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An Overview of Current and Emerging Treatment Options for Hemophilia A & B

Announcer:
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Dr. Ambrose:
So hi everyone. 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 different therapeutic options available for patients with hemophilia. Dr. McDaniel, would you provide us with a brief overview of the current landscape?

Dr. McDaniel:
Yes, I'd love to. Thanks, Dr. Ambrose. So the treatment options for patients with hemophilia have changed a lot in the past decade. It's been a really exciting time to be involved in the treatment of patients with hemophilia. We're all very familiar, I think, with the historical treatment options of clotting factor replacement therapy. And factor replacement has its pros and cons, for sure, for patients with hemophilia.

Clotting factor replacement is delivered via intravenous infusions, which we know can present challenges, especially for some of our really young pediatric patients who may need a central venous line to receive their clotting factor infusions, or our patients with mobility problems, or our elderly patients.

Clotting factor replacement therapy can achieve normal hemostasis with excellent factor levels soon after the infusion. However, we know that those peaks also come along with troughs or low points in between infusions when patients may be at more risk for bleeds, and so patients don't have normal hemostasis all the time with clotting factor replacement therapy.

A newer category of treatment for patients with hemophilia A is the factor mimetics. So these products are bispecific antibodies that serve the same role as factor VIII, but look differently to the body. So these products bring together activated factor IX and factor X, just like factor VIII would. A couple of unique things about factor mimetics is that they can be delivered subcutaneously, which make them much more accessible to the majority of patients. They do have long half-lives and generally provide fairly stable hemostasis, though not entirely normal. And because they look different than factor VIII, factor mimetics can be utilized for patients with or without inhibitors with hemophilia A.

So speaking of inhibitor patients, they are still a challenging population to treat. Patients with hemophilia A or B can develop inhibitors which can neutralize or significantly decrease the efficacy of clotting factor therapy. We have utilized bypassing agents like recombinant VIIa or aPCC for these patients. But even with the bypassing agent therapy, it has been very challenging to prevent bleeds and treat bleeds in inhibitor patients. So that is a population that certainly is ripe for improvements in therapy.

A new and novel category of therapy that we'll talk about in some of our future discussions is the category of rebalancing therapies. So these utilize a novel mechanism. Instead of adding a procoagulant or a clotting factor, these therapies are removing some of the natural

anticoagulants to restore hemostasis in a completely different way. These therapies are also delivered subcutaneously, which do make them accessible, but they do come with some potential risks that we'll dive into later.

The last category of therapeutic options is gene therapy, which is incredibly exciting and a potentially transformative therapy for our patients with hemophilia. Gene therapy involves an infusion or some sort of gene editing to help the patient produce their own factor again, so endogenous production of the missing clotting factor. For gene therapy, patients could receive a single infusion and potentially achieve a significant improvement in their hemostasis, though it may not always be in the completely normal range.

We still have a lot to learn about some of these new therapies, and I'm excited to dive into this with you, Dr. Ambrose, in later discussions. Specifically, I think we have a lot to learn about some of the potential side effects and interactions with some of the novel rebalancing agents. We also want to learn more about our treatment of patients with inhibitors and our women with bleeding are also a population we still need to learn more about how to utilize these treatment options for those patients.

So that's a brief overview of the therapeutic landscape. Dr. Ambrose, do you have anything else to add?

Dr. Ambrose:
Yeah, I think that was fantastic. It's so exciting to see how much the landscape for hemophilia has evolved and is changing.

Well, this has been a great bite-sized discussion. 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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