Aura Biosciences Announces Initiation of Phase 1b Clinical Trial and Receipt of FDA Fast Track Designation for AU-011 for the Treatment of Primary Ocular Melanoma

First patient dosed with light-activated AU-011 at Wills Eye Hospital in Philadelphia

CAMBRIDGE, Mass. – March 30, 2017 – Aura Biosciences, a biotechnology company developing a new class of therapies to target and selectively destroy cancer cells using viral nanoparticle conjugates, announced today that it has enrolled and dosed the first patient in its Phase 1b clinical trial of light-activated AU-011, an investigational, first-in-class targeted therapy in development for the treatment of ocular melanoma, a rare and life-threatening disease. Aura additionally announced today that the U.S. Food and Drug Administration (FDA) has granted AU-011 fast track designation for the treatment of primary ocular melanoma, also known as uveal or choroidal melanoma. This designation enables Aura to have more frequent interactions with the FDA throughout AU-011's drug development process, as well as priority review of the New Drug Application (NDA). Fast track designation is granted to drugs that are for serious or life-threatening diseases and that the FDA believes demonstrate the potential to address unmet medical needs.

"Patients with ocular melanoma currently have few treatment options available that can effectively destroy tumor cells while still preserving vision. Aura's trial is an important step in understanding the potential of AU-011 as a safe and novel therapeutic option to improve the outlook for these patients," said Carol Shields, M.D., Co-Director of the Ocular Oncology Service at Wills Eye Hospital in Philadelphia, where the first patient in this trial was dosed. She is also a professor of ophthalmology at Thomas Jefferson University and a member of Aura's Clinical Advisory Board.

Dr. Shields' work with AU-011 builds on years of pioneering research in early diagnosis of ocular melanoma using clinically relevant risk factors for timely recognition and treatment. The doctors at Wills Eye Hospital and other ocular oncology leaders across the country have pursued early diagnosis in an effort to reduce treatment-related visual loss and prevention of metastatic disease. Writing in a recent issue of *Nature Eye*, Dr. Shields and her team advocate for the development of new treatment options that can be used earlier in the course of disease while preserving vision for patients, based on the advances in earlier diagnosis of ocular melanoma using established risk factors.

"We look forward to evaluating and advancing AU-011 alongside Dr. Shields and other renowned researchers at ocular oncology centers of excellence in the U.S.," said Elisabet de los Pinos, Ph.D., founder and CEO of Aura. "Moreover, we will continue to work closely with the FDA under AU-011's fast track designation to shape our clinical program with their input. Ultimately, our goal is to equip the physicians who diagnose ocular melanoma early with a new targeted therapy that both prevents tumor growth and leaves other key ocular structures unaffected, thereby preserving vision for patients."

Trial investigators will focus on evaluating the safety of two dose levels of AU-011 for the treatment of patients with small-to-medium primary ocular melanoma. The Phase 1b open-label trial will enroll up to a total of 12 adult patients at Wills Eye Hospital and other ocular oncology centers across the country. Potential participants must have a confirmed ocular melanoma diagnosis not previously treated. Study investigators will conduct patient follow-up throughout a two-year observation period. For more information, visit www.clinicaltrials.gov or contact clinical@aurabiosciences.com.

About ocular melanoma

Ocular melanoma, also known as uveal or choroidal melanoma, develops in the uvea, or uveal tract, of the eye, and is a rare and aggressive eye cancer. No targeted therapies are available at present, and current treatments can be associated with potential visual morbidities. 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 cataracts, retinopathy and loss of vision. The alternative is enucleation, or removal of the eye. Ocular melanoma metastasizes to the liver in about 40 percent of cases in the long-term (source: OMF), 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 ocular melanoma, also known as uveal or choroidal melanoma, a rare and life-threatening disease. The therapy consists of viral nanoparticle conjugates that bind selectively to cancer cells in the eye and is derived from technology originally pioneered by Dr. John Schiller of the Center for Cancer Research at the National Cancer Institute (NCI). Upon activation with an ophthalmic laser, the drug rapidly and specifically destroys the membranes of tumor cells while sparing key eye structures, which may allow for the potential of preserving patients' vision. AU-011 for ocular melanoma has been granted orphan drug and fast track designations by the U.S. Food and Drug Administration and is currently in clinical testing.

About Aura Biosciences

Aura Biosciences is developing a new class of therapies to target and destroy cancer cells selectively. Its lead program, AU-011 in ocular 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.

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