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Managing PNH: Key Clinical Considerations

Announcer:
Welcome to *Project Oncology* on ReachMD. This episode is sponsored by Novartis. Here's your host, Dr. Charles Turck.

Dr. Turck:
This is *Project Oncology* on ReachMD. I'm Dr. Charles Turck, and joining me to discuss key clinical considerations in the management of paroxysmal nocturnal hemoglobinuria, or PNH for short, are Drs. Jamie Koprivnikar and Abdulraheem Yacoub. Dr. Koprivnikar specializes in hematology and medical oncology at Regional Cancer Care Associates in Hackensack, New Jersey. Dr. Koprivnikar, thanks for being here today.

Dr. Koprivnikar:
Thank you for having me.

Dr. Turck:
And Dr. Yacoub is an associate professor of hematologic malignancies and cellular therapeutics at the University of Kansas Medical Center. Dr. Yacoub, it's great to have you with us.

Dr. Yacoub:
Thank you very much for involving me in this very important project.

Dr. Turck:
So, Dr. Koprivnikar, would you describe how you work with patients to tailor their individual treatment plans?

Dr. Koprivnikar:
Absolutely. One of the first decisions that we're faced with is whether or not a patient who has been identified to have a PNH clone requires treatment. We know that the leading killer of patients with PNH is thromboembolic complications.[1] And the literature tells us that patients who have evidence of active hemolysis, as well as a PNH-attributable symptom are patients who are most at risk for having a thromboembolic event.[1,2]

So certainly, when I'm determining whether or not a patient with a PNH clone requires therapy, I'm looking for evidence of active hemolysis by checking LDH levels, haptoglobin levels, reticulocyte counts, and bilirubin.[1] I'm also asking a patient about some symptoms that they're experiencing so I can determine if they are having any symptoms, such as chest pain or abdominal pain or hemoglobinuria, that may be attributable to their PNH.[1] Certainly, if I do find that a patient has active hemolysis and a PNH-related symptom, that's definitely an individual who I feel would benefit from complement inhibitor therapy for the treatment of their PNH.

Other indications for the treatment of PNH may be symptomatic or transfusion-dependent anemia.[1] So these are some of the first considerations when I'm approaching an individual patient who has a PNH clone.

Once a patient is determined to have a need for treatment, I sit down and have a conversation with that patient about the available options. Since we don't know which patient will respond best to which individual therapy, what ultimately happens is that I discuss the various available methods of treatment administration and the potential side effects and risks and benefits of each therapy with my patient, and we usually make a shared decision about how to move forward together.

Dr. Turck:
Now coming back to you, Dr. Yacoub, once a patient begins treatment, what strategies do you use to determine if they might benefit from a different therapeutic approach?

Dr. Yacoub:
So we continuously evaluate and reevaluate and revisit the patient's wishes and desires and challenges as they go through therapy. And now that we have more than one approved therapeutic agent with different properties and different administration routes, we can offer patients a change of therapy based on many variables.[3,4]

For example, it is reasonable to consider alternative therapy based on the patient's convenience; if patients would like an I.V. therapy or a subcutaneous therapy; or would like to come to the infusion room; or not come to the infusion room, if they're very reliable and able to handle the infusion at home versus needing the infusion in a healthcare facility.[5]

In addition, we revisit efficacy for patients. So for patients who continue to have anemia or symptoms or transfusional requirements despite therapy, there is always a solid indication to revisit the treatment and success of therapy and whether there is a need for further improvement or alternative interventions at the time.[1,6] So that always needs to be revisited.

These diseases can be associated with complications, such as infections.[1] We continuously provide education and re-education about risk of infections, risk mitigation, vaccinations, and so forth,[6] and primary care prevention. So we go over that with patients continuously, especially as they live with this disease for many years and many phases of their lifetime.

A very key issue is that PNH is a progressive disease and can be associated with other hematological disorders, such as bone marrow failure, aplastic anemia, or MDS.[1,3] And periodically, we have to evaluate patients for disease evolution or secondary hematologic malignancies.[1] So that's something that we keep in mind for patients who are living with this disease.

Dr. Turck:
And before we close, Dr. Koprivnikar, are there any final thoughts you'd like to share with our audience today?

Dr. Koprivnikar:
Absolutely. I think it's a really exciting time for patients with PNH. We've gone from just trying to survive this potentially deadly disease to now having options to optimize therapy for each individual patient with PNH.[4,7] I know we're all quite well aware that PNH is a rare disease.[1] And in medical school, we're taught when we hear hoofbeats, think horses not zebras. Well, let me tell you this, if you say that to a PNH patient, it's not well received. Although this is a rare disease, there certainly are patients out there who are having impairment of their quality of life and are at risk for premature morbidity and mortality because of their diagnosis of PNH.[2] So I promise if you don't think about this diagnosis, you'll never meet a patient with PNH, and you may never make it, so it's important to keep this diagnosis in mind so that we can find and help those individuals out there with PNH.[8]

Dr. Turck:
And how about you, Dr. Yacoub, care to have the final word?

Dr. Yacoub:
We are very fortunate that we have multiple therapeutic options that have revolutionized the treatment for PNH and have changed the outcomes of patients dramatically. Some of those therapies continue to have limitations and challenges.[4,7,9]

Dr. Turck:
That's a great way to round out our discussion on this topic. And I want to thank my guests, Drs. Jamie Koprivnikar and Abdulraheem Yacoub, for joining me to discuss the management of PNH. Dr. Koprivnikar, Dr. Yacoub, it was great having you both on the program.

Dr. Koprivnikar:
Thank you. Great to be here.

Dr. Yacoub:
And thank you very much for providing this service to our patients and our treating physicians.

Announcer:
This episode of *Project Oncology* was sponsored by Novartis. To access this and other episodes in this series, visit ReachMD.com/ProjectOncology, where you can Be Part of the Knowledge. Thanks for listening!

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