



Aerpio Pharmaceuticals Announces Interim Results from its Phase 1b Clinical Trial of Topical Ocular Formulation of AKB-9778 for Primary Open Angle Glaucoma

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CINCINNATI--(BUSINESS WIRE)--Oct. 10, 2019-- Aerpio Pharmaceuticals, Inc. (Nasdaq:ARPO), a biopharmaceutical company focused on developing compounds that activate Tie2 to treat ocular diseases and diabetic complications, today announced interim results from its Phase 1b clinical trial of a topical ocular formulation of AKB-9778 in development as a potential treatment for primary open angle glaucoma (POAG).

The Phase 1b study is an ongoing randomized, double-masked study designed to assess increasing concentrations of AKB-9778 dosed topically as eye drops (5mg/ml QD, 15 mg/ml QD, 40 mg/ml QD and 40 mg/ml BID) in 4 sequential cohorts of 12 subjects, randomized 3:1 to receive AKB-9778 or placebo, for 7 days. The primary outcome of the study is ocular safety and tolerability with change in intraocular pressure (IOP) as a pharmacodynamic outcome. Conjunctival hyperemia grade and IOP were assessed prior to dosing and at 2-, 4- and 8-hours post-dose on Day 1 and Day 7. The unmasked interim analysis was limited to the first three cohorts (5 mg/ml QD, 15 mg/ml QD, 40 mg/ml QD). Topical ocular administration of AKB-9778 was well tolerated over 7 days at all dose levels for the first three cohorts. Compared to placebo, there was a dose dependent increase in minimal to mild conjunctival hyperemia with AKB-9778, which was transient and generally considered non-adverse. There was also a time and dose dependent reduction in IOP that, in the highest QD dose cohort peaked at 4 hours post-dose (-1.47 mmHg; $p = 0.041$)/-10.64%; $p = 0.027$) and was sustained through eight hours on day 7, returning to baseline levels at 24 hours post-dose. Based on these interim results, the Phase 1b protocol has been amended to include patients with ocular hypertension and POAG to further assess safety and activity on IOP in the target patient population. The Company expects to announce full results of the ongoing Phase 1b study in the first quarter of 2020.

"We are encouraged by the early safety findings and potential efficacy signal of this ongoing Phase 1b trial," said Kevin Peters, M.D., Chief Scientific Officer of Aerpio Pharmaceuticals. "The conventional outflow tract is responsible for the majority of fluid drainage from the front of the eye, which is critical for maintaining normal IOP and is often the site of pathology implicated in the increased IOP in patients suffering from POAG. Recent mouse and human genetic data establish a key role for the Tie2 pathway in the development and maintenance of Schlemm's canal, a key component of the conventional outflow tract. AKB-9778, a potent small molecule Tie2 activator, has shown IOP lowering effects after subcutaneous administration in two sequential Phase 2 studies in patients with diabetic retinopathy and normal IOP. We now look forward further to evaluating AKB-9778's safety and activity on IOP in patients with ocular hypertension and POAG."

About AKB-9778

AKB-9778 binds to and inhibits vascular endothelial protein tyrosine phosphatase (VE-PTP), an important negative regulator of Tie2. Decreased Tie2 activity contributes to vascular instability in many diseases including diabetes. AKB-9778 activates the Tie2 receptor irrespective of extracellular levels of its binding ligands, angiotensin-1 (agonist) or angiotensin-2 (antagonist) and may be the most efficient pharmacologic approach to maintain normal Tie2 activation.

About Aerpio Pharmaceuticals

Aerpio Pharmaceuticals, Inc. is a biopharmaceutical company focused on advancing first-in-class compounds that activate Tie2 to treat ocular diseases and complications of diabetes. Tie2 is an important regulator of vascular stability and its down-regulation is found in patients with diabetes and other conditions. Down-regulation is caused by activation of two inhibitors of Tie2, VE-PTP and Ang-2. The Company's lead compound, AKB-9778, is being investigated, in a topical drop formulation, for its potential as a treatment for open-angle glaucoma. For more information, please visit www.aerpio.com.

Forward Looking Statements

This press release contains forward-looking statements. Statements in this press release that are not purely historical are forward-looking statements. Such forward-looking statements include, among other things, the development of the Company's product candidates, including AKB-9778, the Company's plans for future development of its product candidates, including the timing of the Company's planned clinical trials and expected results from such clinical trials, and the therapeutic potential of the Company's product candidates. Actual results could differ from those projected in any forward-looking statements due to several risk factors. Such factors include, among others, the ability to continue to develop AKB-9778 or other product candidates, the inherent uncertainties associated with the drug development process, including uncertainties in regulatory interactions, commencing clinical trials and enrollment of patients in clinical trials, and competition in the industry in which the Company operates and overall market conditions. These forward-looking statements are made as of the date of this press release, and the Company assumes no obligation to update the forward-looking statements, or to update the reasons why actual results could differ from those projected in the forward-looking statements, except as required by law. Investors should consult all the information set forth herein and should also refer to the risk factor disclosure set forth in the reports and other documents the Company files with the SEC available at www.sec.gov.

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Source: Aerpio Pharmaceuticals, Inc.

Investor & Media:

Aerpio Pharmaceuticals, Inc.
Michael Rogers

Chief Financial Officer
mrogers@aerpio.com

or

Burns McClellan, on behalf of Aerpio Pharmaceuticals, Inc.

Media:

Robert Flamm, Ph.D.

rflamm@burnsmc.com

or

Investors:

John Grimaldi

jgrimaldi@burnsmc.com