Aerpio Therapeutics Presents Full Results from Phase 1b/2a Study of AKB-9778 for the Treatment of Diabetic Macular Edema at ARVO Annual Meeting

Cincinnati, OH, May 5, 2014 – Aerpio Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on advancing innovative therapies for vascular diseases, today announced the presentation of the full results of its Phase 1b/2a study of Tie2 activator, AKB-9778, for the treatment of diabetic macular edema (DME) at the Association for Research in Vision and Ophthalmology Annual Meeting (ARVO) in Orlando, FL. In the study, one month of daily AKB-9778 treatment was well-tolerated, produced clinically meaningful reduction in retinal thickness, with concomitant improvement in visual acuity, in some of the patients.

Tie2 plays a central role in maintaining vascular integrity, which prevents vascular leak seen in conditions such as DME. AKB-9778 activates Tie2 by inhibiting the natural brake of Tie2, human protein tyrosine phosphatase β (HPTPβ). This is the first study to show that activation of Tie2 can have a beneficial effect in patients with DME.

“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 these early clinical data, AKB-9778 may provide a patient self-administered alternative that could be helpful in the treatment of diabetic macular edema.”

“We are highly encouraged by the pilot efficacy and continued favorable safety profile for AKB-9778 in this Phase 1b/2a study and have recently initiated a larger, confirmatory Phase 2 study,” commented Joseph Gardner, PhD, President and CEO of Aerpio. “We are hopeful that AKB-9778 could represent a novel, more convenient therapy for patients with DME either as monotherapy or in combination with VEGF inhibitors, the current standard of care.”

The poster presented at ARVO, “A Phase 1b/2a Open-label, Multiple-ascending Dose Cohort Study to Assess the Safety, Tolerability, Pilot Efficacy, Pharmacokinetics and Pharmacodynamic Effects of 28-day Repeat Subcutaneous Doses of AKB-9778 in Subjects with Diabetic Macular Edema,” was authored by Mitchell Brigell, Peter Campochiaro, Raafay Sophie, Michael Tolentino, Daniel Miller, David Browning, David Boyer, Jeffrey Heier, Barbara Withers, Laura Gambino and Kevin Peters. 24 patients with DME participated in the 28-day trial. Cohorts of 6 patients each were treated with 5 mg, 15 mg, 22.5 mg and 30 mg of AKB-9778 delivered subcutaneously BID for 28 days and patients were observed for an additional 56 days. All dose levels of AKB-9778 were well-tolerated, with no serious adverse events observed. A transient, generally asymptomatic reduction in blood pressure was observed at the 22.5 and 30 mg doses. After 1 month of treatment at doses of 15 mg or greater, 7 out of 18 patients demonstrated a reduction in central retinal subfield thickness (CRT) of greater than 50µm and 13 out of

18 patients gained 5 or more letters of visual acuity. These data warrant the continued study of AKB-9778 as a treatment for DME. A Phase 2 study of AKB-9778 alone and in combination with ranibuzumab for the treatment of DME is currently ongoing.

About AKB-9778

AKB-9778 is a first-in-class small molecule that works by inhibiting the human protein tyrosine phosphatase β (HPTPβ) 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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