Aura Biosciences Presents Final Phase 1b/2 Data for its first Virus-Like Drug Conjugate, AU-011, in Patients with Choroidal Melanoma at the American Academy of Ophthalmology 2021 Annual Meeting

November 15, 2021

Presentations Include Final Safety and Efficacy Data from the Phase 1b/2 Trial using Intravitreal Administration and Updated Safety Data from the Phase 2 Trial using Suprachoroidal Administration

CAMBRIDGE, Mass.--(BUSINESS WIRE)--Nov. 15, 2021-- Aura Biosciences, a clinical-stage oncology company developing a novel class of virus-like drug conjugate (VDC) therapies for multiple oncology indications, today announced the presentation of data evaluating its first VDC, AU-011, in indeterminate lesions (ILs) and choroidal melanoma (CM), including final safety and efficacy data from the Phase 1b/2 trial using intravitreal (IVT) administration, as well as updated safety results from the Phase 2 trial using suprachoroidal (SC) administration. The results are presented as part of the American Academy of Ophthalmology (AAO) 2021 Annual Meeting.

“The final safety and efficacy data from the Phase 1b/2 trial using intravitreal administration presented today, along with the data from the Phase 2 trial using suprachoroidal administration, provide a high level of confidence for further clinical development in patients with indeterminate lesions or choroidal melanoma,” said Carol Shields, MD, Chief of the Ocular Oncology Service at Wills Eye Hospital and Professor of Ophthalmology at Thomas Jefferson University. “I believe AU-011 may offer patients a safe, effective first-line therapy for early-stage disease that preserves vision, a critical component in patients’ quality of life often neglected with today’s current treatment options.”

Final 12-Month Safety and Efficacy Data from Phase 1b/2 Trial with IVT administration

The Phase 1b/2 trial (NCT03052127) evaluated the safety and efficacy of AU-011 using IVT administration for the treatment of ILs and CM. A total of 56 patients were enrolled in the Ph1b/2 trial including the single and multiple dose escalation cohorts and received up to two cycles of therapy (therapeutic regimen). As part of an enrichment strategy agreed with FDA, patients with small tumors with active growth were enrolled in the Phase 2 part of the study (expansion cohort). This group of patients (n=14) received the therapeutic regimen and were evaluated for the tumor growth rate, tumor control, and visual acuity preservation as the efficacy endpoints. These endpoints have been agreed with FDA and are planned to be used in the pivotal program. The results at 12 months showed a statistically significant reduction in the tumor growth rate (-0.483 mm/yr, p = 0.018) compared to each patient’s documented growth rate at study entry, and a 64% (9/14) tumor control rate. In addition, the visual acuity preservation rate was 71%, which is unprecedented compared to the current standard of care with radiotherapy. Overall, AU-011 demonstrated a favorable safety and tolerability profile. The majority of adverse events (AEs), which included intraocular inflammation and increased intraocular pressure, were transient and resolved without clinical sequelae. A large number of patients (43/56) had tumors close to the fovea and optic disk and only two patients with juxta-foveal tumors had a treatment related serious adverse event (SAE) of vision loss. No other treatment related SAEs were observed in the trial. These safety and efficacy results indicate that AU-011 may offer a targeted vision preserving therapy for the first line treatment of CM.

Safety Data from Phase 2 Trial with SC Administration

This Phase 2 trial (NCT04417530) includes an open-label, dose escalation phase assessing the safety and efficacy of AU-011 via SC administration in patients with ILs and CM and plans to enroll up to 22 patients. In this preliminary safety data review of the initial dose escalation cohorts (n=14), no treatment related SAEs, dose limiting toxicities (DLTs), or grade 3/4 AEs were reported. Preliminary results indicate a positive safety and tolerability profile for AU-011 via SC administration.

Details for the AAO 2021 Presentations are as follows:

Title: A Phase 1b/2 Trial of AU-011, a First in Class Targeted Therapy for the Treatment of Choroidal Melanoma via Intravitreal Administration
Presenter: Carol L. Shields, Wills Eye Hospital
Session: OP10 Ocular Pathology and Oculoplastics Original Paper Session
Date and time: Monday, November 15 from 9:45 – 9:52 AM CT
Location: 255-257

Title: A Phase 2 Trial of a First in Class Targeted Therapy for Choroidal Melanoma via Suprachoroidal (SC) Administration
Presenter: Hakan Demirci, Kellogg Eye Center
Session: PD08 Ocular Pathology and Oculoplastics Poster Discussion
Date and time: Available on demand beginning Friday, November 12, 2021, at 7:30am PT
Location: Virtually on demand

The presentations can be accessed by visiting the “Scientific Presentations” section of “VDC Platform” page of the Aura Biosciences website.

About Choroidal Melanoma

Choroidal melanoma is a rare and aggressive type of eye cancer. Choroidal melanoma is the most common primary intraocular tumor in adults and develops in the uveal tract of the eye. No targeted therapies are available at present, and current radiotherapy treatments can be associated with severe visual loss and other long-term sequelae such as dry eye, glaucoma, cataracts, and radiation retinopathy. The most common current treatment is plaque radiotherapy, which involves surgical placement of a radiation device on the exterior of the eye over the tumor. The alternative is enucleation, or total surgical removal of the eye. Choroidal melanoma metastasizes in approximately 50 percent of cases with liver involvement in 80-90% of cases and, unfortunately, metastatic disease is universally fatal. There is a very high unmet need for a new vision sparing targeted therapy that could enable early treatment intervention for this life-threatening rare disease given the mortality rate in metastatic disease, lack of approved therapies, and the comorbidities of radioactive treatment options.

About AU-011

AU-011 is a first-in-class virus-like drug conjugate (VDC) therapy in clinical development for the first line treatment of choroidal melanoma. The virus-like component of the VDC selectively binds unique heparin sulphate proteoglycans (HSPGs), which are modified and overexpressed on the tumor cell surface of malignant cells in the choroid and AU-011 delivers a potent cytotoxic drug that is activated with infrared light. Upon activation with an ophthalmic laser, the cytotoxic drug rapidly and specifically disrupts the cell membrane of malignant cells with a pro-immunogenic cell death that can activate the immune system generating long term anti-tumor immunity. The unique specificity of tumor binding by the VDC enables the preservation of key eye structures, which may allow for the potential of preserving patients' vision and reducing other long-term complications of radiation treatment. AU-011 can be delivered using equipment commonly found in an ophthalmologist's office and does not require a surgical procedure, pointing to a potentially less invasive, more convenient therapy for patients and physicians. AU-011 for the treatment of choroidal melanoma is currently in Phase 2 clinical development and the company plans to expand the clinical program into choroidal metastasis.

About Suprachoroidal Administration

The suprachoroidal space (SCS®) injection treatment approach offers unprecedented access to the back of the eye where sight-threatening disease often occurs. Aura believes that delivering AU-011 into SCS within the eye, has the potential to offer certain advantages, including higher bioavailability at the tumor site and reduced exposure of non-targeted tissues, which may lead to an improved therapeutic index for AU-011. Collectively, these features could allow for the treatment of a wider range of tumor sizes, and, therefore, a larger number of patients may be treatable. The Company is partnered with Clearside Biomedical for use of Clearside's SCS Microinjector® for administration of AU-011 into the SCS.

About Aura Biosciences

Aura Biosciences, Inc. is a clinical-stage oncology company developing a novel technology platform based on virus-like drug conjugates (VDCs) to target and destroy cancer cells selectively while activating the immune system to create long lasting anti-tumor immunity. The VDC technology platform is based on the discoveries of NIH Distinguished Investigator Dr. John Schiller of the Center for Cancer Research at the National Cancer Institute. The company has the goal of developing this technology in multiple cancer indications with an initial focus on primary choroidal melanoma, a rare disease for which there are no approved drugs. Aura's lead product candidate belzupacap sarotalocan (AU-011) is currently in Phase 2 development for the first line treatment of primary choroidal melanoma, a vision and life-threatening form of eye cancer where standard of care radioactive treatments leave patients with major vision loss and severe comorbidities. AU-011 was well tolerated in a Phase 1b/2 trial, demonstrating high rates of tumor control and vision preservation. Future pipeline applications for Aura's technology include additional ocular oncology indications like choroidal metastases and solid tumor indications like non-muscle invasive bladder cancer. Aura is headquartered in Cambridge, MA.

Forward Looking Statement

This press release may contain forward-looking statements and information within the meaning of The Private Securities Litigation Reform Act of 1995 and other federal securities laws, including express or implied statements regarding Aura's future expectations, plans and prospects, including, without limitation, statements regarding expectations and plans for presenting clinical data, projections regarding our long-term growth, the anticipated timing of our clinical trials and regulatory filings, the development of our product candidates and advancement of our clinical programs, as well as other statements containing 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 that can be used to identify forward-looking statements. The express or implied forward-looking statements included in this press release are only predictions and are subject to a number of risks, uncertainties and assumptions, including, without limitation: uncertainties inherent in clinical trials and in the availability and timing of data from ongoing clinical trials; whether interim results from a clinical trial, including the Phase 2 SC administration trial, will be predictive of the final results of the trial; whether results from pre-clinical studies or earlier clinical studies will be predictive of the results of future trials, including regarding AU-011's ability to offer vision preserving therapy for the first line treatment of choroidal melanoma; the expected timing of the expansion phase of the Phase 2 SC administration trial; the expected timing for submissions for regulatory approval or review by governmental authorities; 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; risks, assumptions and uncertainties regarding the impact of the continuing COVID-19 pandemic on Aura's business, operations, strategy, goals and anticipated timelines, Aura's ongoing and planned pre-clinical activities, Aura's ability to initiate, enroll, conduct or complete ongoing and planned clinical trials, Aura's timelines for regulatory submissions and Aura's financial position. These and other risks and uncertainties are described more fully in the section titled "Risk Factors" set forth in Aura's filings with the Securities and Exchange Commission. In light of these risks, uncertainties and assumptions, the forward-looking events and circumstances discussed in this press release may not occur and actual results could differ materially and adversely from those anticipated or implied in the forward-looking statements. You should not rely upon forward-looking statements as predictions of future events. Although Aura believes that the expectations reflected in the forward-looking statements are reasonable, it cannot guarantee that the future results, levels of activity, performance or events and circumstances reflected in the forward-looking statements will be achieved or occur. Moreover, except as required by law, neither Aura nor any other person assumes responsibility for the accuracy and completeness of the forward-looking statements included in this press release. Any forward-looking statement included in this press release speaks only as of the date on which it was made. Aura undertakes no obligation to publicly update or revise any forward-looking statement, whether as a result of new information, future events or otherwise, except as required by law.

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