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Decoding Gene Therapy for Hemophilia B: Expert Insights on Common Myths

ReachMD Announcer:
Welcome to ReachMD.

This medical industry feature, titled "*Decoding Gene Therapy for Hemophilia B: Expert Insights on Common Myt*hs," is sponsored by CSL Behring.

Here's your host, Dr. Charles Turck.

Dr. Turck:
This is ReachMD, and I'm Dr. Charles Turck.

In 2022, HEMGENIX became the first ever gene therapy to be approved for hemophilia B. The gene transfer therapy offers patients sustained, elevated Factor IX levels for years and the potential to eliminate the need for routine Factor IX prophylaxis.[1] Today, we'll discuss some common misperceptions around HEMGENIX so providers can know the facts when discussing this therapy with their patients.

Dr. Turck:
Joining me in our discussion today are Dr. Guy Young and Kim Phelan. Dr. Young is a Professor of Pediatrics at University of Southern California's Keck School of Medicine as well as the Director of the Hemostasis and Thrombosis Program at Children's Hospital Los Angeles.

Dr. Young, welcome to the program.

Dr. Young:
Thank you. Thanks for having me.

Dr. Turck:
Also with us is Kim Phelan, who is the CEO of the nonprofit Coalition for Hemophilia B based in New York City. The Coalition's mission includes education, community-building, and advocacy for patients with hemophilia B and their families. Kim, thank you for joining us today.

Kim:
Thank you. It's great to be here.

Dr. Turck:
Now before we get into our discussion, let's take a moment to hear the Indication for HEMGENIX, etranacogene dezaparvovec-drlb.

ReachMD Announcer:
INDICATION
HEMGENIX®, etranacogene dezaparvovec-drlb, is an adeno-associated virus vector-based gene therapy indicated for the treatment of adults with Hemophilia B (congenital Factor IX deficiency) who:

- Currently use Factor IX prophylaxis therapy, or
- Have current or historical life-threatening hemorrhage, or

- Have repeated, serious spontaneous bleeding episodes.

HEMGENIX is for single use intravenous infusion only.

Stay tuned for the complete Important Safety Information for HEMGENIX throughout this episode.

Dr. Turck:
With the Indication in mind, let's begin our discussion by identifying appropriate patients for HEMGENIX. Dr. Young, can you talk about how you would identify patients for HEMGENIX and what the eligibility process looks like?

Dr. Young:
Sure. To be eligible for HEMGENIX, a patient must be an adult with hemophilia B and either currently using Factor IX prophylaxis, have a history of life-threatening hemorrhage, or experience repeated serious spontaneous bleeding episodes.

Once a patient meets one of these criteria, they can move forward with the eligibility screening. This is a process which includes testing for Factor IX inhibitor and assessing liver health with both laboratory testing including liver function tests, as well as a liver ultrasound, and liver elastography.[2]

Providers can also choose to do an AAV5 neutralizing antibody test provided free of charge by the company, but keep in mind that this isn't an exclusion criteria for HEMGENIX.[2] Shared-decision making is encouraged when deciding to move forward with therapy.[3,4] And providers should also discuss post-monitoring requirements, which include testing for ALT, AST, and factor IX levels weekly for three months after administration, and these can be performed at a local lab.

Dr. Turck:
Now it seems that some providers believe that patients who aren't compliant with Factor IX prophylaxis aren't good candidates for HEMGENIX. But Dr. Young, what are your thoughts here and would you consider HEMGENIX for these patients?

Dr. Young:
For patients who struggle with compliance, we know there can be many factors at play. Often, it's because they have to manage weekly IV infusions as a lifelong commitment. Some patients may have difficulty with finding veins, others may miss doses due to travel, and these challenges add up and make it tough to stay on track.

But with HEMGENIX, as we mentioned, the majority of patients—around 94 percent—didn't have to resume prophylaxis after the single infusion.[1,6] So, for those who struggle with compliance, this could actually be an ideal option.

Now, there may be concerns about whether these patients will follow through with the post-infusion weekly lab testing. But it's important to point out that missing weekly IV infusions, which require finding a vein and can take 20 to 30 minutes, is very different from going to a lab for a quick blood test once a week.

So, with the right guidance and making lab access as convenient as possible, I would argue that patients who have trouble adhering to their prophylaxis may actually be ideal candidates for HEMGENIX.

Dr. Turck:
Kim, from your perspective working with patients and their families, how do Dr. Young's insights about HEMGENIX resonate with what you're hearing from the patient community, especially those who struggle with regular prophylactic infusions?

Kim:
Oh, I completely agree with Dr. Young. Patients who struggle with compliance are often the ones who could benefit the most from gene therapy because it offers them an end goal. Their hematologists know them well and can recognize when this might be the right option. This is likely the solution they've been waiting for, and now could be the perfect time to try it.

It's an opportunity for them to take control of their treatment with long-term solution in sight. And I think with the right patients, it could really be a win-win.

Dr. Turck:
Now let's turn to the affordability of HEMGENIX, which may concern some patients or providers. Kim, how can patients manage the costs associated with this treatment?

Kim:
Well, patients learning about this treatment may have questions about managing the costs. I would encourage patients to reach out to their social worker at their hemophilia treatment center for their options. And patients may be able to take advantage of various

programs that can actually help reduce the cost significantly.

Dr. Turck:
And Dr. Young, how do you advise your patients on the costs associated with this treatment?

Dr. Young:
Well, it's important to have a detailed discussion with patients about their insurance coverage and potential out-of-pocket costs. For many patients there's no co-pay or there can be a very little cost due to various support programs and insurance coverage options.

For example, HEMGENIX Connect can assist patients with benefit verification, financial assistance, and travel support. Providers can also work closely with pharmacists and the HEMGENIX Connect team to ensure a smooth process for the administrative details.

Dr. Turck:
For those just tuning in, you're listening to ReachMD. I'm Dr. Charles Turck, and today I'm speaking with Dr. Guy Young and Kim Phelan about common misperceptions around HEMGENIX for hemophilia B. Before we continue, let's take this moment to hear some more Important Safety Information on HEMGENIX.

ReachMD Announcer:
IMPORTANT SAFETY INFORMATION
Warning and Precautions

Infusion Reactions
Infusion reactions, including hypersensitivity reactions and anaphylaxis, may occur. Monitor during administration and for at least 3 hours after end of infusion. If symptoms occur, slow or interrupt administration. Re-start administration at a slower infusion once resolved.

Hepatotoxicity/Hepatocellular Carcinoma
Post-dose, monitor for elevated transaminase levels. Consider corticosteroid treatment should elevations occur. The integration of liver-targeting AAV vector DNA into the genome may carry the theoretical risk of hepatocellular carcinoma development. For patients with preexisting risk factors for hepatocellular carcinogenicity, perform regular (eg, annual) abdominal ultrasound and alpha-fetoprotein testing following administration.

Immune-mediated neutralization of the AAV5 vector capsid
Preexisting neutralizing anti-AAV antibodies may impede transgene expression at desired levels.

Monitoring Laboratory Tests
In addition to monitoring liver function, monitor for Factor IX activity and Factor IX inhibitors after administration.

Please see full prescribing information for HEMGENIX.

Dr. Turck:
Now that we've heard that Important Safety Information, Kim, how would you advise patients who may be hesitant about gene therapy and may be holding off on treatment with HEMGENIX?

Kim:
Well, for people who have questions about gene therapy, I encourage them to learn more about this technology as it's been studied for decades.[7] I also recommend people with hemophilia B who are interested to take a look at the videos where patients share their own experience with gene therapy. It's always very well received, and most patients want to hear from people who have already gone through the treatment—that's what gives them the confidence to move forward. You can find educational resources at The Coalition for Hemophilia B website, as well as the Education Hub. Do your homework, do your research, and see the lived experiences of others.

Dr. Turck:
Dr. Young, some providers might choose to adopt a 'wait and see' approach, thinking there might be better options in the future. What are your thoughts on this?

Dr. Young:
It's understandable that people might be cautious when it comes to a new platform of treatment such as gene therapy. But with HEMGENIX, we already have data extended out three years showing that it's highly effective. And as we discussed earlier, it's a well-tolerated treatment and provides significant bleed protection, with the majority eliminating the need for Factor prophylaxis.[1,2,6,8]

So for providers who see patients who could be eligible for HEMGENIX, a 'wait and see' approach could mean waiting for a very long

time but we know that this therapy could improve lives right now.[1,6]

Dr. Turck:
Thanks Dr. Young. Kim, what advice would you give to providers when it comes to discussing gene therapy and HEMGENIX with their patients?

Kim:
Well, for hematologists, I'd really encourage them to learn more about gene therapy, because your patients will have questions. And uncertainty from the doctor can really affect shared decision-making process. If the patient is ready and the doctor is hesitant, it's not going to lead to the best outcome. After all, the more doctors learn, the less intimidating the idea of gene therapy becomes, and they'll feel more confident in discussing it with their patients.

Dr. Turck:
And Dr. Young, how can patients and providers educate themselves to successfully integrate HEMGENIX into treatment plans?

Dr. Young:
One way to start is by connecting with hematologists at a trained center, which you could locate on the website, HEMGENIX.com. These clinicians are experienced in this area and can provide valuable insights and guidance. I'd also encourage my colleagues to take advantage of all the educational materials available to them.

We also encourage patients to be well-informed so that they understand the language, the process, and all aspects of gene therapy.

Dr. Turck:
Thank you for sharing. Now we're just about out of time for today, but before we close could you share any patient stories that highlight the impact of HEMGENIX on their lives? Kim, let's start with you.

Kim:
Well, I've had some incredible conversations with these patients. They're genuinely very happy. I often have to say, 'Hey guys, it's time to come back.' I'll remind them to share their experience with others in the community, to help educate others who are considering this path, because their stories are so powerful. It's so exciting to see how their quality of life has improved so much that they're able to dream bigger and do more. That's always been the goal, since I started 35 years ago.

Dr. Turck:
And Dr. Young, you get the final word.

Dr. Young:
You know All the patients I've infused have done really well, but one in particular stands out. He's now a little over 5 years post infusion, and at his 5-year visit, we had a really meaningful conversation. He's 43 years old. He's very active, and he said something that really stuck with me. He told me, 'Dr. Young, for the last 30 years, since I was a teenager, I've had to make so many decisions about my hemophilia. Should I use this factor? Should I be on prophylaxis? Should it be weekly? So many decisions. But by far the best decision I made was to participate in the Hemgenix clinical trial. My life is completely different now. It's been 5 years, and I haven't infused even once. I go to the gym all the time, and I've even increased my activity without any bleeds.' Now, individual experiences may vary. In this case, I've known this patient for 17 years and hearing him reflect on how much his life has transformed thanks to HEMGENIX was really, incredibly rewarding.

Dr. Turck:
Thank you both for sharing your insights and experiences with HEMGENIX. And with those final thoughts in mind, I want to thank my guests, Dr. Guy Young and Kim Phelan, for sharing their experience and expertise with us to help address misperceptions surrounding HEMGENIX gene therapy for hemophilia B.

Dr. Young, Kim, it was great speaking with you both today.

Dr. Young:
Thank you for having me.

Kim:
Thank you for having me.

Dr. Turck:
I'm Dr. Charles Turck. Please stay tuned to hear some Important Safety Information.

ReachMD Announcer:

Adverse Reactions

The most common adverse reactions (incidence ≥5%) were elevated ALT, headache, blood creatine kinase elevations, flu-like symptoms, infusion-related reactions, fatigue, nausea, malaise, and elevated AST.

Contraindications: None.

Please see full prescribing information for HEMGENIX.

To report SUSPECTED ADVERSE REACTIONS, contact the CSL Behring Pharmacovigilance Department at 1-866-915-6958 or FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

This medical industry feature was sponsored by CSL Behring. If you missed any part of this discussion, visit Industry Features on ReachMD.com, where you can Be Part of the Knowledge.

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