



Aura Biosciences Announces Update of Clinical Safety and Efficacy Data on Lead Candidate AU-011 for Choroidal Melanoma

June 17, 2018

CAMBRIDGE, Mass., June 17, 2018 — Aura Biosciences, a biotechnology company developing a new class of therapies to target and selectively destroy cancer cells using viral capsid conjugates, today announced new interim safety and efficacy data from an open-label Phase 1b/2 study of its lead program, light-activated AU-011 for the treatment of primary choroidal melanoma. The findings were presented by Ivana Kim, M.D., Co-Director of the Ocular Melanoma Center at Massachusetts Eye and Ear, at the 2018 World Ophthalmology Congress in Barcelona.

AU-011 is an investigational, first-in-class targeted therapy in development for the treatment of primary choroidal melanoma. FDA has already granted Fast Track Designation and orphan drug designation, recognizing that there are no FDA-approved therapies and that the disease is serious and life-threatening.

AU-011 is being evaluated in a Phase 1b/2 open-label, multicenter trial designed to evaluate the safety and efficacy of single and multiple ascending doses in 30 adult subjects with clinically diagnosed small to medium primary choroidal melanoma.

Interim data presented today show that AU-011 has been generally well-tolerated with no related serious adverse events, no severe adverse events and no dose-limiting toxicities observed. Adverse events were manageable with standard of care treatments and had no further clinical sequelae. Pre-treatment visual acuity was maintained in all subjects that have been followed for 6 to 12 months.

Early efficacy results are very promising with two subjects in the first multiple-ascending-dose cohort showing evidence of reduction in tumor height at 3 months. Further evidence of preliminary efficacy has also been demonstrated with subtherapeutic doses in the single-ascending-dose cohorts providing stable disease with vision preservation up to 12 months.

"We are excited by these preliminary findings showing AU-011 provided local tumor control without loss of visual acuity for a majority of subjects dosed to date," said Dr. Kim. "That is an encouraging sign of progress toward developing a new treatment for this cancer that could preserve much more vision than radiotherapy, which is the current standard of care but not FDA approved for this indication."

"These findings indicate that Aura's novel, targeted, light-activated treatment could hold real promise for patients with choroidal melanoma," said Cadmus Rich, M.D., Chief Medical Officer of Aura. "Our team is looking forward to continuing our Phase 1b/2 study with Dr. Kim and our other collaborators at leading ophthalmology centers across the country."

About choroidal melanoma

Choroidal melanoma is a rare and aggressive type of eye cancer. Choroidal melanoma is the most common primary ocular tumor 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 against the exterior of the eye over the tumor. This technique can control the melanoma but can also lead to radiation-related cataract, retinopathy, optic nerve damage and loss of vision. The alternative is enucleation, or removal of the eye. Choroidal melanoma metastasizes to the liver in about 40 percent of cases in the long term (source: [OME](#)), and only 15 percent of patients whose melanoma has metastasized survive beyond five years after diagnosis (source: [ACS](#)).

About light-activated AU-011

AU-011 is a first-in-class targeted therapy in development for the primary treatment of choroidal melanoma. The therapy consists of patented viral capsid conjugates (VCC) with IR-700DX dye molecules that are activated with an ophthalmic laser. The VCCs 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. The IR-700DX dye molecules are produced by LI-COR Biosciences and are licensed exclusively to Aura for treating ocular cancers. Upon activation with an ophthalmic laser, the drug rapidly and specifically disrupts the membranes 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 the 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 is developing a new class of therapies to selectively target and destroy cancer cells. Its lead program, AU-011 in choroidal melanoma, is being developed under a CRADA with the National Cancer Institute (NCI), part of the National Institutes of Health. For more information, visit www.aurabiosciences.com.

About Massachusetts Eye and Ear

[Massachusetts Eye and Ear](#), founded in 1824, is an international center for treatment and research and a teaching hospital of Harvard Medical School. A member of Partners HealthCare, Mass. Eye and Ear specializes in ophthalmology (eye care) and otolaryngology—head and neck surgery (ear, nose and throat care). Mass. Eye and Ear clinicians provide care ranging from the routine to the very complex. Also home to the world's largest community of hearing and vision researchers, Mass. Eye and Ear has pioneered new treatments for blindness, deafness and diseases of the head and neck. Our scientists are driven by a mission to discover the basic biology underlying these conditions and to develop new treatments and cures. For more information about life-changing care and research at Mass. Eye and Ear, please visit our blog, [Focus](#) or visit MassEyeAndEar.org.

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