Apellis Announces Positive Results from Phase 1 Clinical Trials of APL-2, a C3 Complement Inhibitor

LOUISVILLE, Ky., June 23, 2016 - Apellis Pharmaceuticals, Inc. today announced positive results from two Phase 1 clinical trials of its complement C3 inhibitor, APL-2. The trials were randomized, double-blind, placebo-controlled, single and multiple ascending dose studies designed to assess the safety, tolerability, pharmacokinetics (PK) and pharmacodynamics (PD) of subcutaneous injection in healthy adult volunteers.

Forty subjects were administered APL-2 subcutaneously (SC) either as a single dose (ranging from 45 to 1,440mg) or repeated doses for 28 consecutive days (ranging from 30 to 270 mg/day). Both studies concluded that pharmacological doses of APL-2 were safe and well tolerated and that APL-2's PK / PD profile supports daily SC administration. In addition, complement-mediated hemolysis (destruction of the red blood cells) was assessed and daily APL-2 doses of 180mg and 270mg significantly reduced hemolytic activity as early as eight days after the start of dosing, and this inhibition was maintained throughout the dosing period.

"We are pleased to have accomplished this major milestone in the clinical development of APL-2. Targeting C3 is very challenging as it is the most abundant complement protein in the body. It is the first time that a study demonstrates that inhibiting complement at the C3 level can be safely achieved in a clinical study," said Cedric Francois, M.D., Ph.D., Chief Executive Officer of Apellis.

In parallel, Apellis has also initiated two Phase 1b clinical trials in patients with paroxysmal nocturnal hemoglobinuria (PNH) designed to assess the safety, preliminary efficacy and pharmacokinetics of subcutaneously administered APL-2 alone or in combination with eculizumab. Data are expected to be presented at the American Society of Hematology (ASH) annual meeting in December 2016.

About Paroxysmal Nocturnal Hemoglobinuria

PNH is a debilitating disorder characterized by complement-mediated hemolysis with or without hemoglobinuria, an increased susceptibility to thrombotic episodes and/or some degree of bone marrow dysfunction. Even with treatment under the current standard of care, a significant subset of patients 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 to inhibit C3 cleavage and activation into C3a and C3b. The active moiety of APL-2 binds to C3 and its fragments with an affinity (as measured by surface plasmon resonance and expressed as Kd) approximately between 150 and 450 pM. APL-2 effectively inhibits all three pathways of complement activation (classical, lectin, and alternative) with a particularly high potency against the alternative pathway. By inhibiting C3, APL-2 blocks the three principal complement activation pathways and their related effects, which may result in both disease control and disease modification.

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 was the first company to have advanced a C3 inhibitor into clinical trials to treat long-lasting disorders with repeated injections. Apellis is currently evaluating its lead product candidates in Phase 1 clinical trials in paroxysmal nocturnal hemoglobinuria (PNH) and chronic obstructive pulmonary disease (COPD), and in a Phase 2 clinical trial in geographic atrophy in age-related macular degeneration (AMD).

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