Aerpio Therapeutics Announces Publication of Positive Results of Phase 1b/2a Clinical Trial of Novel Tie2 Activator, AKB-9778, for the Treatment of Patients with Diabetic Macular Edema (DME) – TIME-1 Study

Ongoing Phase 2 Study of AKB-9778 Alone and in Combination with Ranibizumab for the Treatment of DME Has Completed Patient Enrollment, with Topline Data Anticipated Q2 2015

CINCINNATI--(BUSINESS WIRE)--Aerpio Therapeutics, Inc., a clinical-stage biopharmaceutical company focused on developing novel therapeutics for vascular disorders with an emphasis on diseases of the eye, today announced publication of positive results from the company's Phase 1b/2a clinical study of lead clinical candidate, AKB-9778, a Tie2 activator. AKB-9778 is a first-in-class inhibitor of human protein tyrosine phosphatase beta (HPTPβ) that activates the Tie2 pathway to promote vascular stability, preventing abnormal blood vessel growth and vascular leakage. The Phase 1b/2a study, named "TIME-1", showed that AKB-9778, administered subcutaneously for 28 days as monotherapy in patients with DME, was well-tolerated and produced clinically meaningful reductions in retinal thickness, correlating with improved visual acuity. The TIME-1 clinical results have been published in an article titled, "Treatment of Diabetic Macular Edema with an Inhibitor of Vascular Endothelial-Protein Tyrosine Phosphatase That Activates Tie2," DOI: http://www.aaojournal.org/article/S0161-6420(14)00901-4/fulltext, which is appearing in the March 2015 print edition of *Ophthalmology*, the journal of the American Academy of Ophthalmology.

A total of 24 patients with DME participated in the 28-day, open-label, dose-escalation Phase Ib/2a trial. Cohorts of six 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. As previously reported, all dose levels of AKB-9778 were well-tolerated, with no serious adverse events observed. After one month of treatment at doses of 15 mg or greater, 7 out of 18 patients demonstrated a reduction in central subfield thickness (CST) in the study eye of greater than 50µm, and 13 out of 18 patients gained 5 or more letters of visual acuity in the study eye as measured by the best corrected visual acuity (BCVA) assessment.

"The publication in the AAO journal is the first to demonstrate that activating Tie2, an alternative pathway to currently available anti-VEGF agents, can have a meaningful impact on both reduction in retinal edema and improved visual outcomes," said David S. Boyer, MD, Retina-Vitreous Associates Medical Group. "Importantly, the study showed a significant correlation between measures of BCVA and CST, providing further proof of concept for this promising therapeutic modality in patients." Based on the clinical results of the Phase 1b/2a study, in February 2014 Aerpio announced the initiation of a randomized, double-masked Phase 2 trial, named "TIME-2," evaluating AKB-9778 alone and in combination with ranibizumab (Lucentis®), for the treatment of DME.

"We are pleased with the progress of our AKB-9778 clinical program and that we have reached the milestone of completing enrollment on schedule in the TIME-2 study," stated Joseph Gardner, Ph.D., President and CEO of Aerpio. "We believe that AKB-9778 has the potential to profoundly impact the treatment of diabetic eye disease, both as monotherapy, as well as an adjunct to existing treatments. Based on our current timelines, we anticipate reporting topline data from the TIME-2 study sometime in the second quarter of 2015."

About AKB-9778

AKB-9778 is a first-in-class small molecule that inhibits 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 vascular destabilization. Aerpio is currently focusing development of its lead candidate, AKB-9778, in diabetic macular edema; however, Tie2 activators have potential utility in a range of important clinical indications, including: diabetic retinopathy; age-related macular degeneration; and retinal vein occlusion. 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 (TIME-2) to confirm efficacy of AKB-9778 alone and in combination with ranibizumab in patients with DME has completed enrollment and is currently ongoing.

About Aerpio Therapeutics

Aerpio Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on developing novel therapeutics for vascular disorders with an emphasis on diseases of the eye. Aerpio is the leader in the development of therapeutics targeting the Tie2 receptor and stabilizing 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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