Aerpio Therapeutics Reports Positive Clinical Results from the Phase 2a Study of Lead Candidate, AKB-9778, in Diabetic Macular Edema (DME): The TIME-2 Trial

- **The combination of Aerpio's AKB-9778, a Tie2 activator, and Lucentis® demonstrated significant benefit versus Lucentis® monotherapy in reduction of macular edema following 3 months of treatment.**
- **Systemically administered AKB-9778 demonstrated the ability to improve underlying diabetic retinopathy by 2 or more steps in eyes with and without DME.**
- **Though not powered for significance, TIME-2 also showed a trend towards improved visual acuity at month 3 when comparing combination therapy versus Lucentis® alone.**
- **AKB-9778 demonstrated an excellent safety profile.**

CINCINNATI--(BUSINESS WIRE)--Aerpio Therapeutics, Inc., a biopharmaceutical company focused on advancing first-in-class treatments for the eye, today announced that clinical data from the company's phase 2a study of its lead candidate, AKB-9778, for the treatment of patients with DME, were reported in an oral presentation delivered by Peter Campochiaro, MD, from Johns Hopkins Wilmer Eye Institute, during the Retina Subspecialty Day at the ongoing American Academy of Ophthalmology (AAO) Annual Meeting, taking place in Las Vegas, NV, November 13-17, 2015. The combination of AKB-9778 (dosed at 15 mg BID subcutaneously) and Lucentis® (ranibizumab injection dosed at 0.3 mg intravitreally) provided a clinically significant benefit in reduction of macular edema, as measured by central subfield thickness (CST), compared to Lucentis® alone at month 2 (p=0.02) and at month 3 (p=0.008). In association with the improvement in CST, the combination therapy showed a trend towards improved visual acuity (proportion of patients achieving improvement of at least 3 lines in visual acuity) when compared to Lucentis® alone. Systemically administered AKB-9778 also demonstrated the ability to improve underlying retinopathy by 2 or more steps on the diabetic retinopathy severity scale in eyes with and without DME. In regard to the safety profile, there were no clinically significant differences in the percentage of patients that experienced ocular or non-ocular adverse events across the three study arms.

"The results from the TIME-2 study demonstrate that AKB-9778 can significantly enhance the benefit of VEGF suppression in the treatment of DME," commented Dr. Pravin Dugel of Retinal Consultants of Arizona. "Another interesting and perhaps more compelling finding is the ability of systemically administered AKB-9778 to improve underlying diabetic retinopathy, both in eyes with and without DME. These early outcomes are very encouraging, and I look forward to the further progress of this program."

"TIME-2 met and exceeded our expectations as a proof-of-concept study to measure the activity of AKB-9778 concurrent with VEGF suppression," said Dr. Steven Pakola, Aerpio's Chief Medical Officer. "We look forward to advancing the program into later stage clinical development. Moreover, given our compound's mechanism of action in promoting the stabilization of vascular beds, we are exploring additional applications such as the treatment of diabetic retinopathy in the absence of DME."

Joseph Gardner, Aerpio's CEO stated, "We are pleased with the continued progress of our lead program, which has demonstrated a statistically significant improvement in the reduction in macular edema when combined with anti-VEGF therapy compared to anti-VEGF therapy alone, a positive trend toward greater visual acuity benefit (to be confirmed in a larger study), and no major impact on side effects when compared to anti-VEGF alone. Based on the promising results from TIME-2, we plan to advance AKB-9778 into later stage clinical studies in DME, as we also consider other ophthalmic indications, such as diabetic retinopathy in the absence of DME and wet AMD."

About the TIME-2 Study

TIME-2 was a phase 2a, randomized, double-masked, placebo-controlled, proof-of-concept study in patients with DME. The TIME-2 study evaluated 144 DME patients randomized equally (1:1:1) to AKB-9778 as monotherapy or in combination with Lucentis® compared with Lucentis® alone for a treatment period of 3 months, followed by a 2-month observation period. The study's primary endpoint measure was mean change from baseline in CST at 3 months. Secondary endpoint measures included visual acuity and safety outcomes.

About AKB-9778

AKB-9778 is a first-in-class small molecule that inhibits the enzyme, human protein tyrosine phosphatase β (HPTPβ), which acts as a negative regulator of the Tie2 receptor. By inhibiting this negative regulator, Tie2 signaling is restored, overcoming the effects of the vascular destabilization. Aerpio is initially focusing development of AKB-9778 in DME, with potential for development in other vascular retinal disorders, including diabetic retinopathy and wet age-related macular degeneration (AMD).

About Aerpio Therapeutics

Aerpio Therapeutics, Inc. is a clinical-stage biopharmaceutical company focused on the development of novel therapeutics for the treatment of vascular disorders with an emphasis on diseases of the eye. Aerpio is a leader in the development of therapeutics 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 small molecule 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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