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Gene Therapy for Hemophilia B: Real-World Perspectives from an HCP and Patient

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This medical industry feature, titled "Gene Therapy for Hemophilia B: Real-World Perspectives from an HCP and Patient," 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, and it offers the potential for sustained, elevated Factor IX levels for years and can eliminate the need for Factor IX prophylaxis.[1,2] So given those potential impacts, today I'll be speaking with hematologist Dr. Doris Quon and Greg, a hemophilia B patient who received HEMGENIX, to gain insights from their clinical and personal experiences with this treatment option.

Dr. Quon is the Medical Director of the Hemophilia Treatment Center at Luskin Orthopaedic Institute for Children in Los Angeles. Dr. Quon, welcome to the program.

Dr. Quon:
It's a pleasure to be here.

Dr. Turck:
Also with us is Greg, who's been living with severe hemophilia B for 72 years and received HEMGENIX in 2024. Greg, thanks so much for joining us today.

Greg:
Thanks for having me.

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:
And with that Indication information in mind, let's begin with you, Dr. Quon. How has HEMGENIX changed your approach to managing patients with hemophilia B?

Dr. Quon:
Traditionally, when discussing treatment goals with my patients, we were very limited in what we could offer with routine infusions of factor IX, which can be quite burdensome.[3–5] But with the data with HEMGENIX showing sustained factor IX levels over three years were very promising because it can significantly reduce this burden for patients.[2] So HEMGENIX opens up new possibilities for managing their condition more effectively and conveniently.

Dr. Turck:
That's a valuable insight, Dr. Quon. Now I'd like to turn it over to you, Greg, so we can hear your perspective. Tell us a little bit about what led you to consider HEMGENIX as a treatment option and why you decided to move forward with it.

Greg:
Well, I've been living with severe hemophilia B my whole life, and I'm 72 years old now. When I was growing up, there were very few treatments available, so all sorts of minor injuries, like losing my baby teeth or playing with friends, can severely impact my health. Once the prophylaxis became available, I gave myself injections—thousands and thousands of them. But there were issues other than spontaneous bleeding to worry about with the peaks and the troughs, like micro bleeds and inflammation in the joints, which caused a lot of pain, which affected my mobility.

So ever since I first heard about clinical trials for the gene therapy, I've followed them closely. I learned a good deal about the technology and stayed current on the treatment's progress to FDA approval. When my doctor reached out to me about HEMGENIX to discuss whether I could qualify, I was hopeful. I'd gotten so tired of doctors telling me that there's really nothing they could do and I wanted to live in a world where a single treatment could help lessen the burden of injections that I've needed every three to four days most of my life. So the idea that a single one- to two-hour infusion could offer that kind of relief was really appealing to me.

Dr. Turck:
Thanks for being open and sharing your experience with us, Greg. And if we turn to you now, Dr. Quon, how do you typically introduce HEMGENIX to your patients?

Dr. Quon:
At our clinic, we ensure that all patients in our hemophilia center are informed about all their treatment options, including HEMGENIX. The follow-up that's required post-infusion is important, so if a patient is interested and wants more information, we'll then dive deeper into what the treatment involves.

Shared decision-making is key, so I ask my patients about their treatment needs, whether they're being met, and their quality-of-life goals. If patients want to learn more, I'll educate them thoroughly on the potential risks, benefits, and commitments to successfully complete the treatment. We'll share printed information we have in the clinic, as well as refer patients to websites and educational resources available online. Then, I encourage the patients to think about it, come back with questions, and only proceed if they're fully on board with post-infusion commitments. It's really important to ensure that the patient are making an informed choice that aligns with their personal and clinical goals.

Dr. Turck:
Those are great points, and as a quick follow-up to that, Dr. Quon, what makes a patient a good candidate for HEMGENIX?

Dr. Quon:
I think the key factor in our hemophilia B patients who we treat with HEMGENIX is that they're very motivated. They're on Factor IX prophylaxis therapy but continue to have unmet needs, such as experiencing ongoing or breakthrough bleeds or life-threatening hemorrhages.

A few patients may also have an aversion to needles, many are frustrated with frequent infusions, or are in significant pain with arthropathy or joint damage. Additionally, those who have been non-compliant with previous treatments may actually be the ideal candidate for HEMGENIX if they're willing to come for their post-infusion follow-up labs. I'll ask my patients the reasons why they're not compliant, because they may be willing and even eager to do blood draws for the limited 12 weeks post-infusion if that could mean managing their hemophilia B without Factor IX infusions for prophylaxis or breakthrough bleeds.[2]

Dr. Turck:
Now staying with you, Dr. Quon, for just another moment, could you share your experience with the eligibility screening process for

HEMGENIX and what infusion day is like for you and your team?

Dr. Quon:
So once we, the patient and our team, makes a decision to move forward with HEMGENIX, we set up the required testing for liver enzymes, ultrasound, elastography, as well as the Factor IX inhibitor titer testing. In our clinic, we also send for the AAV5 neutralizing antibody testing. The presence of these antibodies doesn't exclude the patients from HEMGENIX therapy, and although this test isn't required, we highly encourage our patients to perform the lab to help us gather more information about this data.[1]

Additionally, we'll educate the patients thoroughly on the infusion process, potential infusion-related reactions, and post monitoring for hepatotoxicity and Factor IX inhibitors. And during the eligibility testing, we'll handle the coverage or insurance process with help from HEMGENIX Connect℠. They can provide support with benefits investigation and travel assistance, which can help streamline the process for our patients.

Now, on the day before the infusion, we'll do a final check-in with our patients to make sure they're doing ok and stable, confirm they're coming in, and check whether they're staying nearby. We'll also prepare our staff on this day by making sure everyone is trained and ready for their role on infusion day. This includes reviewing the protocols from beginning to end, identifying who's doing intake, who's preparing the infusion, starting the infusion, and monitoring for infusion reactions. It's also important to review the protocol for next steps if an infusion reaction occurs.

Then, on the day of infusion, the CSL Behring team is available for support as needed. We do the infusion slowly to minimize any infusion-related reactions. And after the infusion is completed, the patient stays for at least three hours to monitor them for infusion reactions before heading out.

Dr. Turck:
Now with that in mind, let's come back to you now, Greg. Would you tell us what your infusion day was like? How did you feel, and what was your experience going through the process?

Greg:
On infusion day, I felt a surge of excitement in the clinic, largely because of the enthusiasm of the entire treatment team. We were all just astonished by the opportunity to use gene therapy in treating a disease that's been around for centuries.

And for me, specifically, it was an incredible moment: I realized I was fortunate to have lived long enough to see medical science advance to this degree. I did wish I were younger and I'd have more time to experience life without some of the limitations of hemophilia, but overall, I felt deeply grateful to see this day finally arrive.

Dr. Turck:
For those just tuning in, you're listening to ReachMD. I'm Dr. Charles Turck, and today I'm speaking with Dr. Doris Quon and Greg, a patient who was treated with HEMGENIX, about their experiences with this gene therapy for hemophilia B.

Before we continue, let's take a moment to hear some more Important Safety Information for 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 at Hemgenix.com/hcp

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

Dr. Turck:
And we're back after that Important Safety Information.

So far, you've taken us up to the day of treatment. So, Dr. Quon, how have your patients responded to treatment, and have you received any feedback on their experience?

Dr. Quon:
From our patient's perspective, it was life-changing. He's been free from the burden of prophylaxis and weekly infusions. HEMGENIX provided him an improved and stable factor IX level, which has improved his symptoms and potential for preventing bleeding. Traveling was an important goal for our patient, and after his successful treatment, he is doing well and feeling more confident to travel.

Dr. Turck:
That's a helpful perspective, Dr. Quon. And Greg, how are you doing now? What's your life like after receiving HEMGENIX?

Greg:
Well since the infusion, I feel better than I've felt in a long time.

But really, the major change for me is a sense of stability I've never had before. After needing frequent factor injections my entire life, it's really liberating not to rely on them anymore or ride the rollercoaster of peaks and troughs. I don't worry as much about breakthrough bleeds, and I don't have to think about managing my hemophilia B every day. It's not just about saving time for me, it's about regaining a sense of normalcy and moving around with more confidence.

Everyone's journey is personal, but for me, this choice has changed my day to day for the better, and I'm so pleased with the outcome.

Dr. Turck:
It's great to hear you're doing well and that you had a positive experience! So staying with you a moment longer, Greg, what advice would you give to other patients who might be considering HEMGENIX?

Greg:
If they're like me and looking for a different treatment option, I'd encourage them to talk to their doctor. Then, their doctor can work with them to go through a process to check to see if they're eligible for HEMGENIX.

You know, I didn't think my insurance company would pay for the treatment. But my medical team worked directly with the manufacturer and my insurance provider to provide the coverage, so I didn't have to spend my time worrying about it. And a few months later, I was thrilled that it was approved. So, I'd advise anyone considering HEMGENIX not to let insurance concerns hold them back and to check with their insurance.

As for the technology itself, I really encourage anybody who's considered gene therapy for their hemophilia B to learn more about it, because the science behind it has been studied for a long time.[1] Check out educational websites on hemophilia B and gene therapy to hear the stories of people like me, who have suffered with this same disease our entire lives, managing our condition without prophylaxis, and thriving in ways we never thought possible.

Dr. Turck:
That's great advice, Greg, thank you. And before we end here today, Dr. Quon, what recommendations would you give to providers who are preparing to treat their patients with HEMGENIX?

Dr. Quon:
I'd be thrilled to see more healthcare providers consider HEMGENIX for their patients, and for those who are, I'd encourage them to proactively identify patients for HEMGENIX and screen patients to assess eligibility. And don't forget it's also important to educate and train other members on your team, like pharmacists, nurses, other providers, and ancillary staff to ensure everyone is prepared come infusion day. By taking these steps, you can offer your patients the opportunity to see meaningful benefits in their health and daily lives.

Dr. Turck:
That's a wonderful thought as we come to the end of today's program. And I want to thank my guests, Dr. Doris Quon for sharing her clinical expertise and Greg for sharing his personal experience with HEMGENIX gene therapy for hemophilia B. Dr. Quon, Greg, it was great speaking with you both today.

Greg:
Thank you for having me.

Dr. Quon:
Thank you, it was my pleasure.

Dr. Turck:
For ReachMD, I'm Dr. Charles Turck. Before we close, let's take a moment to review some Important Safety Information.

ReachMD Annoucer:
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 at Hemgenix.com/hcp

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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