



Apellis Announces FDA Fast Track Designation for APL-2 in PNH

C3 inhibitor is in development for the treatment of PNH, both in patients not previously treated with eculizumab, and in patients who continue to experience hemolysis and require RBC transfusions despite receiving treatment with eculizumab

LOUISVILLE, Ky., December 20, 2016 – Apellis Pharmaceuticals, Inc., a clinical-stage biopharmaceutical company focused on inhibition of the complement system, announced today that the U.S. Food and Drug Administration (FDA) has granted Fast Track designation to the development program for APL-2, a complement C3 inhibitor, in the treatment of patients with paroxysmal nocturnal hemoglobinuria (PNH), who continue to experience hemolysis and require RBC transfusions despite receiving therapy with eculizumab. PNH is a rare, acquired, potentially life-threatening disease characterized by complement-mediated hemolytic anemia.

Fast track is a program designed to facilitate the development, and expedite the review, of drugs to treat serious conditions and fill an unmet medical need. The purpose is to get important new drugs to the patient earlier. Fast Track designation offers various benefits, including more frequent meetings with FDA to discuss the drug's development plan, eligibility for Accelerated Approval and Priority Review, if relevant criteria are met, and Rolling Review, which allows the company to submit completed sections of its New Drug Application (NDA), rather than waiting until every section of the NDA is completed before the entire application can be reviewed.

Cedric Francois, M.D., Ph.D., chief executive officer of Apellis, said: "This is an important milestone for the APL-2 program. Collaboration with the regulatory agency through programs like Fast Track provides further momentum as we continue to move our lead drug candidate through clinical trials. We believe that APL-2 has the potential to offer an important, and hopefully improved, new treatment option for patients suffering with PNH."

Apellis recently presented Phase Ib interim results for APL-2 in PNH at the International PNH Interest Group (IPIG) Annual Scientific Assembly and presented 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](#)" 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.

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