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## Conquering the Burden & Impacts of PNH

### Announcer:

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

### Dr. Turck:

Welcome to *Project Oncology* on ReachMD. I'm Dr. Charles Turck, and joining me to discuss how we can help our patients with the impacts of paroxysmal nocturnal hemoglobinuria, or PNH for short, are Drs. Tim Kubal and Hana Safah. Dr. Kubal is an associate member in the Moffitt Cancer Center Malignant Hematology Department in Tampa, Florida. Dr. Kubal, thanks for being here today.

### Dr. Kubal:

Thank you.

### Dr. Turck:

Dr. Safah is a professor of clinical medicine at the Tulane University School of Medicine in New Orleans. Dr. Safah, it's great to have you with us.

### Dr. Safah:

Thank you.

### Dr. Turck:

Let's dive right in starting with you, Dr. Kubal. Would you give us some background on the burden of PNH?

### Dr. Kubal:

Sure. So it's a lot of different signs and symptoms, and then objective findings, and I think we're going to get through all of those today. There are the obvious ones on the objective side. So the most common thing that we'll see is intravascular hemolysis. So this is going to be chronic and present in almost every patient, although there are rare cases where they may present with say, thrombosis and less hemolysis. So a big thing that's going to be present in the day-to-day in the labs that you see throughout their course is going to be hemolysis. The other thing that you'll see is unusual thrombosis. So there's a substantial proportion of these patients who can present with a weird blood clot. So a splanchnic vein clot or a mesenteric clot in an otherwise young patient. I remember a young patient I had during the first period of time when I was training who had a mesenteric vein clot and had to actually get a small bowel resection and then transplant because of this disease. So there can be tremendous signs and symptoms. The other thing we'll see is pancytopenia. So you're looking at this patient, and you say they might have aplastic anemia, so you're a little nervous about that. You marrow them, they're 15, 20 percent cellular, and then you find the PNH clone. So that's very common.

Interestingly, in my practice, I don't see as many significant aplastic anemia symptoms. So for example, transfusion-dependent thrombocytopenia and significant infections due to low white count. I don't see those as much in my PNH population. The other thing that we see is a lot of times once you establish a diagnosis, you'll find that these patients will have flares. And so a huge part of the burden that you'll see is you've got this patient, they may be on therapy, they go to surgery, they have an infection, say pyelonephritis, they go to a party and have significant alcohol intake, for example, and then they'll flare. And they'll come in, and they'll say that they're really dragging in relation to that. Objectively, you'll see a bump in their LDH, a decline in their hemoglobin, and then they'll feel a little bit different, symptomatically. So these are all the things that we see on the objective side in these patients, both the diagnosis and then throughout their treatment course.

### Dr. Turck:

Now turning to you, Dr. Safah, how might this burden affect a patient's quality of life?

**Dr. Safah:**

When you look at patients with PNH in general, it takes a long time for these patients to receive the diagnosis, and they suffer from symptoms for a long time before that. 40 percent of the patients usually receive their diagnosis within 12 months, and the rest of them, it might take them around five years to get the diagnosis, and they usually see around five, approximately five physicians during that journey because of symptoms that they have to be told that they have PNH. The majority of patients with PNH are diagnosed during their young age. I mean, I would say 30 years to 40 years. This is when the expectation of being most productive in society, but then they're like, right away after getting the diagnosis and knowing what they have, they're thinking, "What's going to happen to my life?" Some of them, they are worried about being labeled with chronic disease. Some of them are going to be like, "What's going to happen?" Like young females, "Am I going to get pregnant?" "Can I be the normal person I want to be?" So these are things that we physicians, whenever that diagnosis is made, we focus on, along with the patient, how are we going to reverse organ failure or prevent organ failure or prevent progression? But quality of life in these patients, when we start taking care of them after diagnosis, is not only affected by the disease, which the fatigue that can be because of the anemia, and it can be because of all the inflammatory ongoing processes as well as the thrombosis. And it is very well known that patients with thrombotic events usually have worse fatigue compared to those who do not have thrombotic events before their diagnosis. But both of those, the fatigue, the thrombosis, and the anemia affect quality of life tremendously, and then comes the treatment. The kind of treatment they receive and complications, whether it's related to treatment or related to the transfusion or whether it's iron overload, which can happen because of the ongoing hemolysis or because of the extravascular hemolysis or because of the blood transfusions. So are they getting IV treatments versus are there other options available for them, like sub-Q? So all of that will affect the quality of life. It's also a burden not only on them but also on their caregivers. Because of the fatigue, they need somebody to take them to the clinic.

**Dr. Turck:**

With that being said, Dr. Kubal, what support tools, resources, or other strategies could we use to help our patients overcome this burden?

**Dr. Kubal:**

Yeah, and that was really well said, Hana. The interesting thing in the pre- and post- diagnosis is a lot of times, as a tertiary provider, you're going to see these patients post-diagnosis, and it's rare that I'll pick one of these up, maybe in the hospital or otherwise. When I do, we go through that journey, and luckily, it's a little easier these days than it used to be with readily available flow. But with regard to tools and strategies, there are choices that you've got to make with the patient. Which is, what are you going to choose from a treatment standpoint? And then what are we going to do to try to treat those symptoms? The symptoms that I've seen that are most notable, the fatigue is profound. The mental foggiest is profound. These people can function, but they're just in a haze all the time. I've gotten a lot of requests for stimulants in these patients over the years. And another one I see a lot of is abdominal pain that's otherwise undiagnosed. So I've had a patient who has had a number of abdominal symptoms, and now that she's on her treatment it has changed over the years, and her pain has started to go away. So what I think is probably most important for any patient is to have a stable medical team that is up to date on what's going on because seeing that same patient and that same team over and over again in a rhythm, say monthly when they're originally diagnosed, every couple of months later, it helps me as the doc to understand them and their body the same way that they live in it.

**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 Drs. Tim Kubal and Hana Safah about the impact of PNH on our patients. So, Dr. Safah, if we switch gears for a moment and take a look at the treatment landscape, what improvements need to be made in order to better care for our patients with PNH?

**Dr. Safah:**

As Dr. Kubal just mentioned, you see that we have done major improvements in the last, I would say decade plus in the treatment of PNH, which we moved a good ways and we have a good number of options. We moved a bit from what we used to do in the past to now. So now we target to lessen the anemia very well, and treatment options are available to lessen the degree of anemia and the need for transfusion. We still have some failures. We have now better options. We know how to deal with the refractor in this or the resistance to the available C5 drugs that target C5, and now we're going to the proximal targets. We have to deal with that like the C3 targeted therapy. And we're having, as I said options that will decrease the treatment burden on the patients, so maybe it's better to give them some freedom from being in the clinic all the time for patients who can do that and use the sub-Q treatment where they can. Do the treatments at home and in their more comfortable environment, I would say and give them the freedom to be more productive. So that option is available now. So that to me is a major improvement, and we're coming up with pills that can help the treatment of PNH, whether as a monotherapy or combination with the available drugs, so that's also going to be a plus in the future. We're now focusing on not only treatment of anemia but decreasing the complications of the disease and the symptoms of the disease. What we have not done

very well yet and we should be targeting the fatigue more. We've learned how to take care of the thrombotic events and decreasing the risk for thrombotic events, but fatigue, whether improving it when we improve anemia, but by itself is still an ongoing problem. So maybe including palliative care in the care of patients, so that we can help them on parts that we hematologists cannot address very well.

**Dr. Turck:**

And before we close, I'd like to hear some key takeaways from each of you. Dr. Kubal, let's start with you.

**Dr. Kubal:**

Yeah, I think the key takeaways for me as somebody who's taken care of a lot of these patients over the years, they used to come into the office, and I would have them on C5 inhibitors, switch them from every two weeks to every eight weeks, and they would have fatigue, and foggiess, and be on disability. And so one of my favorite success stories is a patient who was on 120 milligrams of oxycodone per day for belly pain worked it up a million different ways, multiple surgeries, switched her from C5 to C3, and at this point, a year after switching, she's now on zero. So what I think my key takeaway is, is to be open to having a conversation with that patient about switching if there are symptoms that you don't think you're adequately controlling because those symptoms could absolutely be due to PNH, most notably the fatigue, the mental clarity, or the belly pain, that can all kind of go away.

**Dr. Turck:**

Thanks, Dr. Kubal. And Dr. Safah, you get the final word.

**Dr. Safah:**

Thank you. So again, as Dr. Kubal stated, it's so nice to know that for us and for the patient that different options are available, and again, as I said this allows us to customize the treatment, and also provide treatment that can help those patients who are not doing well on previous options. What I would like to also add is the way we assess our patients when we target the quality of life. So we do have better options that do improve quality of life, but I still think that there is a way to go, and we don't have specific tools to assess the quality of life in PNH patients. We use tools that are available to us from other diseases, from other chronic diseases. So it's time to really come up with tools that are specific for patients with PNH, and I'm sure my patients would agree with me.

**Dr. Turck:**

Well with those key takeaways in mind, I want to thank my guests, Drs. Tim Kubal and Hana Safah, for joining me to explore this important topic in PNH care. Dr. Kubal, Dr. Safah, it was great having you both on the program today.

**Dr. Safah:**

Thank you.

**Dr. Kubal:**

Thank you.

**Announcer:**

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