Hemophilia A — Making It Personal: A Patient Experience

Robert Mocharnuk, MD: Hello and welcome to this CME-certified activity. I am Dr. Robert Mocharnuk, Emeritus Professor of Clinical Medicine. Today we will be speaking with David, a patient who has hemophilia A, about his experience with this disease.

Hi, David, and thank you for joining me today. Could you tell us a little bit about yourself?

David: Thank you for having me. I am 54 years old, and I grew up around the Pittsburgh area. I used to be employed, working with children diagnosed with behavior disorders. I am receiving disability because of hemophilia-related complications. I have 2 daughters that live in Kentucky; both are hemophilia gene carriers. I have 4 grandsons, one of whom is a hemophiliac.

Dr. Mocharnuk: Can you tell us how your hemophilia was diagnosed, David?

David: It would have been in the late 1960s, probably around 1968 or 1969, when my parents were concerned because I was coming up with a lot of bruises and contusions and things like that. So they took me to my home doctor at the time, and he told us to go up to Latrobe. And we saw a hematologist up there, and they diagnosed me with a mild type of hemophilia A, factor level about 12% to 14% range. After that, with testing we discovered that my mother was the carrier. And although my grandfather was not tested, that he was possibly a hemophiliac because he was discharged from the Army for having his teeth pulled in World War II.

Dr. Mocharnuk: Do you recall how you felt once hemophilia was diagnosed?

David: Because I wasn't a frequent bleeder, it really didn't bother me all that much. I tried to live life as normally as possible. I stayed away from contact sports, and a lot of my friends would look out for me. I did eventually go into competitive swimming, and that was considered safe for a hemophiliac.

Dr. Mocharnuk: So how often did you require treatment for your hemophilia at that time?

David: Because I had mild disease, it did not require a lot of regular treatments unless I injured myself like getting hit, twisting my knees/ankles, or falling off bicycle. When I did get treatment, it was approximately once every 3 to 4 years, and I was given cryoprecipitate at the time. I recall a particularly bad injury in 1972 when I bit my tongue, requiring bags and bags of cryoprecipitate to stop the bleeding. In 1986, I was involved in a car accident in West Virginia, which broke my ankle and I was treated with an agent over the course of several days until my condition resolved. Unfortunately, I can't recall the name of that particular agent at the time.

And in 1992, I had a string of bad injuries while living in Kentucky—a fractured wrist playing softball, abdominal muscle bleed from a pulled muscle, and a knee strain all at the same time. And I was treated with recombinant antihemophilic factor. This was given to me by a nurse that came to my house 4 times daily for an entire month. And a week or so after the treatments started, I woke up to find that my wrist was black and blue all the way up to my arm. I thought this was because I had probably slept on my arm, but it turns out that I had developed an inhibitor to factor VIII, for which I was prescribed high-dose steroids. Because of the steroids, the inhibitor eventually went away, but every time I was given factor replacement therapy after that, the inhibitor returned, requiring more courses of steroids.

Dr. Mocharnuk: So, David, were you part of the initial decision-making process to switch from your current therapy in 1986 or in 1992 when you developed inhibitors?

David: I wasn't asked about my preference for treatment back then; I don't think there was much of a call for it. In fact, it wasn't until I had a bicycle accident, after moving back to Pennsylvania, that my new physician at the University of Pittsburgh asked me what I thought about using recombinant factor VIIa. Since then, I've always been involved in making decisions about how to treat my hemophilia; and from 2013 until just recently, I alternated between a recombinant factor VIIa and an anti-inhibitor coagulant complex when I needed treatment. I had a really hard time with this regimen. It took an hour to mix the anti-inhibitor coagulant complex and another hour to infuse it, and I had to give myself the infusion every 6 hours.

By the time I got the anti-inhibitor coagulant complex infused, it was time to start my recombinant factor VIIa treatments to combat the inhibitors activated by the anti-inhibitor coagulant complex. From 2007 until just recently, I was afraid to be physically active because my injury would result in treatment and would make my inhibitor flare up. I put

on a lot of weight during this time, and I'm around 6'2, and currently I weigh about 280 pounds.

Dr. Mocharnuk: David, during this time, did you ever approach your care team to inform them about the challenges you were facing with this regimen and how it was affecting your quality of life?

David: The nurses knew about the hardship—mixing the medicine and the fact it had to be given every 3, 6, 8 hours and so on. This was really inconvenient, but it is what you had to do to get better, so you did it. I stuck to the schedule and did my best to move as little as possible to minimize further bleeding and trauma.

Dr. Mocharnuk: So, David, our healthcare system is currently transitioning to what we call a more value-based delivery model, which really relies on patient's engagement and actively seeking patient input on treatment planning. We call this process shared decision making where the healthcare team works to elicit patient values, preferences, and goals for therapy, while weighing the risks and benefits of possible treatment options. This helps patients and healthcare professionals collectively arrive at a treatment that is best suited for the patient based on their preferences and clinical needs.

As you know, the FDA recently approved a new treatment, emicizumab-kxwh, as prophylactic treatment to prevent or reduce the frequency of bleeding episodes in infants, children, and adults with hemophilia A with or without factor VIII inhibitors.

So how did you make the decision to switch to emicizumab? And did you find out about it on your own, or was it recommended to you by your doctor?

David: A nurse at the hemophiliac center told me about it. And after we discussed the risks and benefits, I made the decision to switch, which is the best thing I ever did.

Dr. Mocharnuk: As you spoke with the nurse at the hemophilia center about emicizumab, how did she help you weigh your options? Did you feel like you had a voice during this discussion, and that your preferences were an important factor in collectively making the decision to switch?

David: I was informed that the new medicine was in a testing phase, by the nurses, well in advance of FDA approval. When the drug was approved, I sat down with the nurse, and we discussed the new medicine. We discussed how it worked on other patients, and how it could potentially help me. We also discussed the possibilities of negative outcomes if you didn't follow the protocols. And after weighing the pros and cons, I made the decision to try it. I was a little scared at first not being able to self-treat whenever a problem arose, but I trusted my healthcare team.

Dr. Mocharnuk: How has emicizumab impacted the quality of your life?

David: I've been losing weight since I started the treatment of emicizumab approximately 3 months ago, and I've tapered my prednisone dose quite a lot. I'm walking more and plan to start swimming again. The good news is that I have not even had one bruise since starting the emicizumab. The other great thing about this product is it's given as subcutaneous injection only 1 time per week.

Dr. Mocharnuk: I'm so glad that you have found something that works well for you and is convenient to administer.

But if you wouldn't mind, I'd like to ask you something rather personal based upon my knowledge, as a hematologist, about what happened to many patients with hemophilia during the 1970s and 1980s. I assume, given the timeframe of your initial diagnosis, that you have known people who contracted HIV and/or hepatitis from use of human factor concentrates.

David: I lost a lot of friends who got AIDS from their hemophilia treatments. I guess I was lucky because I wasn't treated very often with factor replacement during the 1970s and 1980s when I was growing up. However, I was diagnosed with hepatitis C sometime in the 1990s; it would have been around 1992 or 1993. Fortunately, I never suffered from complications of liver disease and just recently completed a course of a newly approved FDA drug, which has essentially eradicated the virus.

Dr. Mocharnuk: People with hemophilia often have major problems with their joints from chronic bleeds. Over the years, how have you fared with joint damage and joint pain?

David: Well, I've lost my left hip and my left knee. Then they've both been replaced, and planning to undergo right hip surgery here January 2nd of next year. The joint damage is probably caused by bleeding and not steroids because I haven't taken steroids for long stretches of time. I have a really high pain tolerance and have lived with joint pain for many years. I have previously used prescription narcotics for pain control but always have gotten off these drugs as soon as I could.

Dr. Mocharnuk: So is there any advice you would like to share with fellow patients or healthcare professionals who treat patients with hemophilia?

David: For others with hemophilia, I think you can live a good and active life. If you play contact sports, which I wouldn't recommend, you'll pay for that later for all the bumps and bruises you get, trust me. Take care of yourself when you're younger, talk with your doctor and your healthcare provider and how treatments affect you. Don't be afraid to challenge their recommendations, and always keep yourself informed about new products and treatments.

For physicians, I would say trust your patients. They know better than anyone else how they're feeling and what they're going through. They can detect early signs of bleeding better than you can, and they know their bodies better than you do. Listen to them and let them be part of the decision-making process. It empowers them to take charge of their own health, and it gives them some skin in the game.

Dr. Mocharnuk: Thank you, David, for sharing your story with us. You've helped a lot of people today, and I've learned a lot from you.

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