UroGen Announces Positive Data from Pivotal OLYMPUS Trial Evaluating Jelmyto™ in Patients with Low-Grade Upper Tract Urothelial Cancer

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PRINCETON, N.J.--(BUSINESS WIRE)--May 15, 2020-- UroGen Pharma Ltd. (Nasdaq:URGN) today announced the presentation of positive data from the UGN-101 (*Jelmyto*™(mitomycin) for pyelocalyceal solution) Phase 3 OLYMPUS trial in patients with low-grade upper tract urothelial cancer (LG-UTUC). The study was accepted for the 2020 American Urological Association (AUA) Annual Meeting, published as a supplement to the April 2020 issue of *The Journal of Urology*® and presented as part of the AUA Virtual Experience. The presentation can be accessed via the AUA website here. More information can also be accessed via UroGen's virtual AUA experience here.

About the Phase 3 OLYMPUS Trial

OLYMPUS (Optimized DeLivery of Mitomycin for Primary UTUC Study) is an open-label, single-arm Phase 3 clinical trial of *Jelmyto*™(mitomycin) for pyelocalyceal solution to evaluate the safety, tolerability and tumor ablative effect of *Jelmyto* in patients with low-grade upper tract urothelial cancer. The trial enrolled 74 patients at clinical sites across the U.S. and Israel. Study participants were treated with six weekly instillations of *Jelmyto* administered via a standard ureteral catheter. Four to six weeks following the last instillation, patients underwent a Primary Disease Evaluation (PDE) to determine complete response (CR), the primary endpoint of the study. PDE involved a ureteroscopy and wash cytology, a standard microscopic test of cells obtained from the urine to detect cancer. Patients who achieved a CR at the PDE timepoint were then followed for up to 12 months to determine the durability of response with *Jelmyto*.

About *Jelmyto*™(mitomycin) for pyelocalyceal solution

Jelmyto™(mitomycin) for pyelocalyceal solution is a drug formulation of mitomycin for the treatment of low-grade upper tract urothelial cancer (LG-UTUC) in adult patients. Utilizing the RTGel™ technology platform, UroGen's proprietary sustained release, hydrogel-based formulation, *Jelmyto* is designed to enable longer exposure of urinary tract tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means. *Jelmyto* is delivered to patients using standard ureteral catheters or nephrostomy tube. The U.S. Food and Drug Administration granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to *Jelmyto* for the treatment of LG-UTUC. *Jelmyto* is the first drug approved for the treatment of LG-UTUC in adult patients.

IMPORTANT SAFETY INFORMATION

You should not receive JELMYTO if you have a hole or tear (perforation) of your bladder or upper urinary tract.

Before receiving JELMYTO, tell your healthcare provider about all your medical conditions, including if you:

- are pregnant or plan to become pregnant. JELMYTO can harm your unborn baby. You should not become pregnant during treatment with JELMYTO. Tell your healthcare provider right away if you become pregnant or think you may be pregnant during treatment with JELMYTO.
  Females who are able to become pregnant: You should use effective birth control (contraception) during treatment with JELMYTO and for 6 months after the last dose.
  Males being treated with JELMYTO: If you have a female partner who is able to become pregnant, you should use effective birth control (contraception) during treatment with JELMYTO and for 3 months after the last dose.
- are breastfeeding or plan to breastfeed. It is not known if JELMYTO passes into your breast milk. Do not breastfeed during treatment with JELMYTO and for 1 week after the last dose.
- **Tell your healthcare provider if you take water pills (diuretic).**

How will I receive JELMYTO?

- Your healthcare provider will tell you to take a medicine called sodium bicarbonate before each JELMYTO treatment.
- You will receive your JELMYTO dose from your healthcare provider 1 time a week for 6 weeks. It is important that you receive all 6 doses of JELMYTO according to your healthcare provider's instructions. If you miss any appointments, call your healthcare provider as soon as possible to reschedule your appointment. Your healthcare provider may recommend up to an additional 11 monthly doses.
- JELMYTO is given to your kidney through a tube called a catheter.
- During treatment with JELMYTO, your healthcare provider may tell you to take additional medicines or change how you take your current medicines.

After receiving JELMYTO:

- JELMYTO may cause your urine color to change to a violet to blue color. Avoid contact between your skin and urine for at least 6 hours.
- To urinate, **males and females should sit** on a toilet and flush the toilet several times after you use it. After going to the bathroom, wash your hands, your inner thighs, and genital area well with soap and water.
- Clothing that comes in contact with urine should be washed right away and washed separately from other clothing.

JELMYTO may cause serious side effects, including:

- **Swelling and narrowing of the tube that carries urine from the kidney to the bladder (ureteric obstruction).** If you develop swelling and narrowing, and to protect your kidney from damage, your healthcare provider may recommend the placement of a small plastic tube (stent) in the ureter to help the kidney drain. Tell your healthcare provider right away if you develop side pain or fever during treatment with JELMYTO.
- **Bone marrow problems.** JELMYTO can affect your bone marrow and can cause a decrease in your white blood cell, red blood cell, and platelet counts. Your healthcare provider will do blood tests prior to each treatment to check your blood cell counts during treatment with JELMYTO. Your healthcare provider may need to temporarily or permanently stop JELMYTO if you develop bone marrow problems during treatment with JELMYTO.

The most common side effects of JELMYTO include: side pain, urinary tract infection, blood in your urine, kidney problems, tiredness, nausea, stomach pain, trouble with urination, vomiting, low red blood cell count, frequent urination, itching, chills, and fever.

To report SUSPECTED ADVERSE REACTIONS, contact UroGen Pharma at 1-855-987-6436 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

Please see JELMYTO Full Prescribing Information, including the Patient Information at www.jelmyto.com.

About UroGen Pharma Ltd.

UroGen is a biopharmaceutical company dedicated to building novel solutions that treat specialty cancers and urologic diseases because patients deserve better options. UroGen has developed RTGel™ reverse-thermal hydrogel, a proprietary sustained release, hydrogel-based platform technology that has the potential to improve therapeutic profiles of existing drugs. UroGen's sustained release technology is designed to enable longer exposure of the urinary tract tissue to medications, making local therapy a potentially more effective treatment option. UroGen's approved *Jelmyto*™ (mitomycin) for pyelocalyceal solution, and pipeline treatment UGN-102 (mitomycin) for intravesical solution are designed to ablate tumors by non-surgical means and to treat several forms of non-muscle invasive urothelial cancer, including low-grade upper tract urothelial cancer and low-grade non-muscle invasive bladder cancer, respectively. UroGen is headquartered in Princeton, NJ with operations in Israel. Visit www.urogen.com to learn more or follow us on Twitter, @UroGenPharma.

Forward-Looking Statements

This press release contains forward-looking statements as that term is defined in the Private Securities Litigation Reform Act of 1995, including, without limitation: the potential of *Jelmyto*™to transform the treatment of LG-UTUC; the potential of UroGen's proprietary RTGel™ technology platform to improve therapeutic profiles of existing drugs; the potential of UGN-102 for LG NMIBC. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: the timing and success of clinical trials 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) therefore and the rate and degree of market acceptance thereof vis-à-vis alternative therapies; UroGen's ability to attract or retain key management, members of the board of directors and personnel; and any negative effects on UroGen's business and product development plans caused by or associated with COVID-19. 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 7, 2020, 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.

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