Apellis

Apellis Reports Positive Interim Results from Phase Ib Clinical Trials of APL-2 in PNH

C3 inhibitor reduces hemolysis in patients with PNH, is generally well tolerated

LOUISVILLE, Ky., December 2, 2016 – Apellis Pharmaceuticals, Inc., a clinical-stage biopharmaceutical company focused on inhibition of the complement system, today will present positive interim results from two ongoing Phase Ib open-label, dose-escalation clinical trials of APL-2, a complement C3 inhibitor, in paroxysmal nocturnal hemoglobinuria (PNH). PNH is a rare, acquired, potentially life-threatening disease characterized by complement-mediated hemolytic anemia.

Interim results suggest that APL-2 reduces hemolysis in patients with PNH who receive daily subcutaneous injections of APL-2 as monotherapy or as an add-on to the standard of care, eculizumab. With APL-2 (270 mg) as monotherapy, three out of three (3/3) PNH patients achieved a reduction in lactate dehydrogenase (LDH) levels to below the standard for control in PNH (500 U/L). With APL-2 (270 mg) as add-on to eculizumab, six out of six (6/6) previously transfusion-dependent PNH patients did not require transfusions during the study and five out of six (5/6) PNH patients achieved hemoglobin levels within the normal range for healthy individuals.

All 15 PNH patients who have been dosed across the two trials to date have completed at least one month of dosing, with five having received more than three months of dosing. Based on preliminary assessment, APL-2 is generally well tolerated.

“Establishing that systemic inhibition of C3 is feasible with daily subcutaneous injections of APL-2 in patients with PNH is a significant achievement that validates C3 as a target in the complement pathway,” said Cedric Francois, M.D., Ph.D., chief executive officer of Apellis. “APL-2’s unique ability to target C3 and block all three pathways of the complement system is indicative of its potential to be an effective treatment for multiple complement-driven diseases. We are encouraged by the preliminary results APL-2 has demonstrated, as well as the favorable safety data observed thus far in the Phase Ib trials and our previous studies in healthy volunteers. All patients in the Phase Ib trials will have completed three months of dosing in January 2017.”

Apellis will present the Phase Ib interim results today at the International PNH Interest Group (IPIG) Annual Scientific Assembly and will present the poster “APL-2, a Complement C3 Inhibitor for the Potential Treatment of Paroxysmal Nocturnal Hemoglobinuria (PNH): Phase I Data from Two Completed Studies in Healthy Volunteers” tomorrow at the American Society of Hematology (ASH) Annual Meeting.

About Paroxysmal Nocturnal Hemoglobinuria

Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, acquired, potentially life-threatening disease characterized by complement-mediated hemolysis with or without hemoglobinuria, an increased susceptibility to thrombotic episodes and/or some degree of bone marrow dysfunction. A significant subset of patients treated with the current standard of care still suffer from debilitating anemia and transfusion dependence.

About APL-2

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) with a particularly high potency against the alternative pathway. This comprehensive inhibition of complement-mediated pathology may have the potential to control symptoms and modify underlying disease in patients suffering from PNH.

About the Phase Ib Clinical Trials of APL-2 in PNH

Apellis is evaluating APL-2 in two Phase Ib clinical trials. PADDOCK (ClinicalTrials.gov Identifier: NCT02588833) is assessing the safety, tolerability, pharmacokinetics (PK), pharmacodynamics (PD) and preliminary efficacy of multiple doses of APL-2 administered by daily subcutaneous injection (SC) in patients with PNH who have not received the standard of care in the past. PHAROAH (ClinicalTrials.gov Identifier: NCT02264639) is assessing the safety, tolerability, PK and PD of single and multiple doses of APL-2 administered by SC as an add-on to standard of care in patients with PNH.

About Apellis

Apellis is a clinical-stage biopharmaceutical company focused on the development of a platform of novel therapeutic compounds for the treatment of a broad range of autoimmune diseases based upon complement immunotherapy. Uncontrolled complement activation can lead to a wide range of life-threatening or debilitating disorders. Apellis is the first company to advance chronic therapy with a C3 inhibitor into clinical trials. Apellis is currently evaluating its lead product candidates in Phase 1 clinical trials in paroxysmal nocturnal hemoglobinuria (PNH) and in a Phase 2 clinical trial in geographic atrophy, the advanced form of dry age-related macular degeneration (AMD). For additional information about Apellis, please visit www.apellis.com.

###

Media Contact:
Annie Starr
6 Degrees
973-415-8838
astarr@6degreespr.com