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What You Need to Know About Pharmacovigilance in Novel Hemophilia A Treatment

Announcer:
Welcome to CME on ReachMD. This activity, entitled "*What You Need to Know About Pharmacovigilance in Novel Hemophilia A Treatment*" is provided by PROVA EDUCATION and is supported by an independent educational grant from Genentech.

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Here is Dr. Shannon Meeks

Dr. Meeks:
Do you know how the pharmacovigilance process works? Are you sure that you're correctly reporting adverse drug events?

This is CME on ReachMD, and I'm Dr. Shannon Meeks.

Dr. Leissinger:
And I'm Dr. Cindy Leissinger.

Dr. Meeks:
Well, Dr. Leissinger, we have a lot to cover today, so let's dive right in. Can you tell us a little bit about the importance of safety surveillance and reporting for novel hemophilia A therapeutics?

Dr. Leissinger:
Sure. You know, drug safety is important for all new drugs, but the safety of new hemophilia treatments is especially important in the hemophilia community. You know, in the early 1980s, over 90% of patients with severe hemophilia A were infected with HIV. They also were exposed to other viral infections that were in the clotting factor concentrates at the time – hepatitis C, hepatitis B – and most of those patients went on to die from those complications, especially AIDS, over the next 10 to 15 years. And so what was a real tragedy in the hemophilia community drove the development of new technologies to make treatments safe.

And so today, we can say unequivocally that we have really very effective and very safe therapies for hemophilia A. We have the recombinant standard half-life factor VIII. We've got the recombinant extended half-life factor VIII. And I think that having these therapies really sets a high bar for new therapies that get approved.

So now the safety of these new therapies becomes paramount. So how do we ensure safety? Well, there's an important system of safety surveillance that starts when the drugs are in the clinical trials. You know, phase 1 and phase 2, phase 3 trials. Before those drugs are ever approved, they're very closely, carefully monitored for any safety signals, any adverse events, you know, any side effects. And those are carefully studied before a drug will be approved by the FDA. But keep in mind that the hemophilia trials often have relatively small numbers of patients. Hemophilia is a rare disorder. And unlike studies in patients who have high blood pressure or new drugs for diabetes, where thousands of patients will be enrolled in the clinical trials before those drugs are approved, in our hemophilia trials, we're doing very good to get 100 patients on a study. A very large hemophilia trial would have 200 patients on the study. So these drugs are being studied in numbers of patients that's not really very large. So it's really important that after these drugs are approved that there needs to be continued surveillance and a way to report new or unexpected adverse events. And this is a

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responsibility that all of us involved in the care of patients have. And we're going to talk more later on today about how to do that.

Dr. Meeks:
Well, thanks, Dr. Leissinger. I really do agree. It's a really exciting time to be in hemophilia, and we've got new drugs that have come to market and a handful of new drugs who are being developed right now and will be in the market in the next few years. So this is a really timely discussion today to look at how we do our part in making sure these drugs are safe.

So let's look a little more closely at the pharmacovigilance or PV process. Dr. Leissinger, can you give us a brief overview of this process and describe the primary aims of adverse drug reaction reporting?

Dr. Leissinger:
Sure. You know, pharmacovigilance is really a system that's been put in place to maintain drug safety, particularly after a drug has already been approved and is on the market. And it relies on healthcare providers and even patients to report adverse drug events or side effects that they experience. In the US, this process is overseen by the FDA, the Food and Drug Administration, which works with the drug manufacturers to maintain drug safety. So the system is really designed to collect, to assess, and to monitor drug reactions. So it's up to us, though, to report significant adverse events after a drug has been approved for use. And so, you know, the question becomes, what should we report? Well, we should report any unexpected adverse drug reaction. Also, we should report any serious adverse drug reaction. And the FDA actually has criteria for what constitutes a serious drug reaction. And that's any reaction that causes death or is life- or limb-threatening, any side effect that causes hospitalizations or extends hospitalizations, a side effect that causes persistent disability, or a side effect that requires treatment in order to avoid any of these things we just mentioned. And certainly, any drug that causes birth defects would also constitute as a serious adverse event. So those all need to be reported.

Now, reporting can go either to the manufacturer or to the FDA. And you don't have to report to both; you go to one or the other. Ultimately, anything reported to the manufacturer of that drug will end up being reported by the manufacturer to the FDA. It's required that they report to the FDA, but the manufacturer will then gather materials and fill out more information and then report to the FDA. To report, it's really fairly simple. The manufacturers all have on their websites easily accessible information with forms and information, instructions on how to fill out a report and submit it. Or if you go to the FDA, their website's very easily accessible on the FDA website that also provides forms and instructions on how to do this. So it's really very straightforward, very simple to do the reporting.

Now, once the FDA has all this information, they'll investigate. They'll look further into this; they'll get more details, particularly if these side effects or a particular side effect has been reported in more than one patient. And ultimately, they can do several things with this information. They can issue a product recall. That would be the most drastic thing, to pull the product off the market. They can issue a boxed warning about when to be careful using the drug in certain situations. They can recommend increased monitoring while the patient's on the drug, or they can recommend to the manufacturer that an altered dose be recommended for patients. And, you know, even like letters that we get to healthcare providers about new drugs. So there are a whole variety of things the FDA can do.

Dr. Meeks:
Thanks, Dr. Leissinger, for giving us that great overview. We've also put together an animation that will help us break this down and understand this pharmacovigilance process a little further. So let's take a look at this illustration of where ADR reports go and what happens next.

[Animation plays.]

For those just tuning in, you're listening to CME on ReachMD, and I'm Dr. Shannon Meeks. And here with me today is Dr. Cindy Leissinger. We're discussing everything you need to know about pharmacovigilance and a novel hemophilia A treatment.

Now that we have a better understanding of the pharmacovigilance process, we need to put it a little bit into clinical perspective and clinical practice. So, Dr. Leissinger, can you give us some information of your best practices for how you've implemented a reporting system within your clinical practice?

Dr. Leissinger:
Sure, I think the first and most important thing is just be alert to potential adverse drug reactions or side effects. And more than that, when starting a patient on a new drug or a new therapy, be sure to talk to them about how the new drugs are working, of course, but also any side effects that they might experience, and encourage them to let you know or your nurses know if they're having a problem. You know, basically just monitor them very carefully with these new drugs.

We already have one novel therapeutic drug that's been available for several years, and that's emicizumab. You know, it was a drug that was first in its class, a bispecific monoclonal antibody, I think, used in hemophilia that, you know, mechanism of action of drug is used in hemophilia first. And I think within the next few years, we're going to see additional novel agents and others that'll be first in

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class, first used in hemophilia before it's used in patients with other disorders. So even more important that we're alert and recognize side effects. You know, therapies like gene therapy, small interfering RNA technology, even more highly modified factor VIII products, and even other monoclonal antibody therapies, I think, will be coming online in the next few years.

And so it really is important for us to stay alert and to pay attention. And remember that we're looking for serious or unexpected side effects. And we need to, you know, in addition to being vigilant and talking to our patients, we also need to communicate to our teams – our nurses and the others who work with our patients – to be on the alert for potential new drug reactions. You know, you need to have a plan. You need to kind of think through how to report – how to find the resources and how to do those reports. It's really, as we said earlier, it's simple; it's straightforward. The instructions are there, but just be willing and ready to do that if needed.

Dr. Meeks:
I agree, Dr. Leissinger. I think that part of it is to have those ears out, both in your nurses, with your patients, including those nurses who may be the home nurses, which may be different from your clinic nurses, just so that everybody's on the same team and looking out for these events so that they can be reported. We all know that when patients come to clinic just once or twice a year, they may not remember all of the details of what happened a few months back. So having them have a good method of recording events as well as contacting the clinic is definitely a key piece of this.

Well, this has been a fantastic conversation, but before we wrap up, Dr. Leissinger, can you share your one take-home message with our audience?

Dr. Leissinger:
Well, I would say as we experience in so many issues of public safety, we hear the expression, if you see something, say something. And I think that is what we need to remember. Be vigilant, remind patients and staff to be vigilant, and if the event is serious or unexpected, report it. And just remember, we play an important role in helping to keep patients safe this way.

Dr. Meeks:
Oh, I really like that quote, Dr. Leissinger, and I agree. I think, you know, my take-home message would also be to be alert, be aware, and know that even though it does add a little bit of additional time to report, our goals here are to really keep this community safe and to understand these drugs. As Dr. Leissinger said, they are tested on patients up front, but 100 to 200 is a big clinical trial for us in hemophilia. So very important to keep that awareness as they come to market as well.

Unfortunately, that's all the time we have for today. So I want to thank our audience for listening in and thank you, Dr. Leissinger, for joining me and sharing all of your valuable insights. It was great speaking with you today.

Dr. Leissinger:
Thank you, Shannon.

Announcer:
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