



Midwest Business Group on Health

Hemophilia & Bleeding Disorders:

Engaging Vendor Partners in Solutions that Effectively Support Patients & Manage Costs

Understanding Rare Diseases

A rare disease is a condition that affects fewer than 200,000 people at any given time. An estimated 25-30 million people in the US are affected by one of the more than 6,800 rare diseases identified today.

While medications for rare diseases have a significant impact on health outcomes and improved quality of life, they often come at a steep price for employers.

In 1983, the FDA created the [Orphan Drug Act](#), which provided incentives for drug companies to develop treatments for rare diseases. Since this act was signed into federal law, more than 340 drugs have been approved to treat these very serious diseases. Even still, it is estimated that 95% of all rare diseases do not have an FDA-approved drug treatment.

According to America's Health Insurance Plans (AHIP) [2019 report](#), the price of orphan drugs is increasing

at a far more rapid pace than other specialty and traditional drugs. [Off-label](#) use for these therapies (i.e. use of a drug beyond its approved FDA indication) is also adding to costs.

The high price tag these drugs often carry can be a significant burden for both the patient and employer. More employers are showing increased levels of concern over providing coverage for orphan drugs and other costly therapies in the pipeline, including gene therapies. With few having strategies in place to impact the rising costs of treatment, scrutiny is expected to intensify.

[Read more](#) about rare diseases from the *National Human Genome Institute*.

A Rare Disease Snapshot: Hemophilia

Hemophilia is a bleeding disorder that affects over 30,000 people in the US. There are two types of Hemophilia, A and B. Hemophilia A affects about 20,000 people in the US. People with hemophilia are lacking one or more

Why should employers care about hemophilia as a rare disease?

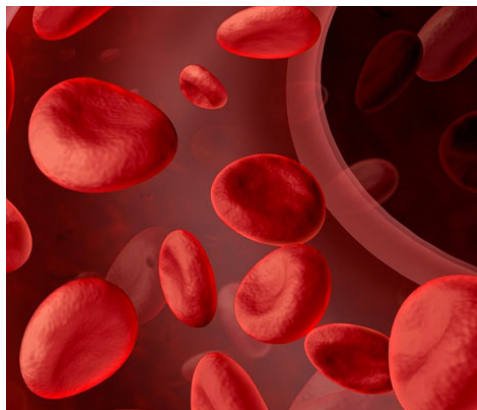
- Hemophilia is among the highest cost claim conditions, averaging **\$300-\$500K** and can be significantly higher for patients who develop an **inhibitor**.
- Pharmacy benefit vendors can add to this cost through wasteful practices that dramatically increase employer costs.
- Significant savings can be realized through evaluating current plan design, reviewing data and making vendors accountable (see employer action steps).



important proteins called clotting factors. Without these proteins, blood cannot clot normally so excess bleeding can occur after an injury or surgery; internal bleeding may damage organs and tissues and even be life-threatening. [Read more](#) about hemophilia from the *CDC*.

Currently there is no cure for hemophilia, but very effective treatments are available including prophylactic medications to prevent bleeds and on-demand medications for acute care of bleeds when they occur. Generally, the goal of treatment is to decrease the frequency and severity of bleeding episodes and prevent complications related to bleeding.





The Economics of Hemophilia

Although hemophilia is a rare condition with extremely low prevalence, it represents an important driver of health care costs and is a [leading high-cost claimant condition](#) for people between age 20 and 39. The average annual cost to manage and treat the condition is [\\$300,000 to \\$500,000](#), 90% of which is related to the use of specialty drugs. This amount can increase significantly depending on disease severity, comorbidities and whether [inhibitors](#) are present. If complications arise, the annual cost to manage and treat this condition can easily reach over \$1 million.

Medical claims for people with Hemophilia A, the most common type, are [twenty times higher](#) when compared with the non-hemophilia population. By comparison, cancer is often the number one high-cost claimant condition for employers based on frequency and cost of claims. However, despite it affecting significantly fewer members, the average cost of treatment for hemophilia is more than three times higher than cancer.

Hemophilia Cost Drivers

Most employers hand off the development of hemophilia cost containment strategies to intermediaries (pharmacy benefit managers (PBMs), carriers, Third Party Administrators (TPAs) and/or pharmacy consultants). While these vendor partners may develop appropriate strategies that result in the lowest total cost of care, there are many instances where conflicts can and do exist that increase employer costs unnecessarily. To better manage costs, it is important to gain a basic understanding of key cost drivers:

Vendor Oversight, Waste and Assay Management

Hemophilia assay management is the process of filling a prescription as

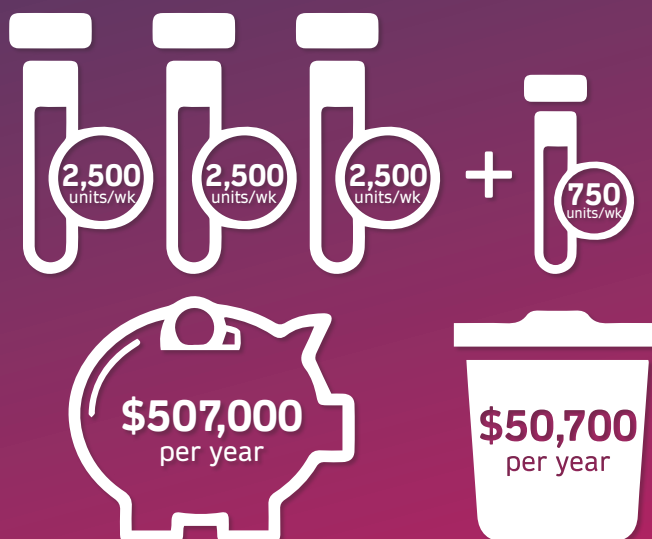
Vendor partners include pharmacy benefit managers (PBMs), carriers, Third Party Administrators (TPAs) and/or pharmacy consultants.



closely to the prescribed target dose as possible using clotting factor vials (vials come in a range of unit or assay sizes). When there is a lack of oversight by vendors on how much clotting factor is sent to the patient and/or consistent over-prescribing, waste occurs and can significantly increase employer costs.

Here is a real-life example:

- Patient is prescribed 2,500 units of clotting factor three times per week
- Guidelines allow specialty pharmacy to ship additional clotting factor at plus (+) or minus (-) 10%; the maximum amount is almost always filled (in this case, 250 extra units per prescription)
- Patient received an extra 750 units each week (250 units X 3)
- The financial impact to the employer is substantial:
 - ▶ Average cost for clotting factor per unit = \$1.30
 - ▶ $2,500 \text{ units} \times \$1.30 = \$3,250 \times 3 \text{ vials/week} \times 52 \text{ weeks} = \text{\$507,000}$ (actual cost of necessary treatment)
 - ▶ $\text{Additional } 750 \text{ units prescribed} \times \$1.30 \times 52 \text{ weeks} = \text{\$50,700}$ (unnecessary cost; added “waste”)



Future cell and gene therapies for hemophilia pending FDA approval may cost up to [\\$2.5M](#) per eligible patient for a one-time treatment to address the cause of the disease.

Site of Care

Hemophilia is a complex disorder requiring a high-touch, individualized approach to patient care. Where care is directed has an important impact on outcomes for members and costs for employers.

Most individuals with hemophilia self-infuse at home and get their medications from specialty pharmacy networks such as independent specialty pharmacies, home care pharmacies, pharmacy divisions owned by national retail chains, specialty pharmacies owned by carriers and PBMs and [Hemophilia Treatment Centers](#) (HTCs).

Medication Management

Hemophilia treatment consists of either infusions or subcutaneous injections which can represent up to 90% of the member's total cost of care. Each treatment has potential for waste. Since these are biologic/specialty drugs, prescriptions are written in a way that does not follow traditional dosing guidelines. This can lead to dispensing excess medication and unnecessary additional cost to the employer, especially related to on-demand use of clotting factor. Also, high out-of-pocket costs can cause patients to avoid or restrict the use of medications. Correct placement of these therapies within a formulary and plan design is essential to ensure adequate coverage and lower out-of-pocket costs.



Hemophilia Treatment Centers

Employers typically carve out their specialty pharmacy network to their carrier or PBM and since they own these pharmacies, they are financially vested... which can result in waste. For specialty pharmacies located within Hemophilia Treatment Centers (HTCs), the top priorities include optimally managing the patient and total cost of care.

Many employers are not aware that HTCs exist. As a medical home model, they provide comprehensive case management and care coordination to patients serving as federally designated centers of excellence established by The Federal Office of Maternal and Child Health of the Health Resources and Services Administration (HRSA). HTCs are required to follow specific [guidelines for treatment](#) which were developed by the National Hemophilia Foundation's Medical and Scientific Advisory Committee to promote evidence-based care and a multi-disciplinary team approach to integrated comprehensive care.

Today, there are over 140 federally recognized HTCs across the US, most of which are embedded in an academic center or hospital. Many HTCs offer discounted medications under the 340B Drug Pricing Program for lower average pricing per unit. View the [Hemophilia Treatment Center Directory](#).



Patient outcomes and the cost of care for HTCs have proven to result in:

- Fewer complications
- Less unnecessary visits to the ER
- Decreased number of bleeds
- Reduced number of infusions
- Proper dosing of clotting factor and adherence to therapy

Prescription/Adherence Management

For people with hemophilia, prescription non-compliance can contribute to poor outcomes and even death. With poor adherence, frequent bleeding and recurrent spontaneous bleeding episodes into the soft tissue and joints can occur. This can lead to joint damage and severe disability. Because the damage is progressive, it can lead to severely limited mobility of joints, muscle deterioration and chronic pain. Significant cost implications can result from bleeding episodes that lead to unnecessary ER visits, hospitalizations and complications from bleed-related joint damage. To reduce waste and minimize health care costs, communication and coordination between the medical care provider, pharmacy and the patient are paramount.

“

We want to make sure we're focused on the whole patient and the family. In our case it was two dependents who had hemophilia. For the family, how has this impacted the father's ability to be effective at work? For the company, how has this impacted disability costs, lost workdays, productivity, etc.?

”

Employer Action Steps

Data, Plan Design & Vendor Management

The key for employers to effectively manage individuals with hemophilia, at the lowest total cost of care with optimal patient outcomes, is to learn what levers are driving the spend; then use that knowledge to require the exchange of pertinent data from vendor partners. This data and the information needed to quantify total cost of care is not routinely provided by these vendors. Data on where the spend is captured – under the pharmacy plan, medical plan, or both – is critical because drug costs do not always appear in pharmacy claims alone. Integrating disability and absence data with medical and pharmacy claims can be a challenge, but essential to view the entire cost and impact.



Employer Action Steps

Use these steps to evaluate your current benefits strategies, track prevalence and identify and reduce wasteful spending. Even if all the steps cannot be completed, there is value in doing as many as possible.

Step 1: Evaluate your current benefit plan design to:

- ❑ Determine which specialty pharmacy network strategy is in place (exclusive, open).
- ❑ Ensure contracted in-network specialty pharmacies adhere to [MASAC #188 guidelines](#).
- ❑ Verify at least one Hemophilia Treatment Centers and one specialty pharmacy are in-network.
- ❑ Confirm members have access to more than one type of health plan (e.g. PPO, EPO, HDHP, etc.); educate them around the different levels of financial responsibility for each.
- ❑ Ensure appropriate case management and care coordination is evidence-based using an integrated multi-disciplinary team approach (see [Hemophilia Treatment Centers](#)).
- ❑ Identify what utilization management strategies are in place (e.g. prior authorization, step therapy, preferred drug



Experts from the [National Hemophilia Foundation](#)

are available to assist employers in evaluating their data at no cost. A consultant/broker may also be able to conduct a similar retrospective review of this data. Be sure to check with them regarding fees that may be charged before starting.

list, etc.); make certain criteria is not overly restrictive and patients have timely access to care without unintended or negative consequences (e.g. severe patient needs to demonstrate a certain number of bleeds in order to get access to particular therapies or is limited to one type of prophylaxis – one bad bleed can lead to permanent joint damage or even death).

- ❑ Determine if a co-pay accumulator adjustment program is being used; they are not recommended for use with high-cost, high-value drugs that have no generic equivalent.

Step 2: Identify the number of individuals with hemophilia to determine the current cost to your plan and help you track potential waste.

- ❑ The following links will allow you to cross reference both your pharmacy and medical claims data to identify claimants and analyze opportunities for savings.
 - ▶ [ICD-9 or ICD-10 codes](#) will include costs on the medical side like hospital and ER visits.
 - ▶ [J-codes and/or NDC numbers](#) will include costs related to hemophilia specialty drugs.
- ❑ Ask your carrier/claims administrator to pull ER and hospital claims data with hemophilia as the primary diagnosis code.
- ❑ If hemophilia drugs are linked to a carve-out approach, a larger component of the drug cost will show up in prescription drug benefit claims.





Step 3:

Hold your vendor partners accountable.

- ❑ Work with a neutral third-party (e.g. National Hemophilia Foundation) to conduct a retrospective claims audit that can be used as the baseline for driving prospective strategies and to ensure your vendors are doing what is required through quarterly reporting.
- ❑ Develop prospective data sharing requirements such as this [sample data collection template](#) which includes:
 - ▶ Assay management performance reviews:
 - Identify target dose as written compared to actual dispensed dose.
 - Identify current contracted allowable +/- over target.
 - ▶ Dose management performance reviews:
 - Ordered versus shipped – oversight/reporting to confirm dispensations match shipments.
 - Patient bleed logs (collected and reviewed) to determine medication adherence and inventory on hand at patient's home.

- Ensure your in-network specialty pharmacy providers:

- ▶ Are required to follow the MASAC 188 guidelines.
- ▶ Do not auto ship medications.
- ▶ Require monthly communication with the patient.
- ▶ Identify current inventory on hand before sending additional doses.
- ❑ Consider integrating performance guarantees into your PBM contract – See Employer Example below.

Step 4: Check stop-loss policy (if utilized) and claims to confirm:

- ❑ Whether medical and/or prescription drug claims are covered in combination or as separate stop-loss thresholds.
- ❑ Amount of coverage and qualifying thresholds for stop-loss coverage to begin.
- ❑ Types of claims covered and timeframe for coverage.
- ❑ Number of treatment episodes reaching payment thresholds over three individual, but consecutive, plan years.
- ❑ Total amount of coverage provided for each.

Employer Example Using Performance Guarantees with PBM

- Worked with PBM and specialty pharmacy to deliver a better patient experience for people with hemophilia. Drove accountability with PBM for cost:
 - ▶ Tracked medical and pharmacy claims for people with hemophilia who have a history of ER visits to capture precursors to care.
 - ▶ Tracked trends year over year for ER visits (percent of decrease and if minimum threshold of visits were exceeded) and hospital length of stay to determine impacts, achieve more effective clinical management and reduce occurrences.
- Drove accountability with PBM for care:
 - ▶ Required performance guarantees be subject to downside financial risk based on patient experience.
- Provided education and information to medical care team(s); shared available hemophilia prescription coverage to assist with appropriate counseling.



*We don't get data on the medical side or receive detailed reports from our PBM for hemophilia therapies so we were not aware how much we were overpaying for inappropriate prescribing. The **MBGH Hemophilia Toolkit** helped us figure this out and fix it.*



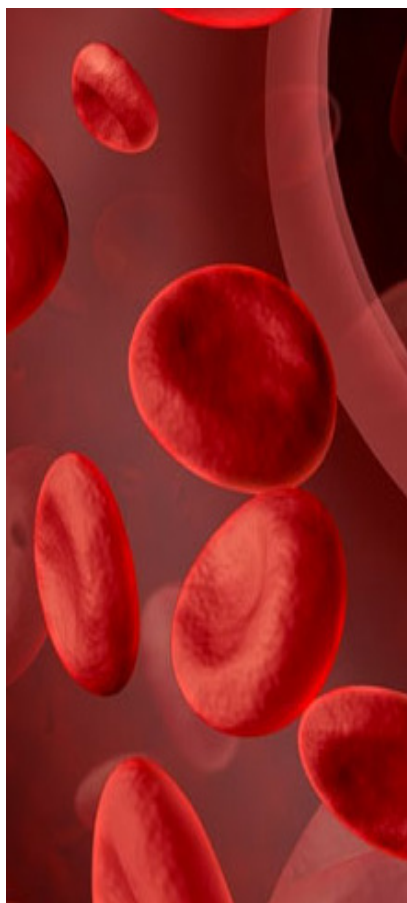


Employers shared with MBGH what they look for when determining the value of new therapies:

- *I don't believe rebates should determine where we cover a benefit – it should be efficacy. Any study that shows efficacy is important. I use those studies to talk to my health plan and PBM...and then I fight back when I need to..*
- *With some of the game changing therapies, we talk directly with the manufacturer to better understand their data and evidence and how the science stacks up compared to others.*
- *I look at random controlled trials, double-blinded studies and peer-reviewed studies that focus on efficacy.*
- *We talk with our health plans, PBMs and consultants to understand how they have evaluated certain new-to-market drugs from a clinical standpoint. It all goes back to data and evidence made available through the different stakeholders.*
- *I look to what the prescribing community has to say, especially with new therapies. I am interested in what medical associations, large hospital groups and prescribers are doing and how they are reacting when a new therapy comes out.*



Important Discussion Topics for Employers with their Vendor Partners



National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) [recommendations](#) establish standard treatment guidelines for Hemophilia A, B and other bleeding disorders.

The Institute for Clinical and Economic Review (ICER) reports provide recommendations for carriers and PBMs on the treatment of [Severe Hemophilia A](#) and [Hemophilia A with Inhibitors](#).

Based on MASAC and ICER reviews, employers should consider these recommendations when discussing hemophilia coverage with payers.

- Ensure coverage of prophylaxis treatment is at a level adequate to provide bleed protection.
- If utilization management programs (UM) such as prior authorization or step therapy

are in place, ask your vendor what clinical criteria is used:

- ▶ Are patients experiencing a delay to care when stepping through the UM programs?
- ▶ Could there be unintended medical consequences or costs due to the UM programs?
- Consider comparative clinical and economic evaluation of prophylaxis treatments in the ICER reports.
- Consider requiring management of prophylaxis be done by or in consultation with a Hemophilia Treatment Center to better manage costs through their integrated comprehensive care model.
- Explore innovative approaches to covering high-impact single use therapies such as gene therapies for hemophilia.

About MBGH

MBGH is one of the nation's leading and largest non-profit employer coalitions. Members are represented by human resources and health benefit professionals for over 135 mid, large and jumbo self-insured public and private companies who provide health benefits for more than 4 million lives. Employer members spend over \$12 billion annually on healthcare. Since 1980, members have used their collective voice to serve as catalysts to improve the cost, quality and safety of health benefits.

Employer Toolkit

MBGH created the online [Hemophilia and Bleeding Disorders toolkit](#) to give employers access to knowledge, best practices and high-quality resources to more effectively manage the high cost of hemophilia and improve the lives of those impacted by this rare but serious disease. Vetted by health benefit professionals from employer member companies, this resource is available to employers across the country to help them:

- Better understand hemophilia prevalence and related cost
- Identify how benefit plan design can support (or hinder) best in class treatments
- Understand the steps to take with vendors to improve overall quality of care and reduce waste

“

When it comes to hemophilia we tend to focus on cost and it's really education that's needed; this is where the [toolkit](#) provides a lot of value. With education we can better understand waste factors to bring costs down.

”

Developed in partnership with Genentech, a member of the Roche Group. M-US-00010867(v1.0) 6/21

Authors

Cheryl Larson
President & CEO, MBGH

Dawn Weddle
Director of Member Engagement, MBGH

Kollet Koulianos
Senior Director of Payer Relations,
National Hemophilia Foundation

The information provided in this resource is based on the authors' and contributors' experiences working in the health benefits and health care industry. For more information on any aspect of this report, please contact info@mbgh.org.

