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Considering the Promise of New and Emerging Treatments for Hemophilia: The Need for ITI?

Announcer:

Welcome to CME on ReachMD. This episode is part of our MinuteCE curriculum.

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Dr. Ambrose:

This is CME on ReachMD. I am Dr. Stephanie Ambrose, and I'm joined today by Dr. Jenny McDaniel. Today, we'll be discussing the need for immune tolerance induction, or ITI, in this age of new therapies for hemophilia. Dr. McDaniel, could you tell us your thoughts?

Dr. McDaniel:

I would be happy to. So for patients with hemophilia, the development of inhibitors or antibodies against the factor are still a significant complication, and we'll talk mostly about hemophilia A today.

So immune tolerance induction, or ITI, is the only established method to treat and eliminate inhibitors for patients that develop them. Historically, the treatment options for patients, once they developed inhibitors, were utilizing bypassing agents, which we know help but are still not as effective as the replacement of clotting factor, factor VIII.

The landscape changed for patients with hemophilia A and inhibitors with the development of emicizumab. The emergence though of emicizumab and a overall very good treatment for patients with hemophilia A with inhibitors, has also prompted now some discussion and perhaps some debate in the hemophilia treatment community. So is emicizumab an alternative to ITI? Or is it an addition? Do patients with hemophilia A with inhibitors still need ITI in an attempt to eradicate their inhibitor? Interestingly, there is some real-world data that suggests that patients with hemophilia A with inhibitors who are treated with emicizumab do demonstrate a reduction in their inhibitor titer, and I think this is likely due to decreased exposure to factor VIII that allows that inhibitor titer to decline over time.

Dr. Ambrose, I'll let you pick up a little bit about some of this discussion that we're having.

Dr. Ambrose:

Yeah, of course. So some providers are initiating emicizumab first in patients who are found to have factor VIII inhibitors, thus delaying or potentially avoiding ITI altogether. There is still a very big debate within the hematology community as to whether or not this is the right decision. But the fact remains that there are many families and patients who are reluctant to pursue ITI when their child is very well managed on emicizumab, if they're not having any bleeding issues.

One of the reasons why this is very difficult for many patients and families is because when these inhibitors are found, it truly is often in the pediatric age range when venous access may be somewhat difficult. So to have a discussion with a family whose child is doing quite well on emicizumab, not having any bleeding complications, to have a discussion with them about going to intense factor VIII replacement with ITI and the potential need for central line is a very difficult conversation, and it's understandable as to why many families are choosing to stay with emicizumab and at least delay ITI.

It's important for adults that we really think about what this may look like for them, because there's a lot of thought in the community that

emicizumab may be used in patients who don't necessarily have an inhibitor. And so the question is becoming the more that these patients get exposure to factor VIII, it may take years for them to get enough of that exposure for an inhibitor to surface. And so it may be more of an adult complication, whereas historically, that's not been the case. It's been thought that adults have reduced capacity for ITI due to thymic involution, and that they were often not considered as candidates. However, there is recent data to suggest that ITI can be successful in these adult patients. So it's really important for us in the hematology community to continue these discussions and continue to pursue clinical trials that can help us to answer some of these questions that we have. Dr. McDaniel?

Dr. McDaniel:

Yeah, great points. And the commitment to ITI, as you said, is quite burdensome. Patients may be getting infusions three times a week, and our pediatric patients may need central venous access to support that. And so these are just complex decisions.

We know that as patients get older, they may run into more issues, needing surgical procedures, or needing anticoagulation even potentially. And so we also just want to be thoughtful about ensuring that we do everything we can to provide the best treatment options for each patient.

There are some tools for decision-making that can help guide us. We're all familiar with Play it Safe, which are the guidelines that help us categorize sports based on bleeding risk. There's also My Game Plan, which is a workbook for patients to identify their priorities and make informed decisions regarding physical activities. Dr. Ambrose?

Dr. Ambrose:

Yeah, so this has been a really great discussion.

The decision-making process has to consider the individual patient's risk factors, the potential for thrombotic complications, what that looks like with combination therapy and needing factor VIII replacement, and also the families, preferences and values. All of these are discussions that are taking place on the frontlines right now with patients and families, and we're really trying to navigate the best path forward as we follow along with data as it becomes available.

Make sure to tune in to the rest of the microlearning activities in this series for more information. Thanks for listening.

Announcer:

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