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**Acucela to Present ACU-4429 Phase 1 Data at the 10th International Symposium on Ocular Pharmacology and Therapeutics**

*Comprehensive Phase 1a and b Findings for Novel, Oral Compound for Dry AMD to be Highlighted*

**Seattle, Washington and Vienna, Austria (November 30, 2011)** – Acucela Inc., a Washington State-based clinical-stage biotechnology company focused on developing new treatments for blinding eye diseases, today announced that data for ACU-4429, the Company’s novel, small-molecule visual cycle modulator (VCM) for the treatment of dry age-related macular degeneration (dry AMD), will be highlighted at the 10th International Symposium on Ocular Pharmacology and Therapeutics (ISOPT) being held in Vienna, Austria from December 1-4, 2011. John W. Chandler, M.D., Acucela’s vice president clinical affairs, will present: “Progress in the Development of ACU-4429 for the Treatment of Dry AMD” during the Retina: Dry AMD portion of the ISOPT program on December 1, 2011, at 4:40 p.m. local time.

Dr. Chandler will discuss comprehensive data from the Phase 1a and 1b clinical studies of ACU-4429. The Phase 1a study assessed the safety, tolerability, pharmacokinetics and pharmacodynamics of a single dose administration of ACU-4429 in healthy volunteers aged 55-80. In this single-center, randomized, double-masked, placebo-controlled, dose-escalation study, oral administration of ACU-4429 was well-tolerated and a dose-dependent modulation of the visual cycle was demonstrated using electroretinography (ERG), an established eye test. Importantly, these data marked the first time that a non-retinoid therapeutic in an oral pill form has effectively targeted the visual cycle in a dose-dependent manner.

The Phase 1b study assessed the safety, tolerability and pharmacokinetics of daily doses of ACU-4429 for 14 days in 40 healthy volunteers aged 26-55. This study found that ACU-4429 was well tolerated for 14 days at doses up to 40 mg per day.

“We are pleased to present the full results of our Phase 1b study for ACU-4429 for the first time at a major medical meeting,” stated Dr. Chandler. “Together, these Phase 1 studies show that ACU-4429 is well-tolerated and demonstrated its ability to slow the visual cycle and potentially decrease accumulation of toxic by-product in the retina. These findings continue to illustrate the potential for ACU-4429 in the treatment of dry AMD, for which there is no currently approved therapy.”

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Initial data from these Phase 1 clinical trials have been presented at other key scientific meetings and were published earlier this year in *Retina: The Journal of Retinal and Vitreous Diseases*. ACU-4429 is currently being studied in a Phase 2 multicenter, randomized clinical trial in patients with dry AMD and the Company expects to announce data from that trial in 2012.

AMD is a chronic degenerative disease of the retina and is the most common cause of permanent visual loss among the elderly population. AMD is classified into two forms: the wet (neovascular) form and the dry (non-neovascular) form. According to Frost & Sullivan, dry AMD is the most common form, representing about 90 percent of all cases, and approximately 15 percent of those who suffer from AMD develop intermediate or advanced dry AMD, typically exhibited with geographic atrophy, with the risk of developing advanced dry AMD increasing with age. Frost & Sullivan estimates that the prevalence of wet AMD and advanced dry AMD in the United States was 1.44 million and 0.72 million in 2009 and will be 1.61 million and 0.81 million, respectively, by 2016.

#### **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 2010, ACU-4429 was granted Fast Track designation by the U.S. Food and Drug Administration. 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 for retinal disorders. 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. ([www.acucela.com](http://www.acucela.com)) 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 compounds, which selectively target cells within the retina to protect visual acuity, have the potential to treat dry AMD, for which there is no currently approved treatment, retinopathy of prematurity, Stargardt disease and diabetic retinopathy. ACU-4429 for dry AMD is being co-developed by Acucela and Otsuka Pharmaceutical Co., Ltd. in North America, and the companies are also co-developing rebamipide for dry eye and OPA-6566 for glaucoma in the United States. Acucela was founded by Ryo Kubota, M.D., Ph.D., a pioneer in ophthalmology and the discoverer of the gene that causes glaucoma.

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