



Aura Biosciences Presents Updated Phase 1b/2 Clinical Data for AU-011 at the 43rd Annual Macula Society Meeting

Light-Activated AU-011 Holds Significant Potential as a First Line Treatment for Choroidal Melanoma

CAMBRIDGE, MA – February 24, 2020 – Aura Biosciences, a clinical-stage biopharmaceutical company developing a new class of tumor targeted therapies for initial application in primary tumors such as ocular and bladder cancers, today announced the presentation of updated clinical data from its ongoing Phase 1b/2 clinical trial evaluating the safety and efficacy of light-activated AU-011, the Company’s lead product candidate for the first line treatment of primary choroidal melanoma, at the 43rd Annual Macula Society Meeting, which took place February 19-22, 2020 in San Diego, CA.

“The Phase 1b/2 data being recognized at the Macular Society meeting this year show that AU-011 continues to be well tolerated, with a statistically significant reduction in tumor growth rate and vision preservation,” said Amy C. Schefler, M.D., Weill Cornell Medical College and Retina Consultants of Houston.

“The high tumor control rates observed to date, together with the high rate of vision preservation, including in patients at high risk for vision loss with sub-macular and peripapillary tumors continue to support AU-011’s significant potential to change the treatment paradigm for patients battling choroidal melanoma,” said Cadmus Rich, MD, Chief Medical Officer and Head of Research and Development of Aura Biosciences. “I am proud of the work the company has done to make a significant progress in this ultra-rare disease.”

Updated Results from the Phase 1b/2 Study Evaluating AU-011

Dr. Schefler gave an oral presentation titled, “Study Update of an Ongoing Phase 1b/2 Open-label Clinical Trial of AU-011 for the Treatment of Small to Medium Choroidal Melanoma,” which highlighted updated data from the ongoing open-label Phase 1b/2 clinical trial, and described the design of the planned Phase 3 trial that Aura expects to initiate during the second half of 2020.

The updated data demonstrate that multiple administrations of light-activated AU-011 were well-tolerated. Among the patients evaluated for safety (n=52), the most common treatment-related adverse events (AEs) were expected and included anterior chamber inflammation, posterior chamber inflammation and increase in intraocular pressure; all were manageable with standard-of-care treatments and the majority resolved without clinical sequelae. Notably, the posterior inflammation appears to originate within and/or around the tumor which is consistent with AU-011’s mechanism of action of acute tumor necrosis. There were two treatment-related serious AEs (vision loss; 3.6%). Tumor control and vision preservation data continue to be supportive of the planned Phase 3 registration trial.

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 (source: OMF). 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 lack of approved therapies, and the comorbidities of radioactive treatment options.

About Light-Activated AU-011

AU-011 is a first-in-class targeted therapy in development for the treatment of primary choroidal melanoma. The therapy consists of proprietary viral-like particle bioconjugates (VPB) that are activated with an ophthalmic laser. The VPBs bind selectively to unique receptors on cancer cells in the eye and are derived from technology originally pioneered by Dr. John Schiller of the Center for Cancer Research at the National Cancer Institute (NCI), recipient of the 2017 Lasker-DeBakey Award. Upon activation with an ophthalmic laser, the drug rapidly and specifically disrupts the cell membrane of tumor cells while sparing 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 has been granted orphan drug and fast track designations by the U.S. Food and Drug Administration and is currently in clinical development.

About Aura Biosciences

Aura Biosciences, Inc. is a clinical-stage biopharmaceutical company developing a new class of oncology therapies based on a novel tumor targeting technology for initial application in primary tumors such as ocular and bladder cancers with the potential to treat other cancers. The Company's proprietary technology platform utilizes viral-like particle bioconjugates (VPBs) that have a dual selectivity mechanism with targeted binding to cancer cells, followed by activation using a common laser. These potent cytotoxic therapies result in acute tumor cell necrosis, while sparing surrounding healthy tissue and activating the immune system to generate an anti-tumor response. Aura's lead product candidate belzupacap serotalocan (AU-011) is currently in Phase 2 development for the treatment of choroidal melanoma, a vision and life-threatening form of eye cancer for which there are currently no approved therapies. In a Phase 1b/2 study, AU-011 demonstrated compelling efficacy, including high rates of tumor control and vision preservation, along with a favorable safety profile, in patients with choroidal melanoma. The development of AU-011 is partially supported by funding awarded under a CRADA from the U.S. National Institutes of Health's National Cancer Institute. Future pipeline applications for Aura's technology include choroidal metastases and primary bladder carcinoma. Aura is headquartered in Cambridge, MA. For more information, visit www.aurabiosciences.com or follow us on [Twitter](#).



Investor and Media Contact:

Joseph Rayne

Argot Partners

617.340.6075 | joseph@argotpartners.com