

FOR IMMEDIATE RELEASE

CONTACT

Media

Ashlea Kosikowski
Pure Communications
910-547-7093
ashlea@purecommunicationsinc.com

Business Development

Keiko Mitsunobu
Acucela
425-527-0648
keiko.mitsunobu@acucela.com

Acucela Announces *Experimental Eye Research* Publication; Paper Outlines First Demonstrated Effect of Visual Cycle Modulation Treatment on Retinopathy

Dr. Ryo Kubota, Acucela President and CEO, a Co-Author with Colleagues at Children's Hospital and Harvard Medical of these Significant Findings

BOTHELL, Wash. (August 9, 2010) – Acucela, a clinical-stage biotechnology company focused on developing new treatments for blinding eye diseases, announced today that a scientific paper co-authored by Ryo Kubota, M.D., Ph.D., chairman, president and chief executive officer of Acucela, has been published in *Experimental Eye Research* (Exp. Eye. Res. 2010, 91;153-61). The paper, “Visual Cycle Modulation in Neurovascular Retinopathy” was also co-authored by scientists from Children’s Hospital and Harvard Medical School in Boston and demonstrates for the first time the impact of treatment with a visual cycle modulator (VCM) on retinopathy in an immature eye.

“These findings are key for illustrating the effect visual cycle modulation can have on the eye and blinding eye diseases,” stated Dr. Kubota. “Visual cycle modulators - like Acucela’s own ACU-4429 - are designed to prevent or inhibit the generation of toxic by-products of the eye’s visual cycle that can lead to degenerative eye conditions like dry age related macular degeneration, or dry AMD. In this paper we were able to demonstrate the possibility of reducing the oxygen demand in the retina by modulating the visual cycle to treat neurovascular retinopathy, like retinopathy of prematurity or the even more common condition like diabetic retinopathy. By showing that VCM treatments can regulate rod function in a preclinical model, my co-authors and I have demonstrated the early promise and potential of this treatment approach for patients suffering from more blinding eye diseases beyond AMD.”

The *Experimental Eye Research* paper describes a preclinical study in *in vivo* models of oxygen-induced retinopathy (OIR), which is a preclinical model of the pediatric disease retinopathy of prematurity (ROP). The study results provide evidence that the eye’s rod cells may be a possible therapeutic target in neurovascular diseases such as retinopathy of prematurity. Recent findings in OIR preclinical models imply a causal role for the rods in the ROP disease process. The study outlined in the publication was conducted to experimentally manipulate rod function and to establish this causal role conclusively. The authors concluded that visual cycle modulation

resulted in altered rod function and in enhanced recovery of retinal vessels in OIR. In summary, these findings are the first demonstration of a treatment effect with a VCM on retinopathy in an immature eye and demonstrate a causal role for the rod photoreceptors in the development of retinal vasculopathy.

Acucela's proprietary visual cycle modulator, ACU-4429, is an investigational oral treatment for dry age-related macular degeneration (dry AMD) that is currently in Phase 2 clinical development as part of the ENVISION Clarity Trial. ACU-4429 is one of the only treatments in development that works to regulate the eye's visual cycle for processing light. By regulating this cycle, ACU-4429 has demonstrated the ability to decrease the levels of toxic by-products in the eye and thereby potentially slow the advancement of dry AMD. AMD is a leading cause of vision loss in people over the age of 50. Dry AMD accounts for 90% of the AMD patients, and there are no therapies currently approved to treat this condition.

About the ENVISION Clarity Trial

The ENVISION (Evaluating a Novel **VISION** treatment for AMD) Clarity Trial is part of Acucela's clinical program evaluating the investigational oral treatment ACU-4429 in patients with dry age-related macular degeneration (dry AMD). The Clarity Trial, a Phase 2 clinical trial of ACU-4429 in patients with dry AMD, was launched in January 2010 and builds upon the promising preclinical findings and initial data from Acucela's Phase 1 clinical studies. These initial Phase 1 data have been presented at the Association for Research in Vision and Ophthalmology (ARVO) 2009 Annual Meeting, the Aegean Retina XI Meeting and the 8th International Symposium on Ocular Pharmacology and Therapeutics (ISOPT) and demonstrate the safety and tolerability of ACU-4429 in healthy volunteers aged 55-80. In addition, these data mark the first time that a non-retinoid therapeutic in a convenient pill form has effectively targeted the visual cycle in a dose-dependent manner.

About ACU-4429

ACU-4429 utilizes Acucela's proprietary visual cycle modulation (VCM) technology, and is designed to prevent or inhibit the generation of toxic by-products of the visual cycle that can lead to degenerative eye conditions like dry AMD. In March 2010, ACU-4429 was granted Fast Track designation by the U.S. Food and Drug Administration. Preclinical data indicate that ACU-4429 slows the rod visual cycle, resulting in decreased accumulation of a toxic by-product that is the precursor of lipofuscin, which are deposits of toxic substances. The chronic accumulation of lipofuscin has been implicated in degenerative retinal diseases. ACU-4429 is administered to patients as an oral, daily pill rather than by injection into the eye, which is typical of many current eye therapeutics. Acucela has forged a strategic partnership with Otsuka Pharmaceutical, Co. Ltd. to co-develop ACU-4429 in dry AMD as well as other potential indications in North America.

About Acucela Inc.

Acucela Inc. is a clinical-stage biotechnology company focused on leveraging promising science in visual cycle modulation (VCM) to develop new methods for treating blinding eye diseases that affect tens of millions of people worldwide. The company's orally-delivered VCM therapies, which selectively target cells within the retina to protect visual acuity, have the potential to be used to treat several devastating eye diseases, including dry age-related macular degeneration

(AMD), retinopathy of prematurity, Stargardt disease and diabetic retinopathy. Acucela is also developing, with Otsuka Pharmaceutical, Rebamipide ophthalmic suspension, an Otsuka discovered product candidate for dry eye in the United States. For more information, please visit www.Acucela.com.

###