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Examining Unmet Needs in Paroxysmal Nocturnal Hemoglobinuria (PNH)

Announcer:
You're listening to *Project Oncology* on ReachMD, and this episode is sponsored by Novartis. Here's your host, Dr. Brian McDonough.

Dr. McDonough:
This is *Project Oncology* on ReachMD. I'm Dr. Brian McDonough and today, we'll examine some of the therapeutic challenges associated with paroxysmal nocturnal hemoglobinuria, or PNH for short.

Now for some background, PNH is an acquired disorder of hematopoietic stem cells.[1] It's primarily a disorder of adults. It's rarely seen in children as the median age of onset is about 36 years.[2] PNH is characterized by a triad of hemolysis, thrombosis, and bone marrow failure.[3,4] The current standard of care is C5 inhibition,[4] which has been shown to improve intravascular hemolysis, thrombosis risk, quality of life, and mortality.[3]

And studies have confirmed that up to one-third of patients on standard-of-care therapy may still experience ongoing hemolysis.[5,6] Additionally, patients in increased inflammatory states, whether through acute infection or chronic autoimmune disease, show evidence of breakthrough hemolysis.[7]

Another therapeutic challenge is that some patients may not achieve adequate hemoglobin resolution for symptom management. In fact, about one-third of patients on standard-of-care therapy fail to achieve hemoglobin stabilization and continue to require blood transfusion therapy,[3,5,6] which can lead to iron overload and other complications.[8,9] Given these and other treatment challenges, additional therapeutic advances are needed to help address the unmet needs of patients with PNH. And for ReachMD, I'm Dr. Brian McDonough.

Announcer:
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