Treating ET: Working With Your Doctor for Optimal Care

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Dr. Verstovsek:
Yes, for ET, that's very important topic today. We have the privilege of having Andi here with us. ET is closely related to the polycythemia vera. We don't have yet, as of now, any ongoing studies to formally test JAK inhibitors for approval in essential thrombocythemia. But please tell us about your experience living with ET.

Andi Malitz:
Well, I think I was diagnosed—I think it was probably about 12, probably 12, 13 years ago, through just a series of high platelet counts, through normal checkups, through CBCs. And immediately went to see a hematologist, oncologist. Was confirmed.

But went on hydroxyurea (Hydrea) and an 81-milligram aspirin. I really was pretty asymptomatic. I didn't really have any typical symptoms, except for the very high platelet count. And but as the years have gone on, and I was with my former physician, he kept watching my platelets go higher, even on the hydroxyurea, and kept increasing the amount to a very high amount, which was causing severe reactions such as mouth sores and very uncomfortable sores.

Dr. Verstovsek:
I can imagine, yes.

Andi Malitz:
Yeah. So about two years ago, I had the honor and privilege to meet Dr. Stein at an MPN event in the city of Chicago and started to see him. And we actually have been able to reduce the hydroxyurea just by one capsule.

So instead of 2,500 milligrams a day, it's 2,000 a day. The mouth sores have gone away. However, my platelets do tend to—my platelets run high. It just must be my physiology. I don't know. I do have a monthly CBC. I see Dr. Stein every six months. I must say that the mouth sores, which may not seem that big to some people, having that go away two years ago significantly was a big difference in my life, because it went on for about eight months. And to the point where I was seeing

other physicians to find reasons and cures for that. I tend to feel really good. I don't seem to have many issues that seem to be associated with an MPN or ET particularly. And try to stay on top of.

Dr. Verstovsek:
This is where we talk about the quality of life. It's not only about controlling the numbers. It's about looking at the person who has to take medications to control the disease and decrease the risk of thrombosis. And these medications may have side effects. And, unfortunately, you did have one that would perhaps not even recognize this many times. And I have seen this in my own practice, that people do not recognize side effects of the medications, like hydroxyurea can cause these mouth ulcers, can cause skin ulcers, causes skin cancer, even, squamous cell carcinoma can come with the hydroxyurea. And I'm glad that you met Brady. Tell us more about the frontline therapies for ET and management, or your view of a side effects profile, or how to do properly in new situations like this?

Dr. Stein:
So this is a really important question about how we treat ET. In my own personal approach, and I think my approach is similar to many, in ET, we expect longevity. This is a chronic disease, and we have many goals. One is making sure that we can help the patient experience the best quality of life as possible. A major focus in the first 10 to 15 years of ET is to try and prevent vascular complications. And that's really what serves as the main trigger for treatment in ET in 2016. So we look at patients, and we try to predict their risk for a blood clotting complication. And we look at things that are, unfortunately, somewhat generic. Things like age and whether they've had a prior blood clot, that could apply to any hematology patient. If they have any of those factors, if they're older, if they've had a blood clot, we know that they're at higher risk. We're hoping for some more specifics about—or more particular features or individualized features for patients that can help us predict whether or not they're at higher risk or lower risk.

So we're looking at the importance of mutations. And what we're learning in the last couple of years is that the mutational profile plays a role in risk stratification. So patients who have JAK too have a higher risk of blood clotting compared to patients who lack it. We also have to look at cardiovascular risk factors. So the hematologist has to be an internist or work closely with an internist to make sure we're looking at blood pressure, making sure that blood glucose has been looked at through the year, cholesterol profiles, making sure that we're counseling about quitting smoking. These are all really important things. So the first step is to predict risk and decide if a patient really needs therapy or not. It's important that the platelet count alone is not always a trigger for treatment. These other factors predict thrombosis rather than a very high platelet count. If a high platelet count is causing symptoms, we need to address it. But if a high platelet count is seen without any symptoms whatsoever, we've gotta be careful, and we've gotta make sure—and I've seen a lot of patients with ET where sometimes the treatment causes more negative issues with quality of life than the disease itself.

So there's a balancing act in a patient with a chronic disease. And for Andi, mouth ulcers were—there, a severe and uncomfortable symptom was affecting quality of life. So we made a minor adjustment. We were hoping to make a more significant adjustment, but it was a balance. But just enough, just a small dose reduction helped eliminate the symptom that was impacting quality of life.

Dr. Verstovsek:
So now you have experienced that side effect of the therapy, and those adjustments may have perhaps not fully then controlled the platelet numbers. So what's your view on a need for new drugs to be developed for ET?

Andi Malitz:
Interesting listening to what you've been talking about, about getting at it earlier.

Dr. Verstovsek:
Correct.

Andi Malitz:
Kind of pre-emptive. Obviously, I'm too far into it for that type of a situation. I think it's important to continue with the monthly CBCs, that we look at my blood count. I can't speak to what kind of treatments are out there. High platelets, as Dr. Stein said, in my case, doesn't attribute to any further symptoms of the actual disease. However, looking at the whole person and what other types of things are going on in my life, both physically and otherwise, I do want to bring up, though,

you mentioned about the increase in skin cancers. And I think we talked about that. I did forget to mention, though, that in the last two years, interestingly, I have had three bouts—two basal and one squamous, actually, cells removed. Wasn't aware that that could possibly be—it could be coincidental as well, but it was something that we've been talking about and will be following in the future.

Dr. **Verstovsek**:
Yes, there is some good evidence that the incidence of skin cancers may be increased by continuous use of hydroxyurea. And therefore, it may be prudent to develop new therapies. And like, we have engaged all together in development of JAK inhibitors in other myeloproliferative neoplasms.