EMPLOYER ACTION BRIEF

MBGH
Midwest Business Group on Health

Hemophilia

Engaging Vendor Partners in Solutions that Support Patients & Manage Costs

Understanding Hemophilia's Impact

Hemophilia is a high-cost, chronic genetic disorder that presents unique challenges for employer-sponsored health plans. Because it is a hereditary condition, it doesn't just affect a single employee; it often impacts multiple family members (dependents) and carries lifelong financial implications. Whether or not you have a current employee or dependent with this condition on your plan, it's important that employers understand the financial exposure of hemophilia.

The Economics of Hemophilia

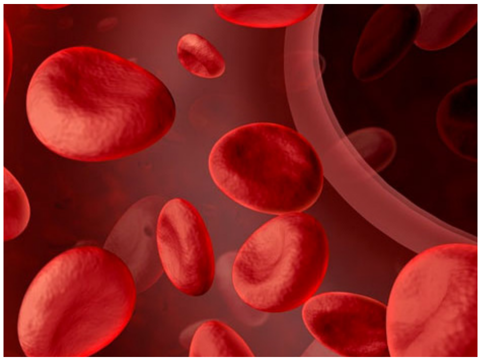
Although hemophilia is a rare disease, it is a leading high-cost claimant condition for people between the ages of 20 and 39. The average annual cost to manage and treat the condition may be $300,000 to $500,000 or higher, 90% of which is related to the use of specialty drugs. This amount can increase 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 B are twenty-five times higher than claims of the non-hemophilia population. Although cancer is often the number one high-cost claimant condition for employers based on case volume, the average cost of hemophilia treatment for one case is three times higher than cancer. The financial burden is only part of the challenge. Hemophilia can cause recurrent joint bleeds that lead to chronic pain, reduced mobility, and long-term musculoskeletal damage. These complications often require time away from work for treatment, recovery, medical appointments, and/or emergency care. In the workplace, these individuals may experience a lack of concentration, fear, anxiety, fatigue, and limited stamina, affecting their ability to perform physically demanding tasks. Employers may need to modify duties, offer flexible schedules and/or support remote work to maintain productivity and safety.

Why should employers care about hemophilia as a rare disease?

- Total annual health care costs can be up to 25 times more for a person living with hemophilia B compared to those who don't have the disease.
- The annual cost of clotting factor therapy is $300,000 and the medical expenses can be twice that. The cost 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 on pages 3-4).

Although employers are a key payer group, they may have very limited knowledge of hemophilia because of its low prevalence. They also receive minimal information from carriers, PBMs, TPAs and pharmacy and medical consultants on best practices. Most often, employers receive information in a retrospective manner, requiring action after the condition presents in claims reports – leaving them without proactive strategies to manage care or spending.



Facts About Hemophilia

Clinically, people with hemophilia are lacking one or more important proteins called clotting factors. In the absence of 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. There are two types of Hemophilia, A and B – each defined by the specific type of missing or deficient clotting factor. Hemophilia A is significantly more common, affecting 80-85% of all cases.

Despite hemophilia being more common in men (90% of all cases are male), women may also be diagnosed with this condition and require treatment.

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.

Read more about hemophilia from the CDC.

Current and future cell and gene therapies for hemophilia are costly, up to $3.5M for a one-time treatment. At the time of publication, these therapies are not considered curative but may allow patients significant time without additional treatments.

Hemophilia Cost Drivers

Many employers rely on intermediaries (pharmacy benefit managers (PBMs), carriers, Third Party Administrators (TPAs) and/or pharmacy consultants) to manage and mitigate the high costs of this disease. While vendor partners may recommend cost containment strategies to reduce the 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, including vendor oversight, site of care and medication management.

Vendor Oversight, Waste and Assay Management

Hemophilia assay management is the process of filling a prescription as 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.

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-administer 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).

By contracting with HTCs, employers can realize significant cost savings including fewer lost work days and decreased complications—reducing unnecessary visits to the ER (HTC patients are 47% less likely to use the ER)—decreased number of bleeds, reduced number of infusions, increased proper dosing of factor replacement, and improved adherence to therapy.

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

Medication /Adherence Management

Hemophilia treatment typically consists of either infusions or subcutaneous injections which can represent up to 90% of the total cost of care.

Infusions and injections have 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 unnecessarily adding cost to the employer, especially related to on-demand use of clotting factor.

High out-of-pocket costs can cause patients to avoid or restrict the use of medications. Plan design, including the correct placement of these therapies within a formulary, is essential to ensure adequate coverage and lower out-of-pocket costs.

For people with hemophilia, prescription non-compliance can contribute to poor outcomes and even death. To reduce waste and minimize health care costs, communication and coordination between the medical care provider, pharmacy and the patient are paramount.

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 150 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.

By using HTCs patients may experience benefits such as:

- 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
- Lower cost of care
- Integrated, individualized care
- 24/7/365 support and follow-up

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:

☐ Understand your pharmacy network strategy.

- Verify at least one Hemophilia Treatment Center pharmacy is in-network.
- Confirm with your benefits consultant and /or vendor partner that your specialty pharmacies adhere to MASAC #188 guidelines.

☐ Provide health plan education including how plan selection impacts the treatment of high-cost diseases. Consider putting in place a decision support tool to help members choose the best health plan for themselves and their dependents.

☐ Ensure case management and care coordination is evidence-based using an integrated multidisciplinary team approach.

Experts from the National Bleeding Disorders 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.

☐ Identify what utilization management strategies are in place (e.g. prior authorization, step therapy, preferred drug 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).

Copay accumulator and maximizer programs create steep, immediate out-of-pocket costs, causing many patients to delay or abandon treatment and to seek care in the emergency department.

These strategies are not appropriate for high-cost, high-value therapies with no generic equivalent, such as hemophilia treatments, and can drive avoidable clinical and financial consequences.

Recommendation: Exempt hemophilia therapies from accumulator and maximizer programs. Several states have already enacted laws restricting or banning accumulator use.

☐ 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.

According to the Journal of Managed Care and Specialty Pharmacy, the intent of prior authorizations is to **ensure drug therapy is medically necessary, clinically appropriate, and aligns with** evidence-based guidelines. The reality for rare disease patients is these tactics delay care, lead to both patient and clinician frustration, and are rarely denied.

Employers: There can be unintended consequences of delayed care when prior authorizations are in place for life-saving drugs.

Step 2: Determine the number of individuals with hemophilia and the current cost to your plan.

- ☐ 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 identify costs on the medical side, like hospital and ER visits.
  - J-codes and/or NDC numbers identify 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 to conduct a retrospective claims audit that can be used as the baseline for driving prospective strategies and ensure your vendors are doing what is required through regular reporting.
- ☐ Share this sample data collection template with your health plan and/or PBM to help you assess clinical and financial performance.
  - 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:
  - 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 toolkit for more info.

Step 4: Gain a better understanding of financial exposure and impact of individuals seeking care.

- ☐ If you have a stop-loss policy, does it provide coverage with condition specific exclusions?
- ☐ What exclusions exist? Are they applied differently to existing employees, new hires, or newly added dependents?
- ☐ Are claims subject to reimbursement limits? How are claims managed if costs span across multiple years?
- ☐ What kind of gene coverage policies does your plan include (if any)?

For more information on financial exposure, click here.

About MBGH

Midwest Business Group on Health (MBGH) is a 501c3 non-profit supporting employers seeking solutions to better manage the high cost of health care and the health and productivity of their covered populations. Founded in 1980, MBGH offers members leading educational programs, employer-directed research project, purchasing opportunities and community-based activities that increase the value of health care services and the health benefits they offer to members. MBGH serves over 170 companies who provide health benefits for more than 4 million lives with employer members spending over $15 billion on healthcare each year.

mbgh.org

Important Discussion Topics for Employers with their Vendor Partners

National Bleeding Disorders 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.

Read more about hemophilia via the Hemophilia Toolkit developed by MBGH.

> *"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."*

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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.