WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION? Paroxysmal nocturnal hemoglobinuria (PNH) is a rare, chronic, progressive, and potentially life-threatening disorder. PNH is caused by an acquired genetic mutation that leads to blood clot (thrombosis). PNH affects a range of blood cell types, including red blood cells (erythrocytes), white blood cells (leukocytes) and platelets, and is associated with a dysregulated complement system.

Beginning in the 20th century, researchers recognized that anemia of unknown cause and unexplained blood clots could be caused by an inherited disorder. Over the next several decades, researchers identified a genetic mutation in a complement factor. Eventually, researchers were able to map the gene to a specific region of chromosome 1 complement factor gene.

In 1999, researchers identified a new gene, CFI, which encodes factor H, a molecule that controls the complement system. Factor H, which is a protective coating on blood cells, leads to complement factors attacking healthy blood cells, which may cause anemia and blood clots.

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WHAT IS THE COMPLEMENT SYSTEM? The complement system is a part of the body's defense against infection. Proteins enable the immune system to 'attack' and destroy foreign invaders. When proteins are missing, it means that many red blood cells have been destroyed within blood vessels. This has been shown to correlate with complications, such as thrombosis and early mortality.

HOW IS PNH DIAGNOSED AND MONITORED? PNH can be diagnosed from a simple blood test (high sensitivity flow cytometry), which can determine if certain blood cells have defects. Another type of blood test is used to measure ongoing complement activity. Monitoring LDH regularly is a very important part of managing PNH.

THE COMPLEMENT SYSTEM

WHAT ROLE DOES INHIBITION PLAY IN TREATING PNH? In PNH, the complement system is activated, leading to destruction of blood cells and blood clots. Factor D, another complement protein, plays a role in the activation of the complement system. Inhibition (by blocking the C5 protein) is the proven standard of care. Alexion is continuing to investigate additional treatments that could offer new treatment delivery choices.

WHAT TREATMENT APPROACH IS BEING STUDIED BY ALEXION? In addition to developing the first approved therapy for PNH, Alexion is continuing to develop new therapies that could offer additional options for those impacted by this devastating disease. Alexion is also investigating new treatments that could offer new treatment delivery choices.