NEWS RELEASE

FDA Grants Orphan Drug Designation to Aura Biosciences' Novel Treatment for Uveal Melanoma

McGill University Health Centre researchers present pre-clinical in vivo data at the Annual Meeting of the Association for Research in Vision and Ophthalmology (ARVO)

CAMBRIDGE, MASSACHUSETTS – May 21, 2015 – Aura Biosciences, a biotech company developing highly tumor-targeted breakthrough therapies for rare cancers, has been granted Orphan Disease Designation by the FDA for its drug AU-011 for the treatment of Uveal Melanoma. The FDA's Orphan Drug Designation program provides orphan status to drugs and biologics, which demonstrate promise for the diagnosis and/or treatment of rare diseases or conditions in the US. In addition, the first oral presentation of Aura Biosciences' pre-clinical research, '*Evaluating the in vivo efficacy of a first-in-class drug for the treatment of primary uveal melanoma',* was recently delivered by McGill University Health Centre researchers at the ARVO (Association for Research in Vision and Ophthalmology) Annual Meeting.

"There are currently no approved drug therapies for the treatment of uveal melanoma which is a rare but life threatening disease. We are thrilled to receive this Orphan Drug Designation that, together with the positive preclinical data, is enabling us to move this drug one step closer to the clinic," said Elisabet de los Pinos, founder and CEO of Aura Biosciences.

Uveal Melanoma, also referred to as Ocular Melanoma (OM), is an aggressive form of cancer that develops in the uvea, or uveal tract, of the eye. The primary tumor is diagnosed when it is still in the eye but has no targeted therapies available and is usually treated with an invasive radioactive plaque placed against the exterior of the eye near the tumor. This treatment requires multiple surgeries and can lead to cataracts, retinopathy and loss of vision. The alternative is enucleation, a surgery to remove the eye.

"There is an unmet medical need in the rare cancer patient community with which we work to have access to treatments that target the primary tumor and spare vision, forgoing risky and invasive surgical intervention. Aura's novel approach has the potential to dramatically improve the outcomes, and hope, for patients with uveal melanoma," said Grant Allen, Co-founder and Chairman of the Ocular Melanoma Foundation. "The Orphan Drug Designation for AU-011 is a huge step forward, enabling Aura to potentially bring this drug to patients in an expedited manner."

"The Melanoma Research Foundation's CURE OM initiative leads the field in OM research support. We appreciate partners who are also working to advance the OM field and applaud efforts to develop new treatments with the goal of saving lives. Today's news about a treatment that could potentially eliminate OM tumors early in the eye brings hope for improved outcomes for patients," said Sara Selig, MD, CURE OM Co-Founder and Director, Melanoma Research Foundation.

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AU-011 consists of Viral-like Nanoparticles that demonstrate highly selective targeting of solid tumors while leaving normal epithelium untouched. Aura's lead product incorporates its Viral-like Nanoparticle conjugated with a potent cell-killing laser-activated molecule that is delivered efficiently and selectively to cancerous cells, thereby reducing or eliminating the risk of non-specific activity and undesirable toxicity. The molecule is activated by a standard ophthalmologic laser and treatment will be administered in an outpatient visit. A video explaining how Aura's technology is applied to Uveal Melanoma can be accessed at the Aura Biosciences website at aurabiosciences.com.

ARVO Presentation – Data on AU-011

In vivo research behind the ARVO oral presentation last week was conducted by *Patrick T. Logan, Sultan Aldrees, Mohammed F. Qutub, Natalia Vila, Vasco Bravo-Filho, and Miguel N. Burnier* at the Henry C Witelson Lab, Ocular Pathology, McGill University, Montreal, QC, Canada. In this study, the data showed that AU-011 is efficacious for treating uveal melanoma tumors in an orthotopic xenograft model.

Key findings of the research include:

- Complete response – following three administrations of AU-011 complete tumor eradication was observed by histopathology.
- The treatment appeared to be "retina sparing."
- The observation of tumor necrosis is consistent with the mechanism of action of AU-011.

"In all of our years running this pre-clinical uveal melanoma animal model that we established more than a decade ago, this is the first time that we have seen such promising and encouraging results for the treatment of primary uveal melanoma. We are very excited about the positive impact this might have in uveal melanoma patients," says Dr. Logan, who is managing the Henry C. Witelson Ocular Pathology pre-clinical unit.

About Aura Biosciences

Aura Biosciences is applying nanotechnology to the fight against cancer. Its novel Viral-like Nanoparticle technology, developed in partnership with the National Cancer Institute (NCI), harnesses the potential of viral evolution and tumor targeting for the treatment of cancer. For more information, visit www.aurabiosciences.com.

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