



Positive Data from Phase 1 Trial of Bel-sar in Patients with Non-Muscle-Invasive Bladder Cancer (NMIBC) Presented at the 40th Annual European Association of Urology Congress

March 24, 2025

Clinical Complete Responses Observed with Single Low-Dose of Bel-sar in Patients with Intermediate and High-Risk NMIBC with Robust Cell-Mediated Immunity and Urothelial Field Effect

Favorable Safety Profile; Only Grade 1 Drug-Related Adverse Events Reported in Less Than 10% of Patients

Data Supports Potential for a Paradigm Shifting Approach in the Front-Line Treatment of patients with NMIBC

Aura Hosting [Virtual Urologic Oncology Investor Event](#) with Key Opinion Leaders Today at 4:30 pm Eastern Time

BOSTON, March 24, 2025 (GLOBE NEWSWIRE) -- [Aura Biosciences](#), Inc. (NASDAQ: AURA), today announced positive data from a completed Phase 1 window of opportunity trial of bel-sar (AU-011) in patients with NMIBC. These data were presented by Dr. Seth Lerner as a late breaker presentation at the 40th Annual European Association of Urology (EAU) Congress, held in Madrid, Spain.

The trial included 15 patients with NMIBC, with the primary endpoints of evaluating the safety and feasibility of local administration of bel-sar alone (n=5) and bel-sar with light activation (n=10). The trial also evaluated tumor necrosis and shrinkage as an indication of biological activity as well as immune mediated changes in the tumor microenvironment (TME). All patients that received bel-sar without light activation (n=5) had intermediate-risk NMIBC, and no patients had a clinical complete response or tumor shrinkage after treatment. In NMIBC patients that received bel-sar with light activation (n=10), 5 patients had intermediate-risk disease and 5 patients had high-risk disease. In the group of patients with intermediate-risk NMIBC (n=5), 4 out of 5 patients demonstrated a clinical complete response of the target lesion with no tumor cells detected in histopathological evaluation and necrosis was observed in 3 out of 5 patients. In the group of patients with high-risk disease (n=5), 1 out of 5 patients demonstrated a clinical complete response and 3 out of 5 patients demonstrated visual tumor shrinkage on cystoscopy. Furthermore, in the light activated cohorts, 4 out of 7 patients (57%) with multiple tumors demonstrated a clinical complete response in at least one non-target tumor with infiltration of effector CD8+ and CD4+ T-cells. Initial multiplex immunofluorescence staining of biopsies after bel-sar treatment in 3 patients revealed significant infiltration of cytotoxic effector cells, as well as de novo formation of tertiary lymphoid structures in both target and non-target lesions. These results support a robust anti-tumor immune response consistent with the observed clinical complete response and urothelial field effect.

"The clinical complete responses and the immune responses seen with a single low-dose of bel-sar in such a short time frame are highly encouraging. This reinforces our belief that this novel mechanism of action could be the key to generating long term durable responses," said Sabine Brookman-May, MD, FEBU, Senior Vice President, Therapeutic Area Head Urologic Oncology of Aura Biosciences. "We look forward to our virtual urologic oncology investor event, where we will outline the Phase 1b/2 trial to further evaluate bel-sar's clinical activity and future development plans."

"The positive data presented at EAU are compelling and suggest that bel-sar has the potential to introduce a new front-line focal treatment approach instead of or ahead of TURBT in patients across different risk categories," said Seth Lerner, MD, Scott Department of Urology, Dan L. Duncan Comprehensive Cancer Center, Baylor College of Medicine. "With its favorable safety profile and the ability to be administered without general anesthesia, this novel modality could expand treatment options for patients and may represent a shift in how we approach NMIBC management."

Trial Design: The completed Phase 1 window of opportunity trial ([NCT05483868](#)) is a two-part, open-label clinical trial, designed to assess the safety and feasibility of bel-sar in NMIBC. The study treatment was administered 7 to 12 days before the scheduled transurethral resection of bladder tumor (TURBT), the standard of care procedure. The participants were followed for safety monitoring over a 56-day period. The trial evaluated bel-sar's biological activity with histopathological evaluation of tissue samples collected at the time of prior scheduled TURBT (regardless of tumor response) with evaluation of focal necrosis and immune changes in the TME as secondary endpoints, as well as visual assessment of tumor shrinkage. The Phase 1 window of opportunity trial is now complete, with Part 1 (n=5) patients receiving a single bel-sar dose without light activation and Part 2 (n=10) patients with a confirmed tumor at time of treatment, receiving either 100ug or 200ug of bel-sar as a single dose. Of these 10 patients, 5 had intermediate-risk disease and 5 had high-risk disease at the time of treatment. Eight of these 10 patients had a history of recurrent bladder cancer and had undergone multiple TURBTs and adjuvant treatments such as Bacillus Calmette-Guerin (BCG), mitomycin, gemcitabine, cetrelimab, and tamoxifen prior to trial enrollment.

Safety Data: Bel-sar was well-tolerated, with drug-related Grade 1 events reported in less than 10% of patients. No Grade 2 or higher drug-related adverse events and no serious adverse events were reported. No significant differences between the light-activated and non-light activated cohorts were observed.

Biological Activity: The data in the 10 patients that received bel-sar with light activation showed clinical activity detectable as soon as 7 days after a single low dose of bel-sar with light activation. This was demonstrated by histopathological evidence of clinical complete response, necrosis, immune activation or visual tumor shrinkage observed on cystoscopy. In contrast, no clinical activity was seen in the 5 patients receiving bel-sar with no light activation. For this analysis, "clinical complete response" was defined as the absence of tumor cells on histopathologic evaluation. Four out of 5 patients with intermediate-risk disease exhibited a clinical complete response of the target lesion with no tumor cells detected in histopathological evaluation post-treatment. In addition, visual tumor shrinkage was observed in several non-target tumors on cystoscopy. One of the patients with high-risk disease (based on BCG failure) had a clinical complete response, and 3 out of 5 patients with high-risk disease demonstrated visual tumor shrinkage on cystoscopy. Immune activation was noted in all patients (n=10) in both target and non-target bladder tumors. Four out of 7 patients with multiple tumors (57%) demonstrated a clinical complete response in at least one non-target tumor with infiltration of effector CD8+ and CD4+ T-cells. This data provides evidence of a bladder urothelial field effect with a single low-dose of bel-sar with light activation, potentially indicating a broader immune response and immune surveillance in the bladder beyond the target tumor in these patients.

Immune Profiling: To evaluate the local immune response after the treatment with bel-sar in the TME, multiplex immunofluorescence staining for key

immune cell types was performed on tumor biopsies. Initial post-treatment results from 3 patients showed significant infiltration per unit area of cytotoxic effector cells demonstrating early activation of both innate and adaptive immunity (Natural Killer cells, CD4+ and CD8+ T cells). In addition, de novo formation of mature tertiary lymphoid structures (TLS) post-treatment was observed in 2 of these 3 participants. Early TLS were also observed in distant, non-target lesions, suggestive of an immune mediated urothelial field effect. These early observations showing induction of effector immunity and the development of local active immunosurveillance, highlight key features of bel-sar's dual mechanism of action and the potential to translate into durable treatment responses.

Virtual Urologic Oncology Investor Event

Aura will host a virtual urologic oncology investor event today, at 4:30 pm Eastern Time, featuring Neal Shore, MD, FACS (Carolina Urologic Research Center), Gary Steinberg, MD, FACS (Rush University) and Jennifer A. Linehan, MD (Saint John's Cancer Institute), who will join company management to discuss the data from the Phase 1 trial.

At the event, Aura will also provide an update on the bladder cancer development program, including the Phase 1b/2 trial and future development plans. A live question and answer session will follow the discussion. To register for the event, [click here](#).

The live webcast of Aura's virtual urologic oncology investor event will be available on the "Investors & Media" page under the "Events & Presentations" section of Aura's website at <https://ir.aurabiosciences.com/events-and-presentations>, where a replay of the webcast will be archived for 90 days following the presentation date.

About Aura Biosciences

Aura Biosciences is a clinical-stage biotechnology company focused on developing precision therapies for solid tumors that aim to preserve organ function. Our lead candidate, bel-sar (AU-011), is currently in late-stage development for primary choroidal melanoma and in early-stage development in other ocular oncology indications and bladder cancer. Aura Biosciences is headquartered in Boston, MA. Our mission is to grow as an innovative global oncology company that positively transforms the lives of patients.

For more information, visit aurabiosciences.com. Follow us on X (formerly Twitter) @AuraBiosciences and visit us on LinkedIn.

Forward-Looking Statements

This press release contains forward-looking statements within the meaning of the Private Securities Litigation Reform Act of 1995, as amended, and other federal securities laws. Any statements that are not statements of historical fact may be deemed to be forward-looking statements. Words such as "may," "will," "could," "should," "expects," "intends," "plans," "anticipates," "believes," "estimates," "predicts," "projects," "seeks," "endeavor," "potential," "continue" or the negative of such words or other similar expressions can be used to identify forward-looking statements. These forward-looking statements include express or implied statements regarding Aura's future expectations, plans and prospects, including, without limitation, statements regarding the therapeutic potential of bel-sar for the treatment of cancers including bladder cancer; statements regarding Aura's plans and expectations for its ongoing and future clinical trials of bel-sar in bladder cancer; statements regarding Aura's beliefs and expectations for bel-sar's ability to provide durable responses in bladder cancer patients; statements regarding the timing and content of Aura's virtual urologic oncology investor event; statements regarding Aura's expectations for an improved quality of life of patients after treatment with bel-sar and changes to the treatment paradigm for patients; and statements regarding the timing of the announcement of bladder cancer program updates, including the Phase 1b/2 trial and future development plans.

The forward-looking statements in this press release are neither promises nor guarantees, and investors should not place undue reliance on these forward-looking statements because they involve known and unknown risks, uncertainties and other factors, many of which are beyond Aura's control and which could cause actual results to differ materially from those expressed or implied by these forward-looking statements, including, without limitation, uncertainties inherent in clinical trials and in the availability and timing of data from ongoing clinical trials; the expected timing for submissions for regulatory approval or review by governmental authorities; the risk that the results of Aura's preclinical and clinical trials may not be predictive of future results in connection with future clinical trials; the risk that early or interim data from ongoing clinical trials may not be predictive of final data from completed clinical trials; the risk that governmental authorities may disagree with Aura's clinical trial designs, even where Aura has obtained agreement with governmental authorities on the design of such trials, such as the Phase 3 special protocol assessment agreement with the U.S. Food and Drug Administration; whether Aura will receive regulatory approvals to conduct trials or to market products; whether Aura's cash resources will be sufficient to fund its foreseeable and unforeseeable operating expenses and capital expenditure requirements; Aura's ongoing and planned preclinical activities; and Aura's ability to initiate, enroll, conduct or complete ongoing and planned clinical trials. These risks, uncertainties and other factors include those risks and uncertainties described under the heading "Risk Factors" in Aura's most recent Annual Report on Form 10-K and Quarterly Report on Form 10-Q filed with the United States Securities and Exchange Commission (SEC) and in subsequent filings made by Aura with the SEC, which are available on the SEC's website at www.sec.gov. Except as required by law, Aura disclaims any intention or responsibility for updating or revising any forward-looking statements contained in this press release in the event of new information, future developments or otherwise. These forward-looking statements are based on Aura's current expectations and speak only as of the date hereof and no representations or warranties (express or implied) are made about the accuracy of any such forward-looking statements.

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