Apellis Pharmaceuticals Presents Update on Phase 1b PHAROAH Trial of APL-2 in Patients with Paroxysmal Nocturnal Hemoglobinuria (PNH) at Annual Scientific Assembly of the International PNH Interest Group (IPIG)

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LOUISVILLE, Ky. and CAMBRIDGE Mass., Dec. 08, 2017 (GLOBE NEWSWIRE) -- **Apellis Pharmaceuticals, Inc.** (Nasdaq:APLS), a clinical-stage biopharmaceutical company developing a platform of novel therapeutic compounds for the treatment of autoimmune diseases, will present an update on its Phase 1b PHAROAH trial at the 12th Annual Scientific Assembly of the International PNH Interest Group (IPIG) in Atlanta. The PHAROAH trial evaluates treatment with APL-2 in combination with eculizumab in patients with paroxysmal nocturnal hemoglobinuria (PNH) who have low hemoglobin levels despite treatment with eculizumab.

Six patients were enrolled in the PHAROAH trial and two discontinued due to reasons unrelated to therapy. Four patients continue to be treated with daily doses of APL-2 270mg in combination with eculizumab for at least 12 months. Their average baseline hemoglobin level was 8.9 g/dL (normal range 12.0-15.0 g/dL) and the average number of transfusions in the 12 months preceding initiation of treatment was 5.25. Three of four patients were being treated with eculizumab at doses or frequencies in excess of 900mg/bi-weekly, while the remaining patient was being treated with 900mg/bi-weekly. The four patients had an average baseline reticulocyte count of 332 10^3 µL (normal range 39.0 – 123.0 10^3 µL) and average lactate dehydrogenase (LDH) levels of 210 U/L (normal range 110 -209 U/L).

All four patients remain transfusion independent with an average hemoglobin level of 11.6 g/dL (range 10.4 – 12.7 g/dL) at one year of treatment. Average reticulocyte count decreased to 56.02 10^3 µL by month one and has remained steady since that time. Average LDH level was 184.5 U/L at one year of treatment.

The three patients co-treated with high dose eculizumab have had their dose lowered to 900 mg bi-weekly during the course of the study, with no impact on hemoglobin, LDH or reticulocytes.

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 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).

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.

Forward-Looking Statements
Statements in this press release about future expectations, plans and prospects, as well as any other statements regarding matters that are not historical facts, may constitute "forward-looking statements" within the meaning of The Private Securities Litigation Reform Act of 1995. These statements include, but are not limited to, statements relating to the implications of preliminary clinical data. The words "anticipate," "believe," "continue," "could," "estimate," "expect," "intend," "may," "plan," "potential," "predict," "project," "should," "target," "will," "would" and similar expressions are intended to identify forward-looking statements, although not all forward-looking statements contain these identifying words.

Actual results may differ materially from those indicated by such forward-looking statements as a result of various important factors, including: whether preliminary or interim results from a clinical trial will be predictive of the final results of the trial; whether results obtained in preclinical studies and clinical trials will be indicative of results that will be generated in future clinical trials; whether APL-2 will successfully advance through the clinical trial process on a timely basis, or at all, and receive approval from the United States Food and Drug Administration or equivalent foreign regulatory agencies; whether, if Apellis' products receive approval, they will be successfully distributed and marketed; and other factors discussed in the "Risk Factors" section of Apellis' Prospectus filed with the Securities and Exchange Commission on November 9, 2017, and the risks described in other filings that Apellis may make with the Securities and Exchange Commission. Any forward-looking statements contained in this press release speak only as of the date hereof, and Apellis specifically disclaims any obligation to update any forward-looking statement, whether as a result of of new information, future events or otherwise.

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