Release Details

Apellis Announces Top-Line Results from Phase 3 DERBY and OAKS Studies in Geographic Atrophy (GA) and Plans to Submit NDA to FDA in the First Half of 2022

September 9, 2021

- OAKS met the primary endpoint for both monthly and every-other-month treatment with pegcetacoplan, demonstrating a significant reduction in GA lesion growth of 22% (p=0.0003) and 16% (p=0.0052), respectively, compared to pooled sham at 12 months
- DERBY did not meet the primary endpoint of GA lesion growth, showing a reduction of 12% (p=0.0528) and 11% (p=0.0750) with monthly and every-other-month treatment, respectively, compared to pooled sham at 12 months
- In a prespecified analysis of the combined studies, pegcetacoplan decreased GA lesion growth in patients with extrafoveal lesions at baseline by 26% (p<0.0001) and 23% (p=0.0002) with monthly and every-other-month treatment, respectively
- Favorable safety profile in both studies; new-onset exudations occurred in 6.0%, 4.1%, and 2.4% of patients in the combined pegcetacoplan monthly, every-other-month, and sham groups, respectively
- Pegcetacoplan has the potential to become the first treatment for patients with GA
- Conference call scheduled today at 4:30 p.m. ET

WALTHAM, Mass., Sept. 09, 2021 (GLOBE NEWSWIRE) -- Apellis Pharmaceuticals, Inc. (Nasdaq: APLS), a global biopharmaceutical company and leader in complement, today reported top-line results from the Phase 3 DERBY and OAKS studies evaluating intravitreal pegcetacoplan, an investigational targeted C3 therapy, in 1,258 adults with geographic atrophy (GA) secondary to age-related macular degeneration (AMD). GA is a leading cause of blindness that impacts more than five million people globally including one million people in the United States.[1,2] Based on results from the studies, the company plans to submit a New Drug Application (NDA) for pegcetacoplan for GA to the U.S. Food and Drug Administration (FDA) in the first half of 2022.

“These results underscore the potential for pegcetacoplan to become the first treatment for geographic atrophy, a progressive and irreversible disease that robs patients of their vision and for which no treatment exists,” said Jeffrey S. Heier, M.D., principal investigator of the DERBY study and director, retina service and director, retinal research, Ophthalmic Consultants of Boston. “Pegcetacoplan demonstrated a clinically meaningful slowing of disease progression with an even stronger effect in GA patients with extrafoveal lesions.”

Monthly and every-other-month treatment with pegcetacoplan met the primary endpoint in OAKS, significantly reducing GA lesion growth by 22% (p=0.0003) and 16% (p=0.0052), respectively, compared to pooled sham at 12 months. DERBY did not meet the primary endpoint, showing a reduction in GA lesion growth of 12% (p=0.0528) and 11% (p=0.0750) with monthly and every-other-month treatment, respectively, compared to pooled sham at 12 months. In a prespecified analysis of the combined DERBY and OAKS studies, monthly and every-other-month treatment with pegcetacoplan reduced GA lesion growth by 17% (p<0.0001) and 14% (p=0.0012), respectively, compared to pooled sham at 12 months.

In a prespecified analysis of the primary endpoint, pegcetacoplan demonstrated a greater effect in patients with extrafoveal lesions at baseline. Patients with GA typically present first with extrafoveal lesions, which then progress toward the fovea where central vision is impacted. In the combined studies, monthly and every-other-month treatment with pegcetacoplan decreased GA lesion growth by 26% ($p<0.0001$) and 23% ($p=0.0002$), respectively, in patients with extrafoveal lesions compared to pooled sham at 12 months.

"Our mission is to develop transformative therapies for people with complement-driven diseases and now, after decades of challenges in this complex disease, pegcetacoplan is the first investigational therapy to significantly slow the progression of GA in a large Phase 3 study," said Federico Grossi, M.D., Ph.D., chief medical officer, Apellis. "Across our ophthalmology development program, pegcetacoplan has demonstrated an efficacy and safety profile with both monthly and every-other-month dosing that we believe supports treatment for GA patients. We look forward to working with regulatory authorities to bring this medicine to patients in need as quickly as possible."

Pegcetacoplan was well tolerated in both Phase 3 studies. The pooled rate of new-onset exudations was 6.0% of patients in the monthly pegcetacoplan groups, 4.1% in the every-other-month pegcetacoplan groups, and 2.4% in the sham groups. Two cases of confirmed infectious endophthalmitis and one case of suspected infectious endophthalmitis were observed in the study eye out of a total of 6,331 injections (0.047%). Thirteen events of intraocular inflammation were observed in the studies (0.21% per injection). No events of retinal vasculitis or retinal vein occlusion were observed. There were no clinically relevant changes in vision for patients who developed infectious endophthalmitis or intraocular inflammation.

"On the heels of our recent FDA approval in PNH, these pivotal results further reinforce the platform potential of targeting C3 across multiple diseases with few or no treatments," said Cedric Francois, M.D., Ph.D., co-founder and chief executive officer, Apellis. "Apellis is singularly positioned to make a meaningful difference for patients living with a broad range of retinal, rare, and neurological diseases by targeting C3 to comprehensively control complement."

The company continues to analyze results from the studies, and detailed data will be presented at upcoming scientific meetings.

Conference Call and Webcast

Apellis will host a conference call and webcast to discuss the results of the Phase 3 DERBY and OAKS studies today, September 9 at 4:30 p.m. ET. To access the live call by phone, please pre-register for the call here. The conference ID is 6595274. A live audio webcast of the event and accompanying slides may also be accessed through the "Events and Presentations" page of the "Investors and Media" section of the company's website. A replay of the webcast will be available for 30 days following the event.

About DERBY and OAKS

DERBY (621 patients enrolled) and OAKS (637 patients enrolled) are Phase 3, multicenter, randomized, double-masked, sham-controlled studies comparing the efficacy and safety of intravitreal pegcetacoplan with sham injections in patients with geographic atrophy (GA) secondary to age-related macular degeneration (AMD). The primary objective of the studies is to evaluate the efficacy of pegcetacoplan in patients with GA assessed by change in the total area of GA lesions from baseline as measured by fundus autofluorescence (p-value less than .05) at 12 months. Patients in DERBY and OAKS will continue on masked treatment for 24 months.

About Geographic Atrophy (GA)

GA is an advanced form of age-related macular degeneration (AMD), a leading cause of blindness. GA lesions affect the central portion of the retina, known as the macula, which is responsible for central vision. Excessive complement activation drives irreversible lesion growth in GA,[3] and C3 is the only target to precisely control complement overactivation. GA is progressive and irreversible, leading to central visual impairment and permanent loss of vision. Based on published studies, more than five million people have GA globally including approximately one million people in the United States.[1,2] There are currently no approved treatments for GA.

About Pegcetacoplan for Geographic Atrophy (GA)

Pegcetacoplan is an investigational, targeted C3 therapy designed to regulate excessive activation of the complement cascade, part of the body's immune system, which can lead to the onset and progression of many serious diseases. Pegcetacoplan was granted Fast Track designation by the U.S. Food and Drug Administration (FDA) for the treatment of geographic atrophy.

About Apellis

Apellis Pharmaceuticals, Inc. is a global biopharmaceutical company that is committed to leveraging courageous science, creativity, and compassion to deliver life-changing therapies. Leaders in targeted C3 therapies, we aim to develop transformative therapies for a broad range of debilitating diseases that are driven by excessive activation of the complement cascade, including those within hematology, ophthalmology, nephrology, and neurology. For more information, please visit http://apellis.com.

Apellis Forward-Looking Statement

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 Apellis' interpretation of results from the DERBY and OAKS trials, its planned timing of regulatory submissions and the potential advantages and therapeutic potential of intravitreal pegcetacoplan for GA. 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 results obtained in preclinical studies and clinical trials will be indicative of results that will be generated in future clinical trials; whether the results of the DERBY and OAKS trials are sufficient to support regulatory submissions; whether a submission for approval of intravitreal pegcetacoplan for GA on the basis of the DERBY and OAKS trials will be accepted by the FDA or foreign regulatory agencies; whether intravitreal pegcetacoplan will receive approval from the FDA or equivalent foreign regulatory agencies for GA when expected or at all; whether, if intravitreal pegcetacoplan receives approval, it will be successfully distributed and marketed; and other factors discussed in the "Risk Factors" section of Apellis' Quarterly Report on Form 10-Q filed with the Securities and Exchange Commission on August 9, 2021 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 new information, future events or otherwise.

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