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Evaluating Emerging Treatment Options: Rebalancing Therapies

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 reviewing clinical information about rebalancing therapies. Dr. McDaniel, would you provide us with an overview of the new rebalancing therapies that were recently approved in the United States?

Dr. McDaniel:
I'd love to. So this class of rebalancing therapies enhances hemostasis by reducing or inhibiting the natural anticoagulants in the body. This category of treatment instead removes natural anticoagulants in efforts to restore that hemostatic balance.

This results in a few unique aspects of treatment for this category of therapy. So first, these rebalancing agents can be utilized for patients with hemophilia A or hemophilia B, potentially with or without inhibitors because of this novel mechanism, and their use may even expand beyond that; that's to be determined. Also, these products are delivered subcutaneously, which is an incredible much more accessible treatment option for some of our patients who really struggle with intravenous access, and I think a lot about the pediatric patients that I treat who may need central lines to receive IV infusions, but also patients that have difficult venous access for other reasons.

Within this category of rebalancing therapies, there are currently two broad mechanisms or targets that we'll talk about in a little more detail today. The first category is anti-TFPI, or anti-tissue factor pathway inhibitor, mechanism. The first product is concizumab. And concizumab is an anti-TFPI that is delivered again subcutaneously as a daily injection. The delivery device is a pen, which makes it fairly transportable. And the dosing for concizumab is based on the patient's weight. Concizumab has been approved for patients older than 12 with inhibitors currently. At this time, there is no dose adjustment required for breakthrough bleeds or minor surgeries.

The other anti-TFPI agent that we'll talk about is marstacimab. This device is also delivered subcutaneously. However, it is administered once a week. It also is delivered via a pen device, so makes it very portable for patients. Marstacimab is currently approved for patients older than 12 years old without inhibitors.

Moving on to the next category of rebalancing agents, we'll talk about our anti anti-thrombin mechanism. So fitusiran was recently approved for patients greater than 12 years of age with hemophilia A or hemophilia B, with or without inhibitors. Fitusiran is delivered subcutaneously every 1 to 2 months, and it comes in a fixed-dose pen or a vial option.

And that is a very brief overview of our rebalancing agents. So Dr. Ambrose, will you tell us a little bit more about the safety profile of these agents?

Dr. Ambrose:

Absolutely. So the clinical trials show a fairly favorable safety profile for these class of medications. For fitusiran, it's important to note that it does have a warning for acute and recurrent gallbladder disease. Some additional adverse events that were reported included viral infections, nasopharyngitis, and some bacterial infections.

And then, of course, the one side effect of the safety profile that many have questions about is the rare but serious reported side effect of thromboembolic events. These have been reported with this class of therapy, and due to the potential for these side effects, we'll talk a bit later, but you must be careful in administering additional factor products and things along with these class of medications to help circumvent or at least decrease the risk of these thromboembolic events.

Well, this has been a great bite-sized discussion, so please 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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