

FOR IMMEDIATE RELEASE

Aerpio Therapeutics Initiates Phase 2 Study of Tie2 Activator AKB-9778 for the Treatment of Diabetic Macular Edema

Cincinnati, OH, February 13, 2014 – Aerpio Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on advancing innovative therapies for vascular diseases, today announced that it has dosed the first patient in a Phase 2 trial evaluating AKB-9778, a Tie2 activator, alone and in combination with ranibizumab (Lucentis®) for the treatment of diabetic macular edema (DME). AKB-9778 is a first-inclass inhibitor of human protein tyrosine phosphatase beta (HPTPβ) that activates the Tie2 pathway to promote vascular stability, preventing abnormal blood vessel growth and vascular leak.

Aerpio announced at the American Academy of Ophthalmology 2013 the results of the Phase 1b/2a study in patients with DME, in which AKB-9778 administered subcutaneously as monotherapy over 28 days was well-tolerated and produced clinically meaningful reductions in retinal thickness, which correlated with improved visual acuity.

"Emerging preclinical and clinical data indicate that the Angiopoietin/Tie2 pathway is a major axis for maintenance and stabilization of retinal blood vessels," said Kevin Peters, MD, Chief Scientific Officer and VP of Research and Development, Aerpio. "Results in preclinical models and in our Phase 1b study indicate that AKB-9778 has great promise as monotherapy in both treatment-naïve and treatment-resistant DME patients. In addition, since Tie2 activation is complementary to the action of anti-VEGF agents, the current standard of care for patients with DME, AKB-9778 could also represent a major advance as an adjunct to anti-VEGF therapy."

"Alternative therapies are needed for treating patients with DME who have persistent macular edema and vision loss despite frequent anti-VEGF injections and also for patients who don't want or don't tolerate intravitreal injections," said Jeffrey Heier, MD, Ophthalmic Consultants of Boston. "Based on a compelling scientific background of preclinical and early clinical data, AKB-9778 may provide a patient self-administered alternative that could be helpful in the treatment of diabetic macular edema."

The randomized, double-masked, double dummy, Phase 2 study is designed to assess the safety and efficacy of AKB-9778 administered over three months as monotherapy and as an adjunct with ranibizumab in subjects with DME. The primary endpoints of the study are change from baseline in visual acuity and change from baseline in central retinal thickness in the groups treated with AKB-9778 monotherapy and AKB-9778 as an adjunct with ranibizumab compared to ranibizumab monotherapy. The study will enroll approximately 120 subjects (40 patients/group) at approximately 35 sites.

About AKB-9778



AKB-9778 is a first-in-class small molecule that works by inhibiting the human protein tyrosine phosphatase β (HPTP β) enzyme, which acts as a negative regulator of the Tie2 receptor. By inhibiting this negative regulator, Tie2 signaling is restored, overcoming the effects of the Ang2-induced vascular destabilization. Aerpio is currently focusing development of its lead candidate, AKB-9778, in diabetic macular edema (DME), however, Tie2 activators have potential utility in a range of important clinical indications. In a Phase 1b/2a study in DME patients, AKB-9778 was well tolerated throughout 28 days of dosing, with evidence of disease improvement in some patients. A Phase 2 study to confirm efficacy of AKB-9778 alone and in combination with ranibizumab in patients with DME is currently ongoing.

About Aerpio Therapeutics

Aerpio Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on advancing innovative therapies for vascular diseases. Aerpio is a leader in the development of small molecule drugs based on Tie2 activation and the stabilization of hypoxia-inducible factor 1α (HIF- 1α). The Company's lead program, AKB-9778, is a first-in-class stabilizer of the Tie2 pathway and is in clinical development for diabetic macular edema. More information is available at www.aerpio.com.

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