# UroGen Pharma Reports Fourth Quarter and Full-year 2021 Financial Results and Recent Corporate Developments

March 21, 2022

- *Jelmyto® net product revenue increased 42% over Q3 2021 with $16.2 million in Q4 2021; full-year net product revenue of $48.0 million in line with guidance*
- *Announced up to $100 million senior secured term loan facility with funds managed by Pharmakon Advisors, expected to support operations to reach cash flow breakeven by 2025*
- *First patient dosed in ENVISION, single-arm Phase 3 pivotal study for UGN-102 in bladder cancer*
- *Conference call and webcast to be held today at 10:00 AM ET*

PRINCETON, N.J.--(BUSINESS WIRE)--Mar. 21, 2022-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced financial results for the fourth quarter and full year ended December 31, 2021, and overview of recent developments.

“I am proud of the progress we made in 2021 on behalf of patients as we’ve expanded access to Jelmyto and set the stage for important commercial and clinical milestones in the year ahead,” said Liz Barrett, President and Chief Executive Officer of UroGen. “Our vision is to completely transform uro-oncology. With our enhanced capital position, solid adoption of Jelmyto in urothelial cancer, clinical progress in bladder cancer dosing patients in the single-arm pivotal Phase 3 ENVISION trial with UGN-102 and IND submission for UGN-301, our anti-CTLA4 antibody, we expect to deliver on that vision.”

**Business Highlights:**

**Jelmyto (mitomycin) for pyelocalyceal solution in low-grade Upper Tract Urothelial Cancer (LG-UTUC):**

- UroGen generated net product revenue of $16.2 million for the fourth quarter of 2021, representing 42% growth over the previous quarter and the highest quarter since launch.
- As of March 1, 2022, 832 sites have been activated, completing their internal processes, and having treated or ready to treat patients. This represents an 18% increase since November 1, 2021.
- Sites that have treated more than one patient as of March 1, 2022, increased to 106, compared to 86 as of November 1, 2021: an increase of approximately 23%.
- Expanded pilot Named Patient Supply program with the addition of Australia to the previously announced five European countries.
- Treated two patients in Israel, representing the first patients outside of the U.S. to receive Jelmyto in a commercial setting.

**UGN-102 (mitomycin) for intravesical solution:**

- UroGen announced trial initiation and dosing of the first patient of the single-arm pivotal Phase 3 ENVISION trial of UGN-102 for the treatment of low-grade, intermediate risk non-muscle invasive bladder cancer (NMIBC). The study will enroll approximately 220 patients across 90 sites and is expected to complete enrollment by the end of 2022.
- The trial is similar in design to the previously completed OPTIMA II study. Based on the results of the OPTIMA II study, the Company believes the ENVISION study carries a high probability of demonstrating a significant benefit for patients and, assuming positive findings, will be the basis of a New Drug Application (NDA) submission in 2024.

**UGN-301 (zalifrelimab) for intravesical solution:**

- Reported positive nonclinical toxicology data for UGN-301, which formed the foundation of an Investigational New Drug Application (IND) submitted to the U.S. Food and Drug Administration (FDA), advancing plans to initiate a first-in-human, multi-arm, Phase 1 clinical study in the first half of 2022.
- The Phase 1 study will evaluate the safety and tolerability of UGN-301 as monotherapy and in combination with other immunomodulators and chemotherapies in recurrent NMIBC. The first arm of the Phase 1 study will evaluate UGN-301 as monotherapy and will take approximately 12 months to complete.
- UGN-301 is the Company’s anti-CTLA4 antibody in development through a strategic collaboration with the University of Texas MD Anderson Cancer Center and is intended for use as monotherapy and in combination with other immunomodulators and chemotherapies to treat high-grade NMIBC.
- This clinical program represents the Company’s expansion into immunotherapy and intends to build upon encouraging nonclinical data showing that intravesical administration of anti-CTLA4 and a TLR agonist leveraging RTGel™ can produce a clinical benefit in a setting of high-grade bladder cancer.

**Up to $100 Million Senior Secured Term Loan Facility with Funds Managed by Pharmakon Advisors**

- Announced the closing of an up to $100 million senior secured term loan facility (term loan facility) with funds managed by

Pharmakon Advisors, L.P. (Pharmakon Advisors) expected to fund the Company's continued product development, commercialization efforts, and other operations allowing the company to achieve breakeven cash flow by 2025, based on its current financial projections.

- The first tranche of $75 million has been received with a second tranche of $25 million available to be drawn by the end of 2022.

**Fourth Quarter 2021 Financial Results:**

**Jelmyto Revenue:** UroGen reported net product revenue of Jelmyto for the fourth quarter 2021 of $16.2 million. Net product revenue was $48.0 million for the full year 2021, compared to $11.8 million in 2020 with the launch of Jelmyto in June 2020.

**R&D Expense:** Research and development expenses for the fourth quarter 2021 were $13.1 million, including non-cash share-based compensation expense of $0.9 million as compared to $12.4 million, including non-cash share-based compensation expense of $1.4 million, for the same period in 2020. Research and development expenses for the full year 2021 were $47.6 million, including non-cash share-based compensation expense of $4.0 million. This compares to $47.3 million, including non-cash share-based compensation expense of $6.4 million, for the full year 2020.

**SG&A Expense:** Selling, general and administrative expenses for the fourth quarter 2021 were $21.4 million, including non-cash share-based compensation expense of $4.5 million. This compares to $22.2 million, including non-cash share-based compensation expense of $5.1 million, for the same period in 2020. Selling, general and administrative expenses for the full year 2021 were $87.5 million, including non-cash share-based compensation expense of $19.1 million. This compares to $90.2 million, including non-cash share-based compensation expense of $21.6 million for the full year 2020.

**Financing on Prepaid Forward Obligation:** UroGen reported financing expense related to the prepaid forward obligation to RTW Investments of $7.3 million for the fourth quarter 2021. Financing expense related to the prepaid forward obligation to RTW Investments totaled $17.3 million for the full year 2021. The rate for 2022 will be 13% based on $48 million of global net product sales of Jelmyto in 2021.

**Net Loss:** UroGen reported a net loss of $28.5 million, or basic and diluted net loss per ordinary share of $1.27, for the fourth quarter 2021 as compared to $30.5 million, or basic and diluted net loss per ordinary share of $1.38, for the same period in 2020. UroGen reported a net loss of $110.8 million, or basic and diluted net loss per ordinary share of $4.96, for the full year 2021 versus $128.5 million, or basic and diluted net loss per ordinary share of $5.90, for the full year 2020.

**Cash & Cash Equivalents:** As of December 31, 2021, cash, cash equivalents and marketable securities totaled $89.8 million, and does not include the first tranche of the term loan facility.

**2022 Revenue, Operating Expense and RTW Expense Guidance:** The Company anticipates full year 2022 net product revenues from Jelmyto to be in the range of $70 to $80 million. The Company anticipates full year 2022 operating expenses in the range of $140 to $160 million, including non-cash share-based compensation expense of $10 to $16 million, subject to market conditions. The Company anticipates full year 2022 financing expense related to the prepaid obligation to RTW Investments in the range of $22 to $26 million, of which approximately $9.1 to $10.4 million will be in cash.

**Conference Call & Webcast Information:**

Members of UroGen's management team will host a live conference call and webcast today at 10:00 AM Eastern Time to review the Company's financial results and provide a general business update.

The live webcast can be accessed by visiting the Investors section of the Company's website at http://investors.urogen.com. Please connect at least 15 minutes prior to the live webcast to ensure adequate time for any software download that may be needed to access the webcast. Alternatively, please call (855) 765-5685 (U.S.) or (615) 247-5916 (International) to listen to the live conference call. The conference ID number for the live call will be 7897224. An archive of the webcast will be available for two weeks on the Company's website.

UROGEN PHARMA LTD.
SELECTED CONSOLIDATED BALANCE SHEETS
(U.S. dollars in thousands)

| | As of December 31, 2021 | As of December 31, 2020 |
|---|---|---|
| Cash and cash equivalents and marketable securities | $ 89,814 | $ 103,911 |
| Total assets | $ 119,746 | $ 122,005 |
| Total liabilities | $ 111,333 | $ 25,650 |
| Total shareholders' equity | $ 8,413 | $ 96,355 |

UROGEN PHARMA LTD.
CONDENSED CONSOLIDATED INCOME STATEMENT
(U.S. dollars in thousands, except share and per share data)

| | Three months ended December 31, | | Year ended December 31, | |
|---|---|---|---|---|
| | 2021 | 2020 | 2021 | 2020 |
| Revenues, net | $ 16,174 | $ 7,966 | $ 48,042 | $ 11,799 |
| Cost of revenues | 1,589 | 652 | 5,157 | 1,009 |
| Gross profit | 14,585 | 7,314 | 42,885 | 10,790 |
| Operating expenses: | | | | |
| Research and development expenses | 13,082 | 12,405 | 47,642 | 47,310 |
| Selling, general and administrative expenses | 21,418 | 22,163 | 87,535 | 90,219 |
| Total operating expenses | 34,500 | 34,568 | 135,177 | 137,529 |

| | | | | |
|---|---|---|---|---|
| Operating loss | (19,915) | (27,254) | (92,292) | (126,739) |
| Financing on prepaid forward obligation | (7,343) | - | (17,291) | - |
| Interest and other income, net | (57) | 102 | 212 | 1,629 |
| Loss before income taxes | (27,315) | (27,152) | (109,371) | (125,110) |
| Income tax expense | 1,137 | 3,374 | 1,449 | 3,374 |
| **Net loss** | $ (28,452) | $ (30,526) | $ (110,820) | $ (128,484) |
| Net loss per ordinary share basic and diluted | $ (1.27) | $ (1.38) | $ (4.96) | $ (5.90) |
| Weighted average shares outstanding, basic and diluted | 22,433,206 | 22,146,581 | 22,347,481 | 21,780,826 |

**About *Jelmyto*®**

*Jelmyto* (mitomycin) for pyelocalyceal solution, is a drug formulation of mitomycin indicated for the treatment of adult patients with low-grade upper tract urothelial cancer (LG-UTUC). 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. FDA previously granted Orphan Drug, Fast Track, and Breakthrough Therapy Designations to Jelmyto for the treatment of LG-UTUC. On April 15, 2020, the FDA approved Jelmyto, making it the first drug approved for the treatment of LG-UTUC in adult patients.

**APPROVED USE FOR JELMYTO**

JELMYTO® is a prescription medicine used to treat adults with a type of cancer of the lining of the upper urinary tract including the kidney called low-grade Upper Tract Urothelial Cancer (LG-UTUC).

**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:** urinary tract infection, blood in your urine, side pain, nausea, trouble with urination, kidney problems, vomiting, tiredness, stomach (abdomen) pain.

You are encouraged to report negative side effects of prescription drugs to the FDA. Visit www.fda.gov/medwatch or call 1-800-FDA-1088. You may also report side effects to UroGen Pharma at 1-855-987-6436.

**Please see JELMYTO Full Prescribing Information, including the Patient Information, for additional information.**

**About Upper Tract Urothelial Cancer (UTUC)**

Urothelial cancer is the ninth most common cancer globally and the eighth most lethal neoplasm in men in the U.S. Between five percent and ten percent of primary urothelial cancers originate in the ureter or renal pelvis and are collectively referred to as upper tract urothelial cancers (UTUC). In the U.S., there are approximately 6,000 - 7,000 new or recurrent low-grade UTUC patients annually. Most cases are diagnosed in patients over 70 years old, and these older patients often face comorbidities. There are limited treatment options for UTUC, with the most common being endoscopic surgery or nephroureterectomy (removal of the entire kidney and ureter). These treatments can lead to a high rate of recurrence and relapse.

**About UGN-102**

UGN-102 (mitomycin) for intravesical solution is an investigational drug formulation of mitomycin in Phase 3 development for the treatment of low-grade intermediate risk NMIBC. Utilizing the RTGel™ Technology Platform, UroGen's proprietary sustained release, hydrogel-based formulation, UGN-102 is designed to enable longer exposure of bladder tissue to mitomycin, thereby enabling the treatment of tumors by non-surgical means. UGN-102 is delivered to patients using a standard urinary catheter. The Company presented results from the Phase 2b OPTIMA II trial in September 2021.

**About the Phase 3 ENVISION Trial**

The Phase 3 ENVISION trial is a single-arm, multinational, multicenter study evaluating the efficacy and safety of UGN-102 (mitomycin) as primary chemoablative therapy in patients with low-grade, intermediate-risk NMIBC. The Phase 3 ENVISION trial is expected to enroll approximately 220 patients across 90 sites and study participants will receive six once-weekly intravesical instillations of UGN-102. The planned primary endpoint will evaluate the complete response rate at three months after the first installation, and the key secondary endpoint will evaluate durability over time in patients who achieve complete response at the three-month assessment. Based on discussions with the FDA, and enrollment expected by end of 2022, assuming positive findings, UroGen anticipates submitting an NDA for UGN-102 in 2024.

Learn more about the Phase 3 ENVISION trial at www.clinicaltrials.gov (NCT05243550)

**About the Phase 3 ATLAS Trial**

The Phase 3 ATLAS trial was a global, open-label, randomized controlled study designed to assess the efficacy and safety of UGN-102, with or without transurethral resection of bladder tumor (TURBT), versus TURBT alone in patients diagnosed with low-grade intermediate risk NMIBC, defined as 1 or 2 of the following: new or recurrent multifocal bladder tumors, a solitary new or recurrent tumor >3 cm, or low-grade intermediate risk NMIBC recurrence in less than 12 months following a prior tumor diagnosis requiring endoscopic surgical resection or ablation.

Patients were randomized 1:1 to either UGN-102 or TURBT. Patients in the UGN-102 arm were treated with six weekly intravesical instillations of UGN-102. At the three-month time point, patients were assessed for response. Patients who have demonstrated a complete response to either UGN-102 or TURBT, will continue for long-term follow-up for evidence of recurrence. Patients who demonstrate presence of persistent disease at three-months, in either arm, will undergo a TURBT and then will also continue for long-term follow up for evidence of recurrence. The primary endpoint of the study was disease free survival. On November 10, 2021, the Company announced that, following discussions with the FDA, it has ceased enrollment in the Phase 3 ATLAS trial and plans to initiate a new, single-arm Phase 3 study of UGN-102 in early 2022. All patients enrolled in the treatment arm of the Phase 3 ATLAS trial will continue to receive treatment and undergo follow up.

Learn more about the Phase 3 ATLAS trial at www.clinicaltrials.gov (NCT04688931)

**About UroGen Pharma Ltd.**

UroGen is a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers 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. *Jelmyto*® (mitomycin) for pyelocalyceal solution and investigational treatment UGN-102 (mitomycin) for intravesical solution are designed to ablate tumors by non-surgical means. 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, statements regarding: the design, objectives and timing of the Phase 3 ENVISION trial; the expected benefits that may be demonstrated by the Phase 3 ENVISION trial; plans with respect to the treatment and follow up of patients previously enrolled in the Phase 3 ATLAS trial; plans with respect to a regulatory submission for UGN-102; further adoption of Jelmyto; the planned Phase 1 clinical study for UGN-301 and the design, objectives and timing thereof; the availability of the second tranche term loan under the term loan facility; our expectations regarding our ability to achieve cash flow breakeven by 2025; our ability to deliver on our vision to completely transform uro-oncology; and financial guidance for 2022. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: clinical trial enrollment challenges that may impact the expected timing of our planned clinical trials, including challenges related to the ongoing COVID-19 pandemic and the Russia-Ukraine conflict; 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 commercialization activities, including complications resulting from the ongoing COVID-19 pandemic; 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; 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, commercialization and product development plans caused by or associated with COVID-19 or geopolitical events. 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-K being filed with the SEC on March 21 2022, 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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