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Released: 04/30/2025
Valid until: 04/30/2026
Time needed to complete: 1h 15m

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Evaluating Novel Factor Replacement Strategies: Extended and Ultra-Half-Life Factors and Factor Mimetics

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

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Dr. McDaniel:
This is CME on ReachMD. I'm Dr. Jenny McDaniel, and I'm joined today by Dr. Stephanie Ambrose. Today, we will be reviewing clinical information about novel factor replacement therapies and factor mimetics. Dr. Ambrose, could you provide us with an overview of factor replacement therapies and then tell us a little bit more about the novel ultra half-life therapy for hemophilia A?

Dr. Ambrose:
Absolutely. So factor replacement therapy is the hallmark of treatment for patients with hemophilia. There are several different categories for factor replacement products. We have some of our standard half-life products that, for hemophilia A or factor VIII, last for about 8 to 12 hours for their half-life. Then we have another class with the extended half-life products. Those last a little bit longer for factor VIII replacement, and it's somewhere in between 14 to 19 hours. And then there is a newer category in the ultra half-life products for factor VIII replacement. Those products have a half-life of about 37 to 41 hours.

Going into that a little bit more, I'd like to talk about some of the safety and efficacy for those newer ultra half-life products. Currently, it is approved for all ages. In clinical trials, it was demonstrated that the ABR, or the annualized bleeding rate, was less than 1 for adolescents and the adult population, which is patients older than 12, as well as for the pediatric population of patients or less than 12 years. the patient-reported outcomes indicated reductions in pain intensity and improvements in physical health. And most bleeds and surgeries in clinical trials were able to be treated and managed with just a single dose of the ultra half-life product. So really is a newer, novel therapy that we haven't been able previously to offer to our patients with hemophilia A.

So our patients with hemophilia B or factor IX deficiency, their replacement products do last a bit longer. So for the standard half-life products for factor IX replacement, these generally have a half-life of about 18 to 30 hours. And the extended half-life products for factor IX replacement is even longer, and lasts somewhere between 82 and 102 hours, so a good bit more than what we get from our factor VIII products.

Dr. McDaniel, now that we've reviewed some of the clinical data for factor replacement therapy, would you provide a similar overview for the factor mimetics?

Dr. McDaniel:
Would be happy to. Thanks for that overview, Dr. Ambrose. So factor mimetics are a category of treatment utilized for patients with hemophilia A only. Factor mimetics are bispecific antibodies that serve the same function or role as factor VIII, but look differently to the body. So these products bring together activated factor IX and factor X to facilitate coagulation in the same way that factor VIII would.

Because these products look different than factor VIII, one of the unique aspects of factor mimetic therapy is they can be utilized for patients with hemophilia A with or without inhibitors.

One of the downsides with factor mimetic therapy, however, is that it does not completely normalize hemostasis, so we are taking patients with moderate or severe hemophilia, or maybe mild hemophilia, and improving their hemostasis into the level probably of a mild hemophilia range. But factor mimetics so far, at least, are not yet completely normalizing hemostasis.

One of the really nice things about factor mimetic therapy is that these products are delivered subcutaneously, so we no longer have to worry as much about intravenous access. And these treatment options are much more accessible to a variety of patients of all ages.

The first factor mimetic therapy that was approved is emicizumab, and it is approved for patients, newborns and older. Of note, there is a black box warning on this product for the risk of thrombotic microangiopathy and thromboembolism, and especially when these products are utilized with bypassing products like aPCC. So there are some mitigation strategies to reduce the risk of TMA or those thrombotic events for patients on emicizumab.

There are a couple of other factor mimetic products in clinical trials. Mim8 is in a phase 3 clinical trial, and then NXT007 is in earlier phase trials as well. With these products, as I mentioned earlier, breakthrough bleeding is still a risk, because we are not yet completely normalizing hemostasis with these therapies.

So 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 and thanks for listening.

Announcer:
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