UroGen Announces FDA Acceptance of its New Drug Application for UGN-102

October 15, 2024

- PDUFA goal date set for June 13, 2025
- UGN-102 would be the first FDA-approved medicine for LG-IR-NMIBC, if approved

PRINCETON, N.J.--(BUSINESS WIRE)--Oct. 15, 2024-- UroGen Pharma Ltd. (Nasdaq: URGN), a biotech company dedicated to developing and commercializing innovative solutions that treat urothelial and specialty cancers, today announced U.S. Food and Drug Administration (FDA) acceptance of the New Drug Application (NDA) for investigational drug UGN-102 (mitomycin) for intravesical solution**.** UGN-102 could become the first FDA-approved medicine for the treatment of low-grade intermediate-risk non-muscle invasive bladder cancer (LG-IR-NMIBC). The FDA has assigned a Prescription Drug User Fee Act (PDUFA) goal date of June 13, 2025.

"The FDA acceptance of our NDA is a pivotal moment in our journey to bring UGN-102 to patients," said Liz Barrett, President and Chief Executive Officer of UroGen. "UGN-102 could be the first FDA-approved medicine for LG-IR-NMIBC, offering a novel approach that could expand treatment options and address unmet needs. There is an urgent need for innovative solutions in this space, and we are dedicated to collaborating with the FDA as we prepare for a potential launch of UGN-102 in 2025."

Dr. Mark Schoenberg, Chief Medical Officer of UroGen, stated, "The NDA for UGN-102 is backed by a robust data set demonstrating impressive durability of response across three clinical trials and a favorable safety profile. Notably, the ENVISION trial successfully met its primary endpoint, showing a 79.6% complete response rate at three months after the first instillation of UGN-102. Additionally, the latest results from that trial revealed an 82.3% 12-month duration of response by Kaplan-Meier estimate in patients who achieved a complete response at 3 months. The most common treatment-emergent adverse events in the ENVISION trial were dysuria, hematuria, urinary tract infection, pollakiuria, fatigue, and urinary retention. Additionally, the safety profile observed in the ENVISION trial was consistent with that seen in other studies of UGN-102. We believe that, if approved, UGN-102's ability to achieve durable complete responses and potentially reduce recurrence rates while extending treatment-free intervals will represent a significant advance in managing LG-IR-NMIBC."

About UGN-102

UGN-102 (mitomycin) for intravesical solution is an innovative drug formulation of mitomycin, currently under regulatory review for approval in the treatment of LG-IR-NMIBC. Utilizing UroGen's proprietary *RTGel*® technology, a 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 in an outpatient setting by a trained healthcare professional. An NDA for UGN-102 is currently under review by the FDA with a potential decision expected by June 13, 2025. The U.S. market for LG-IR-NMIBC that UGN-102 can address, if approved, is valued at approximately $5 billion.

About ENVISION

The Phase 3 ENVISION trial is a single-arm, multinational, multicenter study evaluating the efficacy and safety of UGN-102 (mitomycin) for intravesical solution as primary chemoablative therapy in patients with LG-IR-NMIBC. The Phase 3 ENVISION trial completed target enrollment with approximately 240 patients across 56 sites. Study participants received six once-weekly intravesical instillations of UGN-102. The primary endpoint evaluated the CR rate at the three-month assessment after the first instillation, and the key secondary endpoint evaluated durability over time in patients who achieved a CR at the three-month assessment. Learn more about the Phase 3 ENVISION trial at www.clinicaltrials.gov (NCT05243550).

About Non-Muscle Invasive Bladder Cancer (NMIBC)

In the U.S. bladder cancer is the second most common urologic cancer in men. LG-IR-NMIBC represents approximately 22,000 newly diagnosed bladder cancer patients each year and an estimated 60,000 recurrences annually among patients diagnosed from previous years. Bladder cancer primarily affects older populations with the median age of diagnosis 73 years and an increased risk of comorbidities. Guideline recommendations for the management of NMIBC include TURBT as the standard of care. Up to 70 percent of NMIBC patients experience at least one recurrence and LG-IR-NMIBC patients are even more likely to recur and face repeated TURBT procedures.

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 the 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. Our first product to treat low-grade upper tract urothelial cancer and investigational treatment UGN-102 (mitomycin) for intravesical solution for patients with LG-IR-NMIBC 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 X (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 potential for UGN-102 to be the first FDA-approved medicine for LG-IR-NMIBC; the potential patient benefits and opportunities for UGN-102, if approved; the potential timing for a decision from the FDA on the UGN-102 NDA; the estimated size of the U.S. market that could be addressed by UGN-102, if approved; the potential launch timing for UGN-102, if approved; the potential for UGN-102 to achieve durable complete responses and reduce recurrence rates while extending treatment-free intervals; the estimated annual U.S. patient population and demographics for LG-IR-NMIBC; the potential of UroGen's proprietary RTGel technology to improve therapeutic profiles of existing drugs; and the potential of UroGen's sustained release technology to make local delivery more effective as compared to other treatment options.

Words such as “anticipate,” “can,” “expect,” “if,” “potential,” “will,” or other words that convey uncertainty of future events or outcomes to identify these forward-looking statements. These statements are subject to a number of risks, uncertainties and assumptions, including, but not limited to: even though the NDA for UGN-102 has been accepted by the FDA, there is no guarantee that such NDA will be sufficient to support approval of UGN-102 on the timeframe expected, or at all; the PDUFA goal date may be delayed due to various factors outside UroGen’s control; past results are not necessarily indicative of results that may be seen in the future, including in larger patient populations; the ability to obtain and maintain regulatory approval; complications associated with commercialization activities; the labeling for any approved product; competition in UroGen’s industry; 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 other personnel; UroGen’s RTGel technology may not perform as expected; and UroGen may not successfully develop and receive regulatory approval of any other product that incorporates RTGel technology. In light of these risks and uncertainties, and other risks and uncertainties that are described in the Risk Factors section of UroGen’s Quarterly Report on Form 10-Q quarter ended June 30, 2024, filed with the SEC on August 13, 2024 (which is available at 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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