Dear members of the PNH community,

We are excited to share important updates with you regarding the clinical development of our investigational therapy, pegcetacoplan, for the treatment of paroxysmal nocturnal hemoglobinuria (PNH). Since our last update, we have partnered with Sobi, a global biopharmaceutical company, to develop pegcetacoplan. Apellis will be responsible for bringing pegcetacoplan to individuals with PNH in the United States (U.S.) while Sobi will work to bring pegcetacoplan to people with PNH outside of the U.S.

The U.S. Food and Drug Administration (FDA) and European Medicines Agency (EMA) accepted our marketing applications for review of pegcetacoplan, a targeted C3 therapy. This means that both agencies will evaluate the results of our clinical studies to decide if pegcetacoplan is safe and effective for people with PNH before approving the treatment for use. The FDA will make an approval decision by May 14, 2021, and we hope to have the EMA's approval decision in 2021 as well.

More detail about this announcement can be found in our press release.

This fall, we announced new data, including long term results, that continues to support the efficacy and safety of pegcetacoplan in PNH.

Positive top-line results at Week 48 from the Phase 3 PEGASUS study demonstrated sustained improvements with pegcetacoplan treatment. At 48 weeks, hemoglobin increases were sustained with a mean improvement from baseline of 2.7 g/dL, and 73% of patients were transfusion free. The safety profile was consistent with previously reported data with the most common adverse events (AEs) reported throughout the study being injection site reactions (36%), hemolysis (24%), and diarrhea (21%).

More information about the long term results at 48 weeks is available in our press release.

Thank you to everyone in the PNH community who has supported the development of pegcetacoplan. We are working urgently to bring pegcetacoplan to patients and plan to keep you updated as we receive new study data or updates from regulatory agencies.

If you have any questions, please reach out to patientadvocacy@apellis.com.

Sincerely,

The Apellis Team