Comprehensive Care Sustainability Collaborative

National **Bleeding Disorders** Foundation

CCSC HEMOPHILIA RESPONSE TOOL KIT

A resource for payers to assure optimal outcomes are achieved at the lowest total cost of care

Although hemophilia is a rare disorder, affecting approximately 25,000 people in the U.S., it ranks among the highest cost health care conditions, averaging ~$270,000 per claimant annually.[1] The low prevalence often leaves payers to view these claimants as outliers, with no option but to hand off the development of cost containment strategies to their third-party administrator (TPA), pharmacy benefits manager (PBM), and/or pharmacy consultants. While these partners may develop appropriate strategies that result in the lowest total cost of care, there are certainly many instances where conflicts could and do exist. Such conflicts leave payers vulnerable if they lack a basic understanding surrounding the key cost drivers and the relevant performance data necessary to quantify optimal total cost of care strategies.

CCSC's Hemophilia Response Tool Kit is designed to serve as a step-by-step guide, with embedded resource links, that will help payers identify and uncover key cost drivers, analyze historical performance data, review current plan designs that may inadvertently result in higher total cost of care, and provide the necessary tools and resources that will allow payers to confidently participate in the development of forward-moving strategies.

For more information, visit www.CCSC*Hemo*.com

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Understanding the Basics

STEP 1

People with hemophilia are lacking one or more important proteins, called clotting factors, which are needed for blood clotting. The type of hemophilia a person has depends on the specific type of missing or deficient clotting factor.

People with hemophilia bleed for a longer time than others after injury or surgery. They may also have internal bleeding—especially in knees, ankles, and elbows—that can damage organs and tissues and even be life-threatening.

Types of Bleeding Disorders

Most bleeding disorders are inherited and affect people from all racial and ethnic groups. Although there are several different types, these are the three most common:

Hemophilia A

- Involves a clotting factor VIII deficiency
- Is four times more common as hemophilia B
- Is responsible for 80% of total cases of hemophilia
- 30% of cases are the result of “spontaneous mutations” meaning there is no family history

Hemophilia B

- Involves a clotting factor IX deficiency
- Is responsible for 20% of total cases of hemophilia
- 30% of cases are the result of “spontaneous mutations” meaning there is no family history

Clinical classification of hemophilia is based on factor activity levels and categorized as mild, moderate or severe, with severe being the most common.

von Willebrand Disease (VWD) Type 1, 2 or 3

- Involves a low level of a protein called von Willebrand Factor (VWF)
- Most common inherited bleeding disorder; an estimated 1 in 1,000 Americans have clinically relevant VWD
- Often milder than hemophilia A and B
- More women show symptoms of VWD due to menstruation and childbirth

Understanding the Basics

STEP 1

Inhibitors

An Inhibitor occurs when one's immune system perceives the clotting factor replacement treatment products in the same way it would a foreign substance; this causes their body to stop accepting the clotting factor treatment as a normal part of their blood and try to destroy it with an inhibitory response.

The development of an inhibitor keeps the treatment from working, creating significant clinical management challenges when attempting to stop or prevent bleeding episodes and therefore requires sub-specialized medical expertise.[2]

Inhibitor Occurrence by Type of Bleeding Disorder:

- Hemophilia A: 25% - 30%
- Hemophilia B: 1% - 5%
- VWD Type 3: 5%–10%

The inhibitor stops the clotting factor treatment from working, making it more difficult to stop or prevent bleeding episodes.

STEP 1 KEY POINTS

Hemophilia A, hemophilia B and VWD are rare bleeding disorders that can be costly to treat.

Classified mild, moderate or severe based on factor activity levels; severe being the most common.

Prophylaxis treatment considered the gold standard.

Treatment considerations:
- Intravenous infusion of replacement clotting factor products
- Subcutaneous injection of non-factor monoclonal antibodies

Treatment Considerations

Schedule

- Episodic / On Demand – Treatment intervention occurs only in response to clinically evident bleeding episodes.
- Prophylaxis – Regular and ongoing treatment to prevent bleeding episodes. Prophylaxis treatment is considered the most effective regimen in reducing bleeding rates and preventing target joints, chronic arthropathy and deformities.

Treatment Types

- Factor Replacement Therapy – The most common treatment for patients with hemophilia involves the intravenous infusion of concentrated replacement clotting factor VIII (for hemophilia A) or clotting factor IX (for hemophilia B) to replace low or missing clotting factor.
- Non-Factor Therapy – A first-in-class, non-factor replacement treatment, therapeutic bispecific monoclonal antibody was approved for hemophilia A in late 2018. Rather than intravenous infusions, this prophylactic treatment is administered as a subcutaneous injection, every week, two weeks or four weeks after four weekly loading doses. As with prophylactic clotting factor replacement treatments, the goal is protection from traumatic and unexpected bleeds and prevention of joint damage and joint disease.

Overview of the 3 Key Cost Centers Related to Hemophilia Management

STEP 2

1. Provider / Site of Care

Hemophilia is a complex and rare disorder, requiring a high-touch, individualized approach to patient care for optimal outcomes.

Making sure patients are under the right care, at the right time and the right place is critical.

Hemophilia Treatment Centers (HTCs) are federally recognized centers of excellence dedicated to the treatment of rare/benign bleeding and clotting disorders. These centers serve as a medical home model of care, bringing together an integrated team of doctors, nurses, physical therapists, social workers and other health professionals experienced in treating people with hemophilia.[3] HTCs are the only recognized standard of care for managing hemophilia and other rare bleeding disorders.[4]

A CDC study of 3,000 people with hemophilia showed that those who used an HTC were 40% less likely to die of a hemophilia-related complications compared to those who did not receive care at a treatment center.[5] Similarly, people who used a treatment center were 40% less likely to be hospitalized for bleeding complications and 47% less likely to visit the emergency department (ED).[6,7]

OPTIMAL MANAGEMENT OF HEMOPHILIA IS BASED ON:

- Early detection and diagnosis
- Prevention (prophylactic) and early treatment of bleeding episodes to avoid complications
- Detection and management of inhibitors
- Psychosocial and educational support
- Monitoring for treatment-related comorbidities
- Coordination of care with other providers and payers involved in management of the patient

2. Medication Management

The high cost of hemophilia treatment is mainly due to the requisite use of specialty drugs to manage and treat the condition. The average annual cost of medication to treat hemophilia is ~$270,000 per patient, accounting for an estimated 90% of total direct medical costs.[1] This amount can increase significantly depending on disease severity, comorbidities and whether inhibitors are present. If complications arise, the yearly price tag can soar over $1 million dollars.[1]

Medical claims for people with hemophilia A, the most common type, are approximately five times higher when compared with the non-hemophilia population.[8] By comparison, cancer is often the number one high-cost claimant condition for employers and payers 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 that of cancer.[9]

Overview of the 3 Key Cost Centers Related to Hemophilia Management

STEP 2

The total cost of care can skyrocket when members with hemophilia are unmanaged or undermanaged, resulting in avoidable use of services and treatments, including emergency room visits and hospitalizations. This can happen when members are treated by health care professionals lacking the training or expertise to effectively treat and manage hemophilia.

Recent research identifies hemophilia as a leading high-cost claimant condition for people in their prime working years, between the ages of 20 and 39. This chronic condition is a disproportionate driver of health care spend, frequently appearing in an employer's top 10 catastrophic claims report.[9]

The impact of hemophilia on your company's spend and/or hospital and ER use can be verified with ICD-9 or ICD-10 codes, hemophilia drug J-codes and/or NDC numbers.

3. Prescription / Adherence Management

Patient non-compliance can contribute to poor outcomes and even death. In addition, significant cost implications can result from bleeding episodes that lead to unnecessary ER visits, hospitalizations and complications from bleed-related joint damage. To help reduce waste and minimize health care costs, communication and coordination between the medical care provider, pharmacy and the patient are paramount.

Medication adherence reduces the occurrence of bleeding episodes and maintains joint health in individuals with hemophilia. 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.

According to the World Health Organization (WHO), adherence to a chronic condition treatment regimen is determined by one or more of the following five dimensions or factors:[10]

- Patient-related (resources, knowledge, attitudes/beliefs, expectations)
- Social and economic (high cost-share/copay, low health literacy)
- Condition-related (severity of disease/symptoms)
- Therapy-related (complexity of regimen, side effects)
- Health system-related (transitioning from hospital to home care, site of care)

Overview of the 3 Key Cost Centers Related to Hemophilia Management

STEP 2

Reasons for non-adherence to treatment with bleeding disorders include:

- Convenience issues/time
- Social and family stress
- Lack of commitment, forgetfulness
- Cost of copays and insurance deductibles
- Disease complications
- Poor venous access
- Transition to adulthood

Strategies for Addressing Non-Adherence

The best approach for addressing non-adherence is to first determine the reasons and then implement a plan with the patient to reduce the risk and improve compliance. One approach includes use of an assessment tool (i.e., questionnaire) to determine the reasons for non-adherence. Once the reason(s) are determined, a plan with appropriate interventions can be implemented by trained staff and/or a care coordinator. This approach must be collaborative, and the patient must be interested in improving adherence. If the reason for non-adherence is financial in nature, the plan may also include assistance to help the patient afford the cost of their medications.

Home therapy removes one major reason for non-adherence: inconvenience. Going to the doctor or a treatment center for therapy takes time and requires transportation. The use of home therapy removes this barrier and can be associated with a substantially lower risk for bleeding complications because of enhanced adherence.

STEP 2 KEY POINTS

The average cost of treatment for hemophilia is more than three times higher than that of cancer.[9]

The total cost of care can skyrocket when members with hemophilia are unmanaged or undermanaged.

Making sure patients are under the right care, at the right time, and in the right place, such as an HTC is critical.

To help reduce waste and minimize health care costs, communication and coordination between the medical care provider, pharmacy, and the patient are paramount.

Identifying Relevant Claims Data

STEP 3

1. Begin by determining whether medical and/or prescription drug claims are covered in combination or as separate stop-loss thresholds. Check for the amount of coverage and qualifying thresholds for stop-loss coverage. Review the types of claims that are covered and the timeframe for coverage. Examine the number of treatment episodes reaching payment thresholds over three individual but consecutive plan years. Verify the total amount of coverage provided for each individual.

2. To determine the number of lives diagnosed with hemophilia or other bleeding disorders, the ICD-9/10 codes on the following link can be utilized.

   Complete List of ICD-9/10 Codes

3. To generate a hemophilia related claims report, the HCPCS J-Codes and NCD Numbers on the following link can be utilized.

   Hemophilia Related HCPCS J-Codes
   NDC Numbers

STEP 3 KEY POINTS

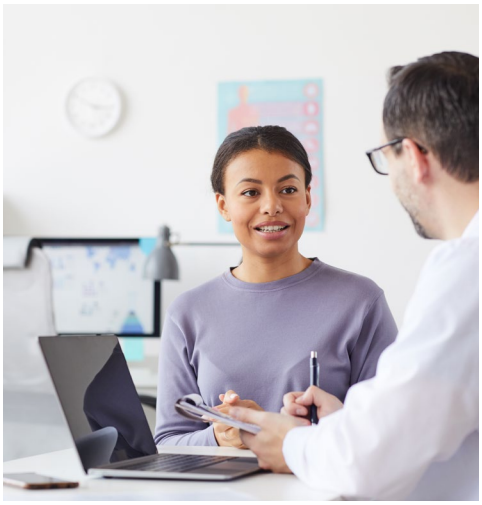
To identify relevant claims data, begin by determining whether medical and/or prescription drug claims are covered in combination or as separate stop-loss thresholds.

Confirm that hemophilia is covered under the appropriate benefit plan (medical and/or pharmacy), amount of coverage, what is included, and the time frame of benefit coverage.

Review Current Benefit Design

STEP 4

1. Hemophilia Treatment Center Inclusion



The care and treatment of members with hemophilia is typically tied to traditional benefit plan design, carrier networks and related provider sites of care. Where care is directed has an important impact on outcomes for members and costs for employers and payers. Although most individuals with hemophilia self-infuse their medications at home, the majority receive their clotting factor from specialty pharmacies and HTCs. Both are considered types of specialty pharmacies with some differences and distinctions.

Because hemophilia is a complex and rare disorder, many providers do not have the experience or expertise to treat and manage the condition effectively. The HTC network, originally established by Congress in 1976, was formed to create a regionalized network of expert multidisciplinary care teams to meet the complex needs of patients with hemophilia and other rare bleeding and clotting disorders. The HTC care model, the original medical home, soon became a blueprint for the management of other chronic diseases. In the 1981 Omnibus Budget Reconciliation Act, the HTC program was referred to as the biomedical success story of the decade.[11]

In the HTC model, the team establishes a proactive comprehensive care plan for each individual that includes a hematologist, nurse coordinator, physical therapist and social worker. Other care providers including dental professionals, genetic counselors, orthopedists, infectious disease specialists and pharmacists are either available onsite or via established partnerships to support the unique needs of people with hemophilia. HTCs offer specialty pharmacy services, providing medications specific to the treatment of bleeding disorders.

Research shows that HTCs are effective at managing outcomes and the cost of care for patients with hemophilia,4 including decreased complications, reducing unnecessary visits to the ER (HTC patients are 47% less likely to use the ER7), decreased number of bleeds; reduced number of infusions, proper dosing of clotting factor and adherence to therapy.

Review Current Benefit Design

STEP 4

Clinical practice guidelines that are the gold standard for treatment of members with hemophilia recommend the use of an integrated medical and pharmacy care model that includes clinical care from a multidisciplinary team. Employers and payers should consider contracting with HTCs as in-network providers of clinical and pharmacy services. (CDC Hemophilia Standards can provide more information on the value of HTCs.) This will help to ensure that an integrated model is being used, members receive optimal care and experience improved outcomes, and employers/payers can manage related ancillary health care services and costs. Having a fragmented care team that is not coordinated with the patient at the center can significantly impact patient outcomes and increase the total cost of care.

If services are carved out to HTCs, it is important to make sure they are in-network for all the services they provide including both medical/clinical care and specialty pharmacy services. The network should consist of at least one specialty pharmacy and one HTC specialty pharmacy option to create competition, drive down unit pricing and improve customer service. Including HTC specialty pharmacy programs in all payer networks either directly or as an allowable option is also recommended. For more information review the NBDF Position Statement on Specialty Pharmacy and Sole Source.

2. Pharmacy Network Adequacy and Restrictions

In specialty pharmacy contracts, employers and payers can be specific about how vendors/partners are required to manage and report on hemophilia drugs. There is an opportunity to provide clarity in both health plan and PBM contracts for these drugs, so hemophilia-related claims can be targeted appropriately and be cost effective. The key is to hold vendors accountable for managing use and spend. One way to accomplish this is by requiring quarterly reports that include agreed upon standard data and metrics, including the following:

- Dispensed clotting factor dose compared to prescribed clotting factor dose (typically measured as a percentage)
- Unit and total cost of clotting factor medication (to ensure the cost is competitive)

During PBM contract negotiations, it is important to get clarification about the cost drivers for hemophilia treatment and their practices for ongoing clinical management of bleeding disorders. It is also important to build a strategy with vendor partners that includes the right contract language, ongoing reporting requirements and whether HTCs are to be included for both medical and pharmacy.

If an employer/plan is under an exclusive specialty pharmacy contract, allowing another pharmacy to dispense would likely violate the contract. However, if you find equitable rates through an HTC, you could consider entering into a single case Letter of Agreement (LOA). This allows the HTC to serve the patient under the medical benefit versus a pharmacy adjudication and avoids violation of the specialty pharmacy contract. When the current specialty pharmacy exclusivity contract expires, consider requiring that HTC's be included in the specialty pharmacy network.

Review Current Benefit Design

STEP 4

3. Adherence to MASAC 188 Requirement

The National Bleeding Disorders Foundation (NBDF) Medical and Scientific Advisory Council developed MASAC 188: Recommendation Regarding Standards of Service for Pharmacy Providers of Clotting Factor Concentrates for Home Use to Patients with Bleeding Disorders, to set the following minimum standards that all specialty pharmacies should adhere to:

- Have staff knowledgeable about clotting factor concentrates, necessary ancillary supplies and the proper handling of both
- Provide the full range of available, FDA-approved clotting factor concentrates, ancillaries and supportive services (either directly or through a third party)
- Process prescription orders in a timely manner (48-hours or less) and fill prescriptions within a certain, acceptable range (e.g., ±5%)
- Be available to patients within normal business hours, have 24-hour emergency services, provide access to multi-lingual customer service representatives, and maintain necessary contact with the treating physicians
- Have appropriate, safe, federally compliant delivery services to ensure timely delivery, a faster process in emergent need cases (less than 12 hours; goal of three hours), and a back-up plan for natural disasters
- Maintain accurate, up-to-date records meeting all federal and state requirements, be HIPAA compliant and:
- Provide patients with accurate information about factor costs per unit and their out-of-pocket responsibilities under their insurance
- Maintain an accurate process to track all shipments
- Participate in the National Patient Notification System for clotting factor concentrate recalls to ensure patient safety

The guidelines should be incorporated into employer/pharmacy provider contracts to ensure that all specialty pharmacy providers meet certain minimum standards when working with people with hemophilia and other bleeding disorders.

Review Current Benefit Design

STEP 4

4. Copay Assistance Restrictions

Due to the rise in health care costs, some employers and payers have implemented cost shifting strategies, such as high deductible health plans, co-insurance, copays and/or higher-tiered placement for specialty drugs. This cost shifting can be especially of concern for members with hemophilia. For specialty medications such as clotting factors, significant costs can result from even one month of treatment. The deductible may be met almost immediately in the coverage year due to the cost of a single order of clotting factor. Members with hemophilia are responsible for substantial costs in a short time frame, as opposed to accruing more moderate costs over the course of the year as typically occurs for non-hemophilia members.

While copay cards have some positive benefits for members such as improving access, affordability and compliance, many employers and payers believe they can also increase costs by encouraging unnecessary use of higher-priced, branded drugs and some believe they circumvent the formulary. But for people with rare diseases like hemophilia, manufacturer copay cards are a mechanism to mitigate the cost burdens that come with ongoing treatment.

Two new specialty copay card programs were introduced in 2017 – accumulator adjustor and copay allowance maximization. Neither program typically allows a member's copay assistance to count towards their deductibles or annual out-of-pocket maximum. When applied to high-cost/high-value drugs, these programs can create a barrier to a member's utilization of necessary and potentially life-saving therapies. In addition, they can reintroduce financial barriers to patient access and negate the benefits of copay assistance programs for those with a chronic/rare disease with no generic alternative.

Employers and payers should consider whether these programs should be applied to life-threatening conditions with no alternative treatments. Employers and payers who implement these programs as a one-size-fits-all option can expose themselves and members to a series of costly unintended consequences. For example, if a member cannot afford their out-of-pocket expenses, they are left with no option but to be non-adherent to their prescribed treatment to prevent bleeds, forcing them to be reactive and seek treatment in the highest cost of care setting, the emergency room. More information can be found by reviewing the NBDF Position on Copay Accumulator Adjustment Programs.

The Health and Human Services (HHS) Notice of Benefit Payment Parameters (NBPP) states that it will not initiate an enforcement action if an issuer of group or individual health insurance coverage or a group health plan excludes the value of drug manufacturers' coupons from the annual limitation on cost sharing, including in circumstances in which there is no medically appropriate generic equivalent available. States may adopt a similar enforcement policy, and HHS will not consider a state to be failing to substantially enforce the annual limitation on cost sharing in cases where a state does so with respect to health insurance issuers.

Review Current Benefit Design

STEP 4

5. Stop Loss Deductible

Because drug treatment protocols for hemophilia can be more maintenance-like than curative, claims can sometimes run well into seven figures in costs. The traditional approach to mitigate the risk of catastrophic claims is to buy stop-loss insurance. As pointed out earlier, it is vital to check for the amount of coverage and qualifying thresholds for stop-loss coverage to begin.

STEP 4 KEY POINTS

Where care is directed has an important impact on outcomes for members and costs.

If services are carved out to HTCs, it is important they are in-network for both medical/clinical care and specialty pharmacy services.

Build a strategy with vendor partners that includes the right contract language, ongoing reporting requirements and whether HTCs are to be included.

MASAC 188 guidelines should be incorporated into employer/ pharmacy provider contracts to ensure that all specialty pharmacy providers meet certain minimum standards.

For people with rare diseases like hemophilia, manufacturer copay cards are a mechanism to mitigate the cost burdens that come with ongoing treatment.

It is vital to check for the amount of coverage and qualifying thresholds for stop-loss coverage to begin.

Collect Prescription Data

STEP 5

1. Obtaining Actual Prescription Information

Hemophilia treatment generally involves the infusion of clotting factor replacement products, which represents upwards of 90% of the member's total cost of care. Since these are biologic drugs, prescriptions are written in a way that does not follow traditional dosing. Clotting factor is made in batches and provided to pharmacies in a variety of vial (assay) sizes. Dispensing pharmacies choose the vial size closest to the patient's prescribed dose, which can lead to over-shipping of product and excess cost to the employer. To further complicate medication management, the script dosage is based on many factors, including a member's weight.

Prescriptions for clotting factor are typically written for + or – 10% for the fill. When the maximum amount is filled every time, it leads to the patient having more clotting factor than needed. Here is a real-life example:

Case Study

A patient in his mid-twenties with hemophilia is prescribed 2,500 units of clotting factor 3x/week based on his weight. Guidelines allow specialty pharmacies to ship additional clotting factor at ±10%. It's not unusual for vendors to take advantage of this allowance, and employers are often not aware that additional factor is being shipped. This can cause waste as the patient now has an extra 750 units (250 units X 3) sent to him every week. If the prescription was followed at 2,500 units without the added 10%, the patient would have all the clotting factor he or she needs.

This process has a significant financial impact. Consider the price tag of the case above:

The average cost for clotting factor per unit = $1.30

2,500 units x $1.30 = $3,250 x 3 vials/week x 52 weeks = $507,000

10% % waste (needed to control the patient's hemophilia)

250 units x $1.30 = $325 x 3 vials/week x 52 weeks = $50,700 (added "waste")

- Now try adding your information in each box above to see the financial impact.
- Or click here to download an Excel spreadsheet with this calculator.

Calculate

Reset Form

Prescription data is the actual prescription that sets the targeted dose to be dispensed. Employers and many payers do not traditionally have access to prescription data. However, they can and should require vendors to collect this information. Having actual prescription data provides the payer with transparency to verify the number of units dispensed, per unit cost and assay management.

Per unit prices differ among specialty/dispensing pharmacies. HTCs are recognized by the federal government and have access to discounted medications under the 340B Drug Pricing Program, which may offer competitive and/or lower average pricing per unit.

The first step is to determine what specialty drug(s) is prescribed. Refer to the Products Licensed for the Treatment of Bleeding Disorders National Drug Code Directory. The prescribed dose is written as #units per kg of a patient's weight. A patient's weight is converted to kg by dividing weight by 2.2. The target dose is #units ordered multiplied by weight in kg. A dosing schedule should be included with the prescription.

Collect Prescription Data

STEP 5

Data Collection Template

A template has been developed to assist with compiling data necessary to better understand how products are being administered. The template can be accessed here: Sample Data Collection Template

2. Obtaining Historical Claim Reports

Gathering claims information will help to evaluate the effectiveness of an existing program, which could lead to plan design changes. Experts from the NBDF and Hemophilia Alliance are available to assist in this evaluation.

Employers and/or payers should work with their medical claims administrator and PBM and/or the appropriate point of contact for specialty pharmacy network benefit design to identify the following:

- How clotting factor procurement and administration are billed under medical and/or pharmacy benefits; this is important for tracking utilization and claims experience to monitor program effectiveness
- Which specialty pharmacy network strategy is in place (exclusive, open); knowing this will help evaluate whether other strategies should be considered to better manage quality and the cost of care
- Whether contracted specialty pharmacies dispensing hemophilia clotting factor products adhere to MASAC #188 guidelines; these guidelines create a standard for pharmacy care and service when dispensing to members with hemophilia
- Which type of specialty pharmacy providers are contracted; this allows for evaluation of services and cost of each pharmacy provider
    - PBM/commercial specialty pharmacy
    - HTC integrated specialty pharmacy
    - Home care company
- If a co-pay accumulator adjustor program has been deployed; this program is not recommended for use with high-cost/high-value drugs which have no generic equivalents (doing so may have unintended, costly consequences)

STEP 5 KEY POINTS

Dispensing pharmacies choose the vial size(s) closest to the patient's prescribed dose, which can lead to over-shipping of product and excess cost to the employer.

Having actual prescription data provides the payer with transparency to verify the number of units dispensed, per unit cost and assay management.

Gathering claims information will help to evaluate the effectiveness of an existing program, which could lead to plan design changes.

Experts from the National Bleeding Disorders Foundation and Hemophilia Alliance are available to assist.

Analyzing Specialty Pharmacy Prescription and Dose Management Performance

STEP 6

1. Target vs Actual Dispense (Assay) Review

As stated previously, hemophilia treatment is focused on replacement of clotting factor – having the right amount of clotting factor at the right time for the patient – and patient adherence to a personalized treatment plan. Because the specialty medications used to treat hemophilia are biologic drugs, prescriptions are written in a manner that does not follow traditional drug dosing. In addition, pharmacies may dispense extra doses for breakthrough bleeds. If the patient already has extra doses on hand, sending additional doses is wasteful and costly. The management of clotting factor, known as assay management and dose/adherence management, can help to reduce waste and avoid unnecessary health care costs.

Assay management, an important element of cost containment, requires rigorous oversight and reporting, which should be expected from any dispensing specialty pharmacy. Assay management is the process of filling a prescription as closely to the prescribed target dose as possible using one or more available clotting factor vials. Vials come in a range of unit or assay sizes. Factor pricing can vary significantly among dispensing pharmacies for the same product. Providers write prescriptions based on units per kilogram (U/kg) of patient weight. Tightly monitoring how much factor medication is dispensed through the specialty pharmacy helps an employer to reduce waste and better manage costs.

The NBDF's Medical Scientific Advisory Council recommends that factor should be dispensed within ±5% to ±10% of the prescribed target dose. Payers and employers can and should require tighter assay management; in most cases, ±1% to ±2% of the target dose can be achieved. More information can be found by reviewing the MASAC Standards and Criteria for the Care of Persons with Congenital Bleeding Disorders.

2. Deviation from Contracted Performance (if Applicable)

Conduct a retrospective review to ensure that your vendor partners are doing what they say they are doing and require quarterly reports with specific requirements, including the following:

- Assay management performance reviews
    - Identify target dose as written compared to actual dispensed dose
    - Identify current contracted allowable ± over target

Analyzing Specialty Pharmacy Prescription and Dose Management Performance

STEP 6

3. Dose Management

Similar to step 2 above, conduct a retrospective review to ensure that your vendor partners are doing what they say they are doing and require quarterly reports with specific requirements, including the following:

- Dose management performance reviews
    - Ordered versus shipped oversight/reporting to confirm that dispensations match shipments
    - Patient bleed logs (collected and reviewed) to determine medication adherence and inventory on hand at the patient's home

4. Auto Shipping Disabled

Similar to steps 2 and 3 above, a retrospective review to ensure that your vendor partners are doing what they say they are doing and require quarterly reports with specific requirements, including the following:

- Whether auto-shipping has been disabled. The patient and pharmacy must have contact by phone, email, or text to refill the prescription and verify doses on hand. For further information refer to MASAC – Emergency Factor Doses.

For all of the above steps, NBDF offers support to employers at no cost.
A consultant/broker may also be able to conduct a retrospective review. Be sure to ask if any fees will be charged for this before starting.

5. Transparency Requirements

Employers and payers who have experience with hemophilia in their covered populations recognize the high costs involved. However, they may not recognize that treatment involves a class of therapeutic drugs that is not highly managed. Approximately 90% of the total cost of hemophilia is related to specialty drug spend, however adequate data and information on these claimants are not routinely provided by PBMs and insurers. For payers and employers to effectively manage their hemophilia spend, they need vendors to be transparent and provide the right information and data. The key is to hold vendors accountable for managing use and spend. One way to accomplish this is by requiring quarterly reports that include agreed upon standard data and metrics.

6. Case Studies with Cost Savings Examples

The case studies listed below were conducted by NBDF, Hemophilia Alliance, self-funded employers and their insurance consultants/brokers. The results highlight the value and benefits of the HTC model for clinical care and integrated HTC pharmacy services, and the benefits of online tools for improving the hemophilia management process.

Analyzing Specialty Pharmacy Prescription and Dose Management Performance

STEP 6

Case Study

A patient with mild hemophilia A and a high-titer inhibitor (more inhibitor present in the blood) was being treated outside of an HTC and was receiving clotting factor from a specialty pharmacy prior to transitioning to an HTC. The patient requested to receive treatment, which included factor VIII and factor VIIa, from the HTC integrated pharmacy after transitioning care.

- The specialty pharmacy dispensed factor between 5% to 10% above target
- HTC's assay management intervention resulted in dispensing at 1% to 4% below target; HTC dispensed factor VIII and factor VIIa at $0.26 and $0.60 lower per unit than the specialty pharmacy*

Outcomes:

- Per-unit price differences resulted in $287,000 per month in cost-savings for the patient's health plan.
- An additional $460,000 in savings for the plan came from precise management of clotting factor fills and expert medical management.

Case Study

A patient covered by a self-funded employer was receiving clotting factor through a specialty pharmacy. The development of an inhibitor necessitated a therapeutic switch to a non-factor therapy that was priced higher. At the same time, the patient's care was transferred to the local HTC to more carefully manage the complications associated with the inhibitor.

Outcomes:

- Despite the switch to a higher-cost therapy, lower per-unit pricing from the HTC integrated pharmacy model afforded a $1M cost-savings projected over three years and a cost-savings of $1.7M projected over five years
- Savings were a result of per-unit cost only; additional savings could be realized through HTC-guided assay management and other ancillary services delivered as componentsof the comprehensive care model

*Pricing varies across HTCs. Each HTC negotiates own contracts.

Analyzing Specialty Pharmacy Prescription and Dose Management Performance

STEP 6

Case Study

A patient with severe hemophilia B was prescribed 55u/kg (90.9 kg) of factor IX CFR with a target dose of 4,999.5 units. The patient followed a dosing schedule of 3x/week with PRN doses for breakthrough bleeds and received factor at a cost of $1.35 per unit through his self-funded employer's specialty pharmacy. An analysis showed that factor could be provided through the integrated pharmacy of the patient's local HTC at a cost of $1.08 per unit.* This difference in per-unit pricing saved the employer $273,559 annually by switching from the specialty pharmacy to the HTC for factor dispensation.

Outcomes:

- The HTC's rigorous assay management yielded an additional $46,656, resulting in a cumulative $320,215 in HTC-derived savings.

STEP 5 KEY POINTS

Assay management, an important element of cost containment, should be expected from any dispensing specialty pharmacy.

Conduct a retrospective review to ensure vendor partners are doing what they say they are doing and require reports with assay and dose management reviews and auto shipping disabled.

To effectively manage hemophilia spend, vendors need to be transparent and provide the right information and data.

Significant cost savings have been achieved from real world case studies.

*Pricing varies across HTCs. Each HTC negotiates own contracts.

Total Cost of Care Analysis

STEP 7

1. Run Claims Report to capture ER and Hospital Admissions (with primary admission related to Hemophilia)

Ask the plan administrator to pull all ER and hospital claims data with hemophilia as principal diagnosis (codes provided in step 3) for total cost of care analysis and identification of any potential obstacles that may be creating barriers to home management.

NBDF offers support to employers at no cost to assist with this analysis if needed.

References

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4 Hemophilia: Treatment Centers. Centers for Disease Control and Prevention website. https://www.cdc.gov/ncbddd/hemophilia/htc.html.
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9 2018 Sun Life Stop-Loss Research Report. Sun Life Financial website. https://sunlife.showpad.com/share/ozxiXOJKGdZv27oVCFib6. Accessed November 2020.

10 Adherence to Long-Term Therapies. World Health Organization. Published 2003. Accessed November 2020.

11 National Hemophilia Foundation. Statement delivered to the Subcommittee on Labor, Health, and Human Services; Education and Related Agencies, Committee on Appropriation U.S. House of Representatives. April 1990.

Find Additional Tools and Resources STEP 8

This section contains relevant and up-to-date resources to help employers/payers with the issues and challenges faced when creating a strategy for managing bleeding disorders in a covered population.

For Employers/Payers

Research/Reports

High-Cost Claims and Injectable Drug Trends

Sun Life Financial Stop Loss Research Reports

- 2022 Report
- 2023 Report

Hemophilia: Treatment Landscape, Costs and Disease Management
AJMC, April 26, 2018

Hemophilia Burden of Disease: A Systematic Review of the Cost-Utility Literature for Hemophilia
Journal of Managed Care & Specialty Pharmacy, July 2018

Hemophilia Management via Data Collection and Reporting: Initial Findings from the Comprehensive Care Sustainability Collaborative
Journal of Managed Care & Specialty Pharmacy, January 2017

Treatment Adherence in Hemophilia
Patient Preference and Adherence, September 2017

Burden of illness: Direct and indirect costs among persons with hemophilia A in the US
Journal of Medical Economics, June 2015

An Actuarial Study of Hemophilia: Implications for Commercial and Medicaid Managed Care Plans
Milliman Inc., October 2013

Population-based surveillance of haemophilia and patient outcomes in Indiana using multiple data sources
Haemophilia: The Official Journal of the World Federation of Hemophilia, March 2019

Hemophilia: Articles & Key Findings
Centers for Disease Control

Find Additional Tools and Resources STEP 8

National Bleeding Disorders Foundation (NBDF)

- Home page, National Bleeding Disorders Foundation (NBDF)
- Home page, Comprehensive Care Sustainability Collaborative (CCSC)
- Comprehensive Hemophilia Management: A Payer's Guide to the Hemophilia Comprehensive Care Model
- Medical & Scientific Advisory Council (MASAC)
- Managed Care Hemo: Resources, provided by the NBDF
- National Bleeding Disorders Foundation (NBDF) on Accumulator Adjustment Programs (CCSC)
- Hemophilia Response Pathway for Employers
- Video: Hemophilia Treatment Centers: The Value of the Integrated Care Model in Creating Positive Outcomes

Hemophilia Alliance

Overview: What We Do

Services and Tools

- HTC Operations and Organizational Management
- HTC Group Purchasing
- Payer Relations
- Advocacy
- Legal & Compliance

Hemophilia Federation of America

- Home page
- Word from Washington
- Free Resources for Patients & Families

Find Additional Tools and Resources

STEP 8

Centers for Disease Control & Prevention (CDC)

Data and Statistics on Hemophilia

Hemophilia Treatment Centers (HTCs)

Hemophilia Treatment Centers: Directory

Do the 5! Awareness campaign funded by the CDC focusing on five strategies to help people with hemophilia live a longer and healthier life

Free Educational Materials on Hemophilia

For Members & Dependents

National Bleeding Disorders Foundation (NBDF)

- Bleeding Disorders
- Community Resources

Hemophilia Federation of America

- Understanding Bleeding Disorders: Bleeding Disorders 101
- Complications: Joint Damage

Centers for Disease Control (CDC)

- Hemophilia homepage/basics
- Hemophilia Treatment Centers Directory
- Inhibitors and Hemophilia

National Heart, Lung and Blood Institute

- Hemophilia

Find Additional Tools and Resources

STEP 8

Mayo Clinic

Hemophilia Overview

MedlinePlus

Hemophilia

National Organization for Rare Disorders

Hemophilia A

Hemophilia B

World Federation of Hemophilia

Home page

Research & Data Collection

Find Additional Tools and Resources

STEP 8

For Parents & Caregivers

KidsHealth

- For Parents: Hemophilia

National Bleeding Disorders Foundation (NBDF)

- Community Resources/Chapter Directory
  For help connecting with other families affected by bleeding disorders
- Locate a Camp Near You
  Directory of camps for children with bleeding disorders (or sibling/family)

Hemaware: The Bleeding Disorders Magazine, NBDF

- Care for the Caregiver
- Raising a Child with Hemophilia: A Practical Guide for Parents

Hemophilia News Today

- Living with Hemophilia: When your child has hemophilia

Hemophilia Federation of America

- Challenges: Emotional and Psychological

For Further Assistance or to Learn More, Contact: ccsc@impactedu.net

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