



## **Apellis Pharmaceuticals Announces that APL-2 Met its Primary Endpoint in a Phase 2 Study in Patients with Geographic Atrophy, an Advanced Form of Age-Related Macular Degeneration**

### *Statistically Significant Slowing of Disease Progression Seen at 12 Months*

LOUISVILLE, KY., August 24, 2017 - Apellis Pharmaceuticals, Inc., a clinical-stage biopharmaceutical company developing a platform of novel therapeutic compounds for the treatment of autoimmune diseases, today announced that its complement C3 inhibitor, APL-2, met its primary endpoint in a Phase 2 clinical trial (FILLY) in patients with geographic atrophy (GA) associated with age-related macular degeneration (AMD). At 12 months, APL-2, administered monthly via intravitreal injection, showed a 29% ( $p=0.008$ ) reduction in the rate of GA lesion growth compared to sham. With every other month administration, a 20% ( $p=0.067$ ) reduction was observed. Additionally, in a post hoc analysis, a greater effect was observed during the second six months of the study: a reduction in growth rate of 47% ( $p<0.001$ ) with monthly administration, and a reduction of 33% ( $p=0.01$ ) with every other month administration.

The most frequently reported adverse events in the study eye were associated with the injection procedure. A higher incidence of exudative AMD was observed in the treatment groups, predominantly in subjects with a history of exudative AMD in the fellow eye, and was managed with the administration of standard-of-care therapies.

“We are very excited about the results of this study,” said Cedric Francois, MD, PhD, founder and chief executive officer of Apellis. “In addition to demonstrating a statistically significant slowing of disease over 12 months, APL-2’s effect appears to increase in the second six months of the study, slowing down the rate of degeneration by almost half. We plan to move forward with Phase 3 studies as soon as possible.”

David Boyer, MD, of Retina-Vitreous Associates Medical Group, said, “These results are very exciting for all people afflicted with dry AMD with geographic atrophy. It is currently an untreatable condition, and the reduction of the progression of atrophy in this trial offers new hope for vision maintenance for our patients.”

Results, including an analysis of genetic markers, will be presented at an upcoming major medical meeting.

#### **About the FILLY trial**

The FILLY trial is a 246-patient Phase 2 multicenter, randomized, single-masked, sham-controlled clinical trial of APL-2 in patients with GA conducted at 40 clinical sites, located in the United States, Australia and New Zealand. APL-2 was administered as an intravitreal injection in the study eye monthly or every other month for 12 months, followed by six months of monitoring after the end of treatment. Eyes were evaluated for GA by fundus autofluorescence photographs (FAF). The rate of GA area growth was

measured by mean change in square root area of GA lesion from baseline to month 12. The primary endpoint was the change in GA lesion size from baseline to month 12, compared to sham.

### **About APL-2**

APL-2 is designed to inhibit the complement cascade centrally at C3, and may have the potential to treat a wide range of complement-mediated diseases more effectively than is possible with partial inhibitors of complement. APL-2 is a synthetic cyclic peptide conjugated to a polyethylene glycol (PEG) polymer that binds specifically to C3 and C3b, effectively blocking all three pathways of complement activation (classical, lectin, and alternative). In addition to the FILLY trial in GA, Apellis is currently evaluating APL-2 in two clinical trials for systemic administration in paroxysmal nocturnal hemoglobinuria (PNH). Interim data from these trials demonstrated meaningful improvements in lactate dehydrogenase and hemoglobin levels in previously untreated patients as well as patients who are suboptimal responders to eculizumab, the current standard of care in the treatment of PNH. Phase 3 studies are planned in GA and PNH, and future clinical studies of APL-2 are anticipated in other diseases in which complement is implicated.

### **About geographic atrophy (GA)**

GA is an advanced form of age-related macular degeneration (AMD), a disorder of the central portion of the retina, known as the macula, which is responsible for central vision and color perception. GA is a chronic, progressive condition that leads to central blind spots and permanent loss of vision. Based on published studies, we estimate that approximately one million people have GA in the United States alone. There are currently no approved treatments for GA.

### **About Apellis**

Apellis Pharmaceuticals, Inc. is a clinical-stage biopharmaceutical company focused on the development of novel therapeutic compounds for the treatment of a broad range of life-threatening or debilitating autoimmune diseases based upon complement immunotherapy through the inhibition of the complement system at the level of C3. Apellis is the first company to advance chronic therapy with a C3 inhibitor into clinical trials. For additional information about Apellis and APL-2, please visit <http://www.apellis.com>.

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