Pharmacy Focus:
Hemgenix® – A New Gene Therapy for Hemophilia B

Key Takeaways Regarding Hemgenix®

- Gene therapy approved November 22, 2022, for adults with Hemophilia B (without inhibitors) who use prophylactic treatment and have a history of a life-threatening bleed or repeated serious spontaneous bleeding episodes
- Offers a one-time therapy to potentially increase Factor IX levels to reduce both bleeding episodes and the need for prophylactic therapy
- Market price of $3,500,000 for the one-time dose

Hemophilia B Disease Overview[1,2,3,4,5]

Hemophilia is a rare disorder characterized by excessive bleeding due to impaired blood clotting. Those with hemophilia most commonly present with unusual bruising and bleeding, which can be both external or internal. Excessive bleeding can occur with cuts or scrapes, dental work and surgeries. However, with more severe cases, bleeds can appear spontaneously. Larger joints, such as knees, ankles and elbows are most commonly affected.

There are different types of hemophilia, including Hemophilia A, Hemophilia B, and von Willebrand's Disease, but only Hemophilia B will be covered in this article. Hemophilia B (or Christmas Disease) is caused by decreased or absent levels of clotting factor IX. Patients with Hemophilia B may have mild, moderate, or severe cases of this disease based on their factor IX levels.

It is estimated that less than 5,000 people in the United States have Hemophilia B, with less than one percent having a severe instance of the disease. Hemophilia B more commonly affects males than females, with all races and ethnic groups affected equally. Generally, those with severe disease states will be diagnosed between birth and the first few years of life, while those with mild or moderate cases or acquired Hemophilia may not be diagnosed until later in life.

Current Treatment Options[5]

The current standard of care treatment for those with Hemophilia B is the replacement of Factor IX clotting factors to prevent bleeding episodes and other complications. Factor replacement products can be derived from human blood/plasma or be recombinant (manufactured in a laboratory with no human blood proteins). Recombinant factor replacement products are favored as they have no risk of virus transmission.

Continued...

	Factor IX Replacement Products (e.g., Alprolix®, Idelvion®, Rixubis®, etc.)	**Hemgenix®**
FDA Approved Use	**With children and adults for:** • On-demand treatment and control of bleeding episodes • Perioperative management of bleeding • Routine prophylaxis	**With adults who:** • Currently use Factor IX prophylaxis • Have any history of life-threatening bleeds • Have a history of repeated, serious bleeding episodes
Average Annual Cost	$500,000 to $1,100,000*	$3,500,000 for a one-time infusion plus 6 months of continued prophylaxis following Hemgenix®
Payment Structures	Medical and/or Prescription Benefits	Medical Benefits
HCPCS Codes for Medical Billing	J7193-J7195, J7200-J7202	J3490, J3590, C9399

**Average cost for Factor IX replacement products seen in HM Insurance Group cases (2021-2022).*

Complications of factor replacement therapy can include infusion reactions and the development of inhibitors. Inhibitors prevent the current factor treatments from working the same as before, making it more difficult to prevent or stop a bleeding episode. The presence of inhibitors requires the use of increased doses and can end up costing more than $1.5 million per year, versus $500,000 to $1,100,000 for severe patients without inhibitors.

Hemgenix® Overview[6,7]

Hemgenix® is a new gene therapy produced by CSL Behring. It was approved November 22, 2022, for use in adult patients with Hemophilia B who currently use Factor IX prophylaxis, have had a life-threatening bleed, or have had repeated, serious spontaneous bleeding episodes. There is a low likelihood of use in patients with co-morbidities including HIV, hepatitis, end organ disease and thromboembolic events. Patients who currently have inhibitors present and liver abnormalities/disease are not eligible to receive Hemgenix®.

Overall, the goal of the one-time Hemgenix® infusion is to increase the production of Factor IX clotting factor to reduce both bleeding episodes and the need for prophylactic and/or as needed factor replacement therapy. The full effect of Hemgenix® takes weeks to months – participants in the clinical trial continued prophylactic treatment for at least six months after receiving the gene therapy infusion, with two of 54 participants unable to stop prophylactic treatment at all. After receiving Hemgenix®, most participants attained factor IX levels similar to that of those with mild disease severity and required only as needed factor IX replacement treatment for acute bleeding episodes.

Continued...

Cost Containment Considerations

As part of its HMConnects™ cost containment program, HM Insurance Group (HM) works to support cost management opportunities around the use of gene and cell therapies and other high-cost pharmaceutical treatment options that can impact our clients' bottom line. The Pharmacy Operations (RxOps) team watches the market – and our book of business – to anticipate how current and future advancements will impact financial risk levels for HM's client base. Standard practices include reviewing, auditing and collaborating on the content of current policies, monitoring trends and implementing appropriate cost savings techniques. Additional practices include identifying the stockpiling of medications, determining if prescriptions are filled via in-network pharmacies and confirming that prescriptions are properly dosed based on weight and lab values when appropriate. All of these services are provided to HM's clients at no additional cost to them.

Pharmacy Focus provides valuable information about pharmaceutical industry developments and their associated costs that can impact the growing claims trend in the self-funded insurance market. Be aware of influences and gain insight into approaches that may help to contain costs. Please share topic suggestions or feedback with **HMPharmacyServices@hmig.com**.

HM Insurance Group

800.328.5433 | hmig.com

Resources: [1]"Hemophilia," Mayo Clinic, https://www.mayoclinic.org/diseases-conditions/hemophilia/symptoms-causes/syc-20373327, accessed November 23, 2022; [2]"Treatment of Hemophilia," CDC, https://www.cdc.gov/ncbddd/hemophilia/data.html, accessed November 23, 2022; [3]"Data & Statistics on Hemophilia," CDC, https://www.cdc.gov/ncbddd/hemophilia/data.html, accessed November 23, 2022; [4]"Hemophilia B,"GARD, https://rarediseases.info.nih.gov/diseases/8732/hemophilia-b, accessed November 23, 2022; [5]"Hemophilia B," NORD, https:// rarediseases.org/rare-diseases/hemophilia-b/#:~:text=Hemophilia%20B%20can%20range%20from,less%20than%201%25%20of%20normal, accessed November 23, 2022; [6]Hemgenix, package insert, CSL Behring LLC, 2022; [7]Phase III, Open-label, Single-dose, Multi-center, Multinational Trial Investigating a Serotype 5 Adeno-associated Viral Vector Containing the Padua Variant of a Codon-optimized Human Factor IX Gene (AAV5-hFIXco-Padua, AMT-061) Administered to Adult Subjects With Severe or Moderately Severe Hemophilia B, ClinicalTrials.gov identifier: NCT03569891, https://clinicaltrials.gov/ct2/show/NCT03569891?term=etranacogene&cond=hemophilia+B&draw=2&rank=1, accessed November 23, 2022.

12/22 MX2132451