How would you define treatment success for your patients with myelofibrosis?

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Dr. Kuykendall: How would you define treatment success for your patients with myelofibrosis? I think this is certainly individual, right? I think, in terms of trials, we think about spleen volume reduction, symptom improvement. I think, individually, treatment goals may be different on a patient-by-patient basis. I think it really comes down to what you're trying to accomplish for that patient. I think, in some manner, all these JAK inhibitors are trying to improve quality of life. I always go back to by and large, if patients think that their quality of life is worse after being on a JAK inhibitor, then largely that treatment is not succeeding as much as we'd like it to.

That being said, I think that it's really important to make sure we're establishing what the goals of therapy are with patients going into this. In patients that I'm trying to gear up for, let's say a transplant who have large spleens, the goal really is to try to reduce that spleen size as much as possible before going to transplant. We may be pushing the dose of ruxolitinib to a point that may cause cytopenias in an effort to get that spleen down. Now, in someone else who is not a transplant candidate who has a large spleen, who we're not trying to get to that endpoint, we may be a little bit more nuanced in how we dose the ruxolitinib in order to avoid the need for too many transfusions, but still have adequate control of symptoms.

Largely, these are agents that we're trying to improve quality of life. I think that's what we have to focus on. We are doing MPN 10 scores* in the clinic that looks at symptom scores over time, because I think this is one way to really, you know, objectively try to evaluate the improvement in symptoms over time, because I think sometimes it can be hard to remember how you felt six months before.

Certainly, we're doing physical exams on patients, and at sometimes we're doing ultrasounds or imaging to assess spleen volumes. We're not doing I think what's going on in trials. We're not doing spleen MRIs every 12 or 24 weeks to evaluate spleen response, in that way, it's somewhat more subjective.

What are your thoughts, Prithi?

Dr. Bose: No, absolutely, Andrew. I think symptom benefit how they feel is most important, right there. They have to have a good quality of life. That's one of the main goals of using a JAK inhibitor because of the whole mechanism of suppressing cytokines and things like that. Also, as you said, if you're getting someone ready for transplant, you want that spleen down optimally shrunk for the best transplant outcomes.

No one likes transfusions at the end of the day, so that is important. That is certainly, if that is something that can be ameliorated by switching without compromising on efficacy in other parameters, I think that is highly desirable. That is an important area for some of these second-line agents perhaps in the cytopenic patients.

* MPN Tracker Tools
http://www.mpntracker.com

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