63 million in net proceeds in an over-subscribed IPO in May 2017. Jefferies LLC and Cowen and Company were the joint book-running managers and Raymond James & Associates and Oppenheimer & Co. Inc. were co-managers. The company believes current capital (pro forma about $74 million) is sufficient to fund operations through all current key milestones.

UroGen is in late-stage trials in two of the most difficult to treat forms of urological cancers -- low-grade cancer of the upper urothelial tract (UTUC) and non-muscle invasive bladder cancer (NMIBC).

Both cancers have been historically difficult to treat due to challenging anatomy and the inherent limitations of

Notable & Upcoming

1H17 – Completed NASDAQ IPO, raising $63M in net proceeds

1H17 – Started MitoGel Ph. 3 in low-grade UTUC

2H17 – Data updates Ph. 2 MitoGel in low-grade UTUC

2H17 – Data updates Ph. 2a VesiGel in NMIBC

4Q17 – Expected start Ph. 2 in overactive bladder (Allergan)

1H18 – Start Ph. 2b VesiGel in low-grade NMIBC

2Q18 – Topline Ph. 3 MitoGel in low-grade UTUC

2H18 – MitoGel NDA filing for marketing approval

2H18 – Start Ph. 2 Vesimmune in advanced bladder cancer

conventionally-formulated drugs. There are no approved drugs for UTUC and tumor resection procedures are particularly difficult to conduct in the upper urothelial tract, rendering nephrectomy, the removal of the kidney and the upper urothelial tract, the standard of care for the treatment of UTUC.

UroGen aims to change the standard of care by using its proprietary platform technology, RTGel, to empower cytotoxic agents to kill cancers cells instead of resecting them with delicate surgery and before vital organs like the bladder and kidneys are in need of removal.

About RTGel

Up to now, there has been no successful way to utilize drugs for first-line treatment of many urological cancers, particularly the three addressed in Urogen's current pipeline.

The rapid flow and voiding of urine through the urinary tract has made it nearly impossible to keep drugs 'in place' on the tumor long enough to penetrate the tumor effectively. Simply put, the drugs get washed away too quickly. A significant market opportunity awaits a solution.

Getting a Grip on Speed

Urine flows out of the body at an average rate of about one-half ounce per second. This means that most people (men and women alike) urinate at the rate of 30 ounces per minute. Of course, rates will differ between individuals based on, among other things, age and bladder fullness.

Enter RTGel, a proprietary inert polymer-based gel made of GRAS (Generally Accepted as Safe) ingredients that mixes well with drugs like UroGen's current choice, generic mitomycin C, which is currently used only as adjuvant therapy after surgery in several urological cancers due to its limited dwell time in its existing saline water formulation.

The RTgel-drug combination is liquid when cold. But once administered, body temperature turns the liquid back to its gooey, gelatinous state. The gel sticks to the tumor and dissolves slowly at a rate that increases mitomycin C's 'dwell time' on the tumor up to eight hours, vs. only 5-30 minutes without RTGel. This significantly increases mitomycin C's ability to penetrate the tumor.

In addition to increasing 'dwell time', the RTGel technology allows much higher dosing of mitomycin C than is possible in its naked form. For example, 8mgs of the drug can be formulated into 1ml of RTGel, several times higher than the amount of mitomycin C that can be dissolved in the same amount of water.

MitoGel

MitoGel, a RTGel formulation of mitomycin C, is being developed as first-line therapy in UTUC, a kidney-related cancer for which there currently is no approved drug. The standard of care is a roughly $80,000 surgical removal of the affected kidney, and, in many cases, this is followed by a life-long commitment to dialysis.

Fortunately, the disease has a relatively low incidence in the U.S. (roughly 7500 new cases annually), which qualified it for Orphan Drug Designation. This generally facilitates a faster review at the FDA. The company expects a favorable reception from payors based on the cost savings and improved quality of life of a drug-based treatment vs. surgery.

The currently enrolling Phase 3 OLYMPUS trial is expected to report topline data 2Q18, paving the way for an abbreviated New Drug Application filing 2H18 utilizing the 505(b)(2) pathway.

The open-label, single arm OLYMPUS pivotal study was authorized by the FDA based on a 100 percent Overall Response Rate and a 62% Complete Response (CR) rate of evaluable patients in a small compassionate use program with MitoGel.

The company believes that the likelihood of success in the roughly 70 patient Phase 3 OLYMPUS study is high. The company believes a 15-20 percent CR would be clinically meaningful and approvable. Analysts value the potential market at roughly $200+ million yearly with a potential market launch date in 2019.

VesiGel

VesiGel is a higher dose of mitomycin C formulated in RTGel.

VesiGel is under development as a first-line non-surgical alternative for low-grade non-muscle invasive bladder cancer (NMIBC). If the cancer can't be halted at this stage, it either spreads to other organs or begins to invade the bladder's muscle tissue, almost always requiring removal of the bladder.

There is no approved drug for NMIBC. The typical standard of care is a surgical procedure called TURBT which involves removal of tumors on the bladder. Over 100,000 such procedures are performed annually in the U.S. Forty to 60 percent of the patients undergo repeat surgeries after 12 months.

Benefitting from the same 'dwell-time' advantages as MitoGel, the hope for VesiGel is that it will avoid the need for tumor-removal surgeries, prevent metastasis, and spare the patient's bladder.

VesiGel recently completed a Phase 2a study. Results at the primary evaluation time showed a dose-dependent response with the highest dose of VesiGel achieving a compelling 86 percent CR rate. Fuller data on the Phase 2a will be reported shortly.

A Phase 2b of VesiGel in the U.S. is expected to start 1H18.

With approximately 80,000 new cases of NMIBC each year in the U.S., analysts say the addressable market for a product with VesiGel's expected profile should be about $700 million annually.

Vesimmune

The third UroGen candidate, Vesimmune, combines RTGel-formulated mitomycin C with a checkpoint inhibitor for high-grade muscle invasive bladder cancer. The new immuno-oncology therapy recently completed a Phase 1 study demonstrating initial single agent activity.

Summary Points

- **UroGen is the leader in developing drug-based methods to treat three of the most difficult forms of urological cancers for which surgery is now the standard of care.**
- **The company's lead program, MitoGel, is in an open label Phase 3 pivotal trial, with topline results due 2Q18, followed by an expected 2H18 abbreviated NDA filing for approval via the 505(b)(2) pathway. The candidate has Orphan Drug and Fast Track designations. Marketing could commence in 2019.**
- **A larger patient market (low-grade non-muscle invasive bladder cancer) is being addressed with VesiGel, currently scheduled to start a U.S. Phase 2b 1H18.**
- **Next in line: an immuno-oncology therapy, Vesimmune, which combines RTGel and mitomycin C with a checkpoint inhibitor for high-grade muscle-invasive bladder cancer.**
- **UroGen is well capitalized, having raised roughly $63 million in net proceeds earlier this year through an over-subscribed IPO.**
- **Proven management team and Board, headed up by Ron Bentsur and Arie Belldegrun, MD.**

For additional information, contact:

Redington, Inc. • CT 203 222-7399 • NY 212 926-1733 • www.redingtoninc.com
UroGen Pharma Ltd. • 212 213-0006 • www.urogen.com