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Treating PNH Patients: A Clinical Perspective

Announcer:

You're listening to *Project Oncology* on ReachMD, and this episode is sponsored by Novartis Pharmaceuticals Corporation. Here's your host, Dr. Charles Turck.

Dr. Turck:

Welcome to *Project Oncology* on ReachMD. I'm Dr. Charles Turck, and joining me to share his experiences treating patients with paroxysmal nocturnal hemoglobinuria, or PNH for short, is Dr. Tim Kubal. He is an associate member in the Moffitt Cancer Center Malignant Hematology Department in Tampa, Florida. Dr. Kubal, thanks for being here.

Dr. Kubal:

Thank you for having me.

Dr. Turck:

So from your experience, Dr. Kubal, what are some key assessment questions you ask your patients who might have undiagnosed PNH?

Dr. Kubal:

So back in the day, I remember when I was a resident, we used to be asking about the color of their urine. And I think to some degree that's been supplanted by very sensitive objective tests, and so right now, in my office, I order probably half a dozen PNH flows a month. And the PNH flow is incredibly sensitive for granulocytes and then red cell changes[1] as well. So in my mind right now, it's less about the questions and more about what patient should you be doing objective testing on to figure out if they have PNH. In my mind, that's actually more patients than you would think, and one of the things I've always said about this kind of testing and genetic testing for younger patients is you should get a bunch of negatives. So what I typically tend to do is, I will test in patients with significant hemolysis, patients with pancytopenia, and then sometimes even in patients with unexplained, single lineage cytopenias. I actually have one patient who has dominant thrombocytopenia who has a 99 percent PNH clone in her granulocytes that has resulted in thrombosis, but otherwise, has no anemia so very rare case, and we've had to treat her very differently because we were able to get objective description of what's going on with her. So for me, it's less about questions and more about tests, and we should typically do more.

Dr. Turck:

And once you diagnose your patient with PNH, how do you strategize selecting treatment options?

Dr. Kubal:

This is a good question because this question didn't use to exist. And so what I like to do with a newly-diagnosed patient is, I like to figure out what it is that makes their disease bad. So are there things that they can't do? Do they feel sick? Are they requiring transfusions? And then walk through their treatment choice.[2] I find that over time, some patients get a little bit ground down if they're coming in to receive it in the infusion center. Now because they perceive themselves as being sick, when they're around other patients who are like that, and they feel better, once they start treatment, they don't want to come in and be reminded that they're sick if they feel okay. I really, and this is dynamic, so I have this dialogue, and if one agent isn't working for them, I'll switch over to the other. If one agent isn't working as well, we switch over to the other.

Dr. Turck:

Now if we look beyond therapies, Dr. Kubal, what patient management strategies have you found to be effective in your practice?

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Dr. Kubal:

You've got to see these people, you've got to talk to them, and you've got to listen. So what I think has to happen with these patients is you've got to either see them at their transfusion interval, which can be once a month in some of these patients depending on how they're doing with their hemolysis. But if you're not seeing them then because they're transfusion-independent, you've still got to see them every two or three months, and then what you get is you gather tons of data, so you check this same set of labs every couple of months,[2,3] and then you say, "How are you feeling, how are you doing?" And then you compare those two things to get a gestalt feeling for how they actually are, who they are, and then how is their disease behaving. And then based on that, you make your next set of decisions. So most of patient management for me is about understanding them as a human being, and then being able to graph all that data, and then placing how they feel today in a moment of time that reflects part of their history.

Dr. Turck:

And are there any other strategies you could share about how we could best communicate with our patients throughout their PNH healthcare journey to enhance the care we give?

Dr. Kubal:

So I want to say two things. The first is a lot of these patients will get onto websites and support groups. I think that's a good idea because you're probably—unless you're traveling to a conference specific for PNH patients—you're not going to run into one of these people on the street.[2,3] It's not going to be one of your friends, it's not a commonly discussed process. However, in a support group—online or otherwise—people will say, "I tried this, this is what worked, this is the doctor I work with, this is how I'm feeling. Is there anything different I should do?" So you'll learn things about the PNH that you otherwise might not learn. So the first is I agree with support groups. The second is I think this is mission-critical. We are all programmed as human beings when someone asks us how we're doing, we say great. And for a PNH patient, that is basically never a true answer, but they come in and you say, "Hey, how you feeling?" They say, "I'm doing great. I'm doing great." And then you've got to ask a couple more questions. The first is "Okay, can you do everything that you want to do?" And that's really the truth-teller because they say, "Well, I've had a lot of trouble walking down to the mailbox. I've got a lot of pain that prevents me from traveling," and then the real truth-teller is I always like to include a family member or friend, someone who spends a lot of time with the patient, in the meetings, whether it's on Zoom or otherwise. These patients almost always come with a significant other or a friend, and as soon as they answer the, "Can you do everything that you want to do?" Once they're done talking, I look at the significant other or the family member, and I say, "Is that true?"

Dr. Turck:

For those just tuning in, you're listening to *Project Oncology* on ReachMD. I'm Dr. Charles Turck, and I'm speaking with Dr. Tim Kubal who's discussing management strategies for patients who are undergoing treatment for paroxysmal nocturnal hemoglobinuria, or PNH for short.

Now if we bring all this together, Dr. Kubal, would you share a case that demonstrates the management strategies you discussed earlier?

Dr. Kubal:

Yeah, I would love to. This case is a long history, I'm not going to give a ton of numbers or things like that, but it's just interesting to have followed somebody for maybe 10 years now, over the course of the way PNH has changed. So it was a young woman. She was originally diagnosed in her early 30s,before I was even practicing in this practice. She came to me from another doctor when he departed. She had significant hemolysis, occasional transfusions, ran in the eight to nine range, a lot of fatigue, very dragging fatigue, very foggy in her visits, and had a lot of abdominal pain. So with these patients—and there's a couple of important things here—one is always investigate for causes of problems that have nothing to do with PNH.[4] These patients can have something different than PNH.[4] At the same time, if you look, it might just be PNH if you don't find anything. So in this woman, she comes in, she's got bad belly pain. It's her dominant symptom. We look for clots. Nothing. We end up taking her gall bladder out because that's most consistent with the pain. She feels much better. She's better for about a year. Then her pain gets worse again. At this point, she says, "What's going on?" We image her, nothing is going on. We end up having a discussion about doing an ERCP, and we find that she actually had a retained stone. So interestingly, we took her gall bladder out, got better, then got worse, had a retained stone. So then we take the stone out. She gets better again, then she starts to get worse again. Bad belly pain, same fog, and everything, and I'm thinking, "Well, what could this possibly be?" So each time her pain gets worse, we go back, and we investigate the underlying cause.

Dr. Turck:

Well, with those final comments in mind, I want to thank my guest, Dr. Tim Kubal, for providing his insights on care strategies for our patients with PNH. Dr. Kubal, it was great having you on the program.

Dr. Kubal:

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Thank you for having me.

Announcer:
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