What is PNH?
Paroxysmal Nocturnal Hemoglobinuria (PNH) is a rare, chronic, progressive, and potentially life-threatening blood disorder.

It is characterized by red blood cell (RBC) destruction and extravascular hemolysis (also known as extravascular hemolysis, or EVH) and white blood cell and platelet activation, which may lead to blood cell destruction.

PNH is caused by the acquired genetic mutation (not inherited) that occurs any time after birth and results in the production of abnormal blood cells:
- EVH leads to destruction of these abnormal blood cells.

What role may complement inhibition play in treating PNH with EVH?
Complement inhibition has been shown to play a critical role in treating PNH with EVH by:
- Preventing the destruction of PNH red cells and activation of the complement system
- Plexanizing complement proteins to prevent the destructive, uncontrolled cascade of reactions
- Protecting vulnerable plasma proteins and body tissues

What treatment approach is being studied by Alexion?
In addition to developing the first approved therapy for PNH, Alexion is currently pursuing an investigational therapy, Phase 3 clinical trial for people living with PNH who have clinically significant EVH while on a C5 inhibitor.

Alexion continues to advance the understanding and treatment of PNH and EVH to unlock new opportunities to improve outcomes and continue to pioneer innovations in targeted complement inhibition and advance PNH research.

References: