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The Promise and Future of Gene Therapy

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 current and upcoming gene therapy strategies for hemophilia. Dr. McDaniel, would you provide us with an overview of new gene therapies for hemophilia?

Dr. McDaniel:
Will do. So the goal of gene therapy for patients with hemophilia is to compensate for the dysfunctional gene that they have and in some way insert a new gene that allows them to produce their own factor VIII or factor IX endogenously. There are gene therapy products approved for both hemophilia A factor VIII deficiency or hemophilia B factor IX deficiency.

The currently available and approved gene therapy products use AAV vectors that target the liver, but there are some other mechanisms that are being studied in clinical trials. Currently, gene therapy is limited to adult male patients with moderate to severe hemophilia. And since these AAV vectors are targeting the liver, it is extremely important that patients have the appropriate assessment of their liver health to determine candidacy for these treatments, but then also long term, maintain good liver health, hopefully to ensure durability of these treatments.

Dr. Ambrose, can you tell us a little bit more about the currently approved gene therapy products?

Dr. Ambrose:
Of course. So currently, there are two approved gene therapy products. One is for hemophilia A, which is valoctogene roxaparvovec. And then there's one for hemophilia B, which is etranacogene dezaparvovec. There was another approved gene therapy for hemophilia B, which was fidanacogene, but it was removed from the market in 2025.

It's important to note that with these therapies, they do require long-term monitoring, and patients really need to understand that prior to receiving these therapies. The long-term efficacy and safety for these liver-directed gene therapies are still being investigated.

Dr. McDaniel, what can you tell us about the future of gene therapy?

Dr. McDaniel:
We're excited. We know we're really just at the cusp of gene therapy for patients with hemophilia and many other disorders. So there are a lot of other mechanisms for gene therapy that are being discussed, and in early phase studies.There are different mechanisms for gene therapy, like utilizing CRISPR-Cas9 for gene editing. There are also cellular-based therapies that may target B cells or your hematopoietic stem cells using transduced lentiviral vectors. And then there's also discussion about lipid nanoparticles and platelet-targeted gene therapy. So a lot of exciting things that we all look forward to learning more about as we get more information.

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The next-generation approaches to gene therapy hopefully will help address some of the limitations of our currently available gene therapy products. So some of these limitations include the current inability to re-dose after a patient has received an liver-directed gene therapy product. So there is potential for redosing with some of these other therapeutic options that may emerge.

And then the other thing that we also look forward to is expanding access and patient eligibility for gene therapy products. We need more information on females.

We need more information on younger patients or different populations of patients. So lots to learn, and very exciting thinking about the future of gene therapy for patients with hemophilia.

Well, this has been a great bite-sized discussion. Please make sure to tune in to the rest of our microlearning activities in the series for more information. Thanks for listening.

Announcer:
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