

# Addressing the Needs of Members with Hemophilia in Medicaid Managed Care: Issues and Implications for Health Plans

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Clinical Brief  
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## Executive Summary

Hemophilia is a rare, inherited blood disorder affecting approximately 20,000 individuals in the United States. Hemophilia results in the inability of the body to form blood clots, which are required to stop bleeding. People with hemophilia may have uncontrolled bleeding, which often starts spontaneously and can result in permanent damage to major joints. In rare cases bleeding from hemophilia can be fatal. Hemophilia cannot be cured, but it can be treated through replacement of clotting factors to prevent bleeding. Proper treatment can prevent the long-term consequences arising from repeated bleeding into the joints and surrounding tissues, the leading cause of disability related to hemophilia.

In the U.S., hemophilia care is delivered by highly specialized federally designated Hemophilia Treatment Centers (HTCs), which treat approximately 70% of people with the disorder. HTCs offer comprehensive, multidisciplinary hemophilia services. HTCs also offer integrated pharmacy services, frequently through a “340b pharmacy.” Because HTCs treat a great volume of patients, they are able to develop expertise in management of the condition, which is needed to prevent complications. Pharmacy services are essential to ensure timely and appropriate dosing of clotting factor agents, a critical aspect of care for people with hemophilia. Many people with hemophilia infuse clotting factor at home, either preventively or in response to bleeds. Factor products and home infusion services can be delivered by specialty pharmacies, 340b pharmacies, and specialized pharmacy benefit managers.

Hemophilia treatment can be extraordinarily expensive—sometimes ranging up to a million dollars per individual—and unpredictable. The high cost of care, together with the highly specialized treatment system required for people with hemophilia, presents unique contracting, care management and financing considerations for health plans. This Issue Brief examines issues relating to health care delivery and financing specifically for individuals with hemophilia who current are or may be enrolled in state Medicaid programs.

To date, most Medicaid health plans report having few enrolled members with hemophilia. With the implementation of the Affordable Care Act, this may change. Medicaid health plans can expect to see greater numbers of people with hemophilia over the coming years as a result of Medicaid program expansion, enrollment of more men, and the move from fee for service into managed care for disabled and dual eligible populations. Medicaid health plans need to anticipate the needs of members with hemophilia. Keys issues are:

- Treatment Access and Quality: Health plans should anticipate hemophilia care in network management and medical management strategies, to ensure access to specialized medical and pharmacy providers. This includes contracting or developing agreements with Hemophilia Treatment Centers.
- Care Management: Health plans should consider how best to coordinate multi-disciplinary outpatient and home-based services for members with hemophilia. Patients served in HTCs may have care coordination services from the HTC. Health plans should determine what oversight and additional care coordination are needed and clearly designate accountability.
- Cost Management: Plans need to consider factor pricing and cost effective approaches for administration of costly factor replacement products, while keeping in mind the individualized treatment needs of children and adults with hemophilia.
- Pharmacy Management: Plans should evaluate the full spectrum of services required to manage hemophilia, and contract with the most appropriate pharmacy to provide cost effective and timely factor replacement services for routine and emergency needs.

## Hemophilia in Medicaid Managed Care - MHPA Issue Brief

- Risk Adjustment and Risk Management: Plans may need to work with the advocacy community and states to anticipate enrollment of members with hemophilia. These stakeholders can proactively recommend financing solutions to ensure member access to appropriate care; this may include risk adjustment or carve outs to avoid risk selection adversely impacting plans and members.
- Patient Involvement: Plans need to include members in decisions impacting their care, support member involvement in self-management and facilitate social support networks.

As Medicaid health plans prepare for expansions, it is important to work with state policy makers to plan for the needs of members with rare disorders. For members with hemophilia, access to specialized treatment and lifesaving therapy are essential elements of quality care.

## **Addressing the Needs of Members with Hemophilia in Medicaid Managed Care: Issues and Implications for Health Plans**

### **Contents**

Executive Summary.....	2
Introduction .....	5
Part 1: About Hemophilia .....	6
Sources of Care for Patients with Hemophilia .....	7
Hemophilia Treatment.....	8
Treatment Guidelines .....	9
Factor Replacement .....	10
Inhibitors .....	100
Complications.....	11
Sidebar: Hemophilia Treatment Guideline Excerpt .....	12
Part 2: Hemophilia and Medicaid .....	13
Hemophilia and Medicaid Managed Care .....	14
Treatment Access and Quality .....	16
Care Management .....	17
Cost Management.....	18
Pharmacy Management.....	19
Risk Adjustment .....	21
Patient Involvement.....	22
Conclusion.....	22
Part 3: Resources .....	24
Federal Organizations .....	24
Advocacy Organizations.....	24
Hemophilia Guidelines.....	25
State Hemophilia Program Examples.....	25
Health Plan Resources .....	26
Publications Relevant to Medicaid Health Plans .....	26
References .....	27

## **Addressing the Needs of Members with Hemophilia in Medicaid Managed Care: Issues and Implications for Health Plans**

### **Introduction**

Hemophilia is a rare, inherited blood disorder affecting approximately 20,000 individuals in the United States. Hemophilia results in the inability of the body to form blood clots required to stop bleeding. People with hemophilia may have uncontrolled bleeding, which often starts spontaneously and results in permanent damage to major joints. Joint damage is a common and disabling complication of hemophilia. In rare cases hemophilia bleeds can be fatal. Hemophilia cannot be cured, but it can be treated through replacement of clotting factors to prevent bleeding. Proper treatment can prevent the long-term co-morbidity and disability resulting from repeated bleeding into the joints and surrounding tissues.

This Issue Brief examines issues relating to health care delivery and financing specifically for individuals with hemophilia who are or may be enrolled in state Medicaid programs. Hemophilia treatment can be extraordinarily expensive—sometimes ranging up to a million dollars per individual—and unpredictable. Approximately 70% of people with hemophilia are treated in highly specialized federally designated Hemophilia Treatment Centers. These Centers offer comprehensive services and access to specialists tailored to the unique needs of people with hemophilia.

The high cost of care, together with the highly specialized treatment system required for people with hemophilia, presents unique contracting, care management and financing considerations for health plans. To date, most Medicaid health plans report having few enrolled members with hemophilia. This is changing. Medicaid health plans can expect to see greater numbers of people with hemophilia as the Medicaid program expands overall, more men are enrolled, and more disabled and dual eligible members move from fee for service into managed care. Health plans need to anticipate how to address the needs of these members.

This Issue Brief is based on literature and discussions with patient advocates, Hemophilia Treatment Centers, and health plans. The Brief addresses care management, policy, and payment alternatives to ensure that members with hemophilia have access to high quality treatment, facilitated and promoted in a sustainable financing system for patients, health care providers and health plans. Although

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CVS / Caremark

Perform RX

Hemophilia Treatment Center of Western Pennsylvania

Region IX HTC / UCLA and The Center for Inherited Blood Disorders

Region V HTC / Hemophilia Foundation of Michigan

Blue Cross Blue Shield of Michigan

Coventry Health Care, Inc.

National Hemophilia Foundation

UPMC for You

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hemophilia has unique treatment needs, the strategies, relationships, and payment considerations may also be relevant to other rare diseases.

## Part 1: About Hemophilia

Hemophilia is caused by a genetic mutation resulting in the inability of the body to produce clotting factors needed to form blood clots that stop bleeding. It is primarily an inherited bleeding disorder, although it results from spontaneous mutation of a gene in about one third of cases (meaning there is no prior family history).<sup>1</sup> Because it is linked to the X-chromosome carried by females, it is passed from female carriers of the gene to their male children. It affects roughly 1 in 10,000 live births or 1 in 5000 male births.<sup>2</sup> Hemophilia is almost exclusively expressed in males. Female carriers may have a very mild form of the disease.<sup>3</sup>

The two most common types of hemophilia are A and B. Patients with hemophilia A have a deficiency or altogether lack of clotting factor VIII. Hemophilia A comprises 80-85% of the population with hemophilia. People with Hemophilia B make up the remaining 15-20%. Individuals with hemophilia B lack or are deficient in clotting factor IX.\* Treatment entails replacement of clotting factor. This requires infusions of clotting factor on a regular basis, both as a prophylactic measure and to stop acute bleeding incidents. Regular “assay testing” determines the level of endogenous factor in the body, and guides dosing of replacement factor.

Hemophilia is expressed in a range of severity. The majority of patients under treatment have the severe variation of the disorder, defined as a level of Factor VIII or IX of less than 1% of the normal standard.<sup>4</sup> Patients with severe hemophilia bleed spontaneously and have frequent or severe bleeds. Individuals with mild or moderate hemophilia rarely experience spontaneous bleeding. As such they may not be identified unless they encounter an acute event such as surgery or an accident.

Hemophilia is present at birth; those with severe hemophilia are frequently diagnosed as infants, often following a bleeding episode related to circumcision. The age of diagnosis of hemophilia tends to vary based on severity, and based on whether there is a family history. Those with mild or moderate hemophilia may not be diagnosed until adulthood. According to the Centers for Disease Control and Prevention (CDC), children with severe hemophilia on average are diagnosed at one month, while those with mild hemophilia have a median age of 36 months at diagnosis.<sup>5</sup>

Since hemophilia is a genetic disease, it can affect siblings, cousins, grandparents, and other blood relatives within an extended family. Testing of children is recommended if there is a family history of hemophilia or if the mother is a known genetic carrier. Genetic counseling and testing is recommended when there is a family history of the disease.

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\* Factor VIII and IX are essential for blood coagulation. In this Issue Brief Factor VIII and Factor IX are collectively referred to as “factor.” Factor may be produced by concentrating human plasma or synthetically through recombinant technology. Recombinant factor eliminates the potential for transmission of other diseases such as HIV and HCV, which are a risk of plasma based factor production. Although the mechanism is not well understood, individuals may respond differently to different factor products, and the risk of inhibitor development may go up when factor products are changed. For this reason national organizations such as the National Hemophilia Foundation recommend that factor not be restricted on a state or plan formulary. (National Hemophilia Foundation. “ MASAC Recommendations Regarding Factor Concentrate Prescriptions and Formulary Development and Restrictions. Document # 159, 2005)

## Sources of Care for Patients with Hemophilia

Hemophilia is managed by hematologists specializing in bleeding disorders. A small subset of hematologists has developed the expertise and experience to provide the comprehensive care required by patients with hemophilia. The rareness of disease means that the majority of providers – even hematologists – do not treat the volume of patients required to develop expertise. For this reason a highly customized delivery system for hemophilia care has been created through federal funding.

Hemophilia Treatment Centers (HTCs) are required to offer comprehensive, multi-disciplinary hemophilia services. Because the centers treat relatively large numbers of hemophilia patients, they develop expertise in management of the condition to prevent complications. An estimated 70% of hemophilia patients are cared for by HTCs.<sup>6</sup> The population served by HTCs, particularly the children population, is growing.<sup>7</sup>

The federal Health Resources and Services Administration (HRSA) funds the HTC program through eight regional specialty centers—grantees that provide leadership, direction and monitoring for a network of 130 nationally recognized hospitals and academic medical centers throughout the U.S. and several territories. This structure ensures access to highly specialized care that might otherwise not be available. It also establishes systematic oversight for achieving federal goals and national comprehensive care guidelines.<sup>8</sup> . HTCs may provide services through remote consultations for rural areas, or be the designated hemophilia center for a state other than the one in which they are located.

HTCs arrange for or provide specialty services required for management of hemophilia, including preventive services, home infusion, and emergency care. Specialized care coordinators coordinate multidisciplinary home and center-based services with patients. Services under the umbrella of HTCs include:

- Coagulation Laboratory: initial diagnosis and ongoing monitoring
- Physician Services: Hematology, pediatrics, and orthopedics
- Nursing Services: education, anticipatory guidance, home infusion teaching
- Physical Therapy
- Nutrition
- Dental services
- Social work /Social Support
- Research: clinical trials of new therapies
- Surveillance: nationwide monitoring of blood disorder complications funded by the Centers for Disease Control and Prevention
- Nationwide hemophilia registry through the American Thrombosis and Hemostasis Network

According to the Hemophilia Alliance, 105 HTCs participate in the federal 340b Drug Discount Program. The HRSA-managed 340b program allows eligible entities, including HTCs, to purchase outpatient pharmaceuticals at a discounted rate. The 340B program requires that the savings (in the form of revenue) to be used to serve more patients and expand the scope of services offered. Most HTCs include integrated pharmacy services in their comprehensive hemophilia care programs and provide for or arrange infusion services in the patient's home. Pharmacy services can also be provided through non 340b independent sources such as specialty pharmacy providers.

As the case example from the Hemophilia Center of Western Pennsylvania illustrates, many HTCs have used 340b program income to cover the specialized, care coordination services and patient education in self-infusion provided by the HTC. Some states, such as Massachusetts have negotiated enhanced dispensing fees for HTCs specifically to cover enhanced hemophilia service delivery. Contracting issues are discussed in greater detail further in this brief.

The Centers for Disease Control and Prevention (CDC) Division of Blood Disorders also has a role in hemophilia care. CDC is charged with maintaining safety of the blood supply. CDC provides funding to HTCs for research, surveillance and prevention relating to blood disorders. Much of the research on hemophilia care and outcomes is now generated through HTCs, partially supported by CDC and HRSA funds. Through the partnership with CDC, HTCs maintain the Universal Data Collection System for Blood Disorders (UDC) which provides much of the information on epidemiology and health services utilization for hemophilia.

## Hemophilia Treatment

People with hemophilia can have a near normal life expectancy. Thus, prevention of disability is a critical treatment priority. Advances in preventive treatments mean that children born with hemophilia can now prevent the joint bleeds that previously have led to co-morbidities and serious disability.

Hemophilia care focuses on prevention of bleeding and immediate infusion of clotting factors if excessive bleeding does occur. As noted, individuals with hemophilia are at high risk of internal, muscular, and joint bleeding. Patients can also experience prolonged bleeding following trauma or surgery. Bleeds in some areas of

### **The Hemophilia Center of Western Pennsylvania Partnership with Medicaid Health Plans**

The Hemophilia Center of Western Pennsylvania contracts with four Medicaid health plans, Medicare, and several commercial health plans. The Hemophilia Center of Western Pennsylvania provides comprehensive medical, nursing and psychosocial services to persons with hemophilia and other bleeding and clotting disorders residing in Western Pennsylvania. The Center's pharmacy services are limited to the distribution of clotting factor and related supplies only. The Center's goal is to provide patient care resulting in reduced morbidity, mortality, hospitalizations and emergency department visits for people with hemophilia.

The Center is the designated provider for members with hemophilia and other blood disorders for UPMC for You, a Medicaid managed care plan. UPMC for You contracts for physician and clinical nursing services and clotting factor from the Hemophilia Center of Western Pennsylvania. UPMC's clinical team indicates that the Center has the lead on coordinating medical and pharmacy services for UPMC for You members. UPMC's clinical leadership teams meet periodically with the Center's leadership to address additional care management issues.

The Center has diverse contracting arrangements with Medicaid and commercial health plans for medical and pharmacy services. Three Medicaid managed care plans other than UPMC for You contracting with The Center also use physician and nursing services, but receive their clotting factor from independent pharmacies. Jeff Wahal, the Center's Executive Director, notes that the Center does not bill for its psychosocial services, physical therapy and genetic counseling that are also provided to the Center's patients. Funding for the Center's psychosocial services, physical therapy and genetic counseling is obtained through federal and state grants and 340B program income.

the body—such as the head and throat—can be life-threatening. Treatment for hemophilia requires ongoing replacement of clotting factors, enabling blood to clot properly and thus stop bleeding.

There have been major advances in treatment and prevention of bleeds, including the development of recombinant factor replacement biologicals, and the use of prophylactic (preventive) factor infusion protocols when necessary. For all hemophilia patients, treatment and ongoing disease management through the lifespan are needed.

Priorities in care management include:

- Accurate Diagnosis and Assay Testing: Coagulation labs are needed to ensure correct diagnosis, to conduct ongoing testing to evaluate endogenous factor levels which guide weight-based dosage, and to gauge adequate hematologic dosing during elective and emergency surgery. HTCs typically offer these specialized coagulation laboratory services.
- Factor Management: Patients with hemophilia need expert treatment and dosing of prophylactic factor on a routine and emergent basis. This entails coordinating with multiple specialty providers (medical and pharmacy) and laboratories as well as with home care services.
- Assay Management for Dose Monitoring: Factor testing is conducted regularly to evaluate endogenous factor levels. Current patient weight must be obtained regularly to guide weight-based dosage.
- Management of Hemophilia Complications: Patients require access to specialists with expertise in managing hemophilia patients. Specialized services include orthopedic services, physical therapy and rehabilitation, and HIV / AIDS care. In addition, anticipatory assistance is needed. Routine health issues that might generate bleeding – such as dental care, elective surgery, or falls – are much more complex for a person with hemophilia.
- Management of Chronic Conditions: As people with hemophilia are living longer, they are now experiencing chronic conditions associated with aging, including cardiovascular disease. People with hemophilia require the same cardiovascular disease prevention and treatment services as the general population.
- Social Support: Members with hemophilia can expect to have substantial responsibility for home management of factor infusions and a lifetime of careful management. Although children with hemophilia can participate in mainstream school activities, there are some restrictions on sport and other activities. It is critical that members with hemophilia (and their caregivers) are linked to social support, particularly patient groups.

### **Treatment Guidelines**

The World Federation of Hemophilia has developed treatment guidelines for hemophilia.<sup>9</sup> These evidence-based guidelines address parameters for infusion of factor replacement products, as well as emergency care, complications, and preventive services. The National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) has also developed recommendations regarding clinical practice, treatment guidance and delivery of care, which are followed by the HTCs and other providers of hemophilia care.<sup>10</sup> There are some differences in the treatment recommendations of the WHF and the NHF MASAC. Hemophilia experts stress that treatment response on hemophilia is highly individualized, necessitating expert clinical judgment to ensure patients receive the treatment resulting in the best response.

## Factor Replacement

Clotting factor must be infused intravenously (IV), and can be provided either in response to a bleed, or preventively. Dosing of clotting factor is weight based, but dosage may vary quite substantially among individuals due to variations in metabolism of the factor. Patients may also respond differently to different brands of factor replacement. Patients with moderate to severe hemophilia and who require regular infusions are often taught to administer factors intravenously at home.

There are two approaches to replacing missing clotting factors: preventive replacement (called prophylaxis) and as needed replacement (called on-demand). The reactive or “on-demand” treatment approach calls for the administration of clotting factor at the time of an acute bleed. On demand therapy is more commonly used for patients with mild-moderate hemophilia or for members who do not wish to infuse factor preventively.

Prophylaxis plays a critical role in preventing bleeding episodes and the associated hemophilic joint diseases—particularly for children.<sup>11, 12</sup> According to the WFH, the goal of prophylaxis is to preserve normal joint function. The WFH’s prophylactic treatment strategy calls for administration of clotting factor on a regular schedule – either two or three times a week, depending on the type and severity of hemophilia, with the goal of maintaining an endogenous level of clotting at approximately 1% of normal levels. The National Hemophilia Foundation recommendations are somewhat different. Dosing is based on weight of the patient; regular testing evaluates the patient’s level of clotting factors and tests for the presence of inhibitors. Adherence to prophylactic administration can drop off as patients age<sup>13</sup> or with changes in insurance status.

For children, prophylaxis enables improved psychosocial well-being such as participation in physical activities and school attendance. Approximately 80% of children with severe hemophilia treated in Hemophilia Treatment Centers receive prophylaxis, generally through home infusion.<sup>14</sup>

### Definitions of Factor Replacement Therapy Protocols (Excerpted from WHF Guideline 2013)

**Episodic:** treatment given at the time of clinical evident bleeding

**Primary prophylaxis:** regular continuous\* treatment initiated in the absence of documented osteochondral joint disease, determined by physical exam and / or imaging studies, and started before the second clinically evident large joint\*\* bleed and age 3 years

**Secondary prophylaxis:** regular, continuous treatment started after 2 or more bleeds into large joints and before the onset of joint disease documented by physical examination and imaging studies

**Tertiary prophylaxis:** regular continuous treatment started after the onset of joint diseases documented by physical examination and plain radiographs of the affected joints.

**Intermittent (periodic) prophylaxis:** treatment given to prevent bleeding for periods not exceeding 45 weeks a year.

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\*Continuous is defined as the intent of treating for 52 weeks per year and receiving a minimum of an a priori defined frequency of infusions for at least 45 weeks (85%) of the year under consideration.

\*\*Large joints = ankles, knees, hips, elbows and shoulders.

## Inhibitors

An ‘inhibitor’ is the result of antibody formation that interferes with effectiveness of infused clotting factor. According to the World Federation of Hemophilia, inhibitor formation occurs at some point in 20-30% of patients with severe hemophilia A, and in 5-10% of those with mild to moderate hemophilia A.<sup>15</sup> Inhibitors occur in only about 5% of patients with hemophilia B. At any given time of between 5-7%

of patients have inhibitors.<sup>16</sup> The underlying causes for development of inhibitors are not completely understood. Inhibitor development is thought to be influenced by a variety of risk factors including age, type of hemophilia, product, and frequency of infusion.<sup>17</sup>

According to the Centers for Disease Control and Prevention, treatment of inhibitors can be through infusion of a greater volume of factor replacement, use of “bypassing agents,” or induction of “immune tolerance” to enable response to factor replacement.<sup>18</sup> In addition to treating the inhibitors, practitioners also need to treat any bleeding that occurs while the patient is low on clotting factors. People with inhibitors temporarily require significantly larger doses of factor, and may also require additional biologics to achieve acceptable clotting.<sup>19</sup> Inhibitors may resolve with proper treatment, but when present, they greatly increase patient treatment needs and costs. Patients with inhibitors present care management challenges as they require highly specific and closely monitored dosing, and may have side effects, hospitalizations and other co-morbidities.

### **Complications**

Arthropathy (joint damage) is a common co-morbidity of hemophilia resulting from bleeding into joints. It is a painful, degenerative condition. Arthropathy can result in contracted limbs, and is a primary source of hemophilia-related disability. Prevention of bleeds into joints is a primary goal of treatment.<sup>20</sup> Patients that do experience joint bleeds require immediate treatment to remove the blood from the joints, followed by physical therapy to prevent joint damage. In the longer term, patients with hemophilia often require joint replacements to restore mobility.

Physical therapy is required to prevent contractures and long term disability resulting from joint bleeds and is used much more extensively for members with hemophilia than in the general population. As with all providers of hemophilia services, physical therapy practitioners must have expertise in the unique requirements of hemophilia. This ensures appropriate joint management and reduces risk of exacerbating joint damage.<sup>21</sup>

In the 1980s before HIV testing of the blood supply, many people with hemophilia were infected with HIV. Thus older patients with hemophilia may also require treatment for HIV or hepatitis. Due to improvements in the blood supply, this is not the case for patients born after 1985. Patients using plasma derived factor replacement products are still at risk for Hepatitis A, B and C. As a result, many patients with hemophilia need access to liver specialists with expert knowledge of both hemophilia and hepatitis.



## Part 2: Hemophilia and Medicaid

Approximately a third of people with hemophilia are covered through states Medicaid programs.<sup>22 23</sup> States are increasingly turning to managed health care plans as an alternative to fee for service to serve beneficiaries in the Medicaid program. Managed care plans offer the state more predictable costs through risk adjusted capitation agreements. When using managed care, states have the option to “carve-out” some services or types of beneficiaries if the state wants greater control of the service.

When Medicaid beneficiaries are served in managed care, health plans are required to meet state expectations for ensuring enrollee’s access to care and quality of care. For services that are “carved in,” the health plan is accountable for ensuring access and paying for the service. Health plans typically contract with health care providers and other vendors to deliver the care. Plans also assume the administrative functions of managing vendor contracts such as with home health agencies, pharmacies, and durable medical equipment suppliers. Services that are “carved out” are not managed by the health plan, and the plan has no financial responsibility for them. Health plans are subject to state financial and quality oversight. They typically report clinical quality and operational performance metrics to the state on a regular basis.

Several states have enacted special provisions to help manage the high cost of hemophilia care in Medicaid, often by separating out coverage for pharmaceutical and medical care. Hemophilia services that are “carved out” by Medicaid are generally paid on a fee for service basis and are arranged independent of health plan services. Some states also address quality and coverage gaps for hemophilia care. While there is diversity in state approaches based on the Medicaid eligibility category of the patient, typically, states have approached hemophilia coverage in Medicaid through one of the following approaches:

- Fee for service: States using fee for service pay for providers and pharmacy services directly for each service delivered. Generally the patient chooses the provider from among those contracted to the Medicaid agency. Many providers are not willing to accept the rates paid by state Medicaid program, which particularly limits access to specialty care providers.
- Managed care with carve in both medical and pharmacy: The carved in approach means that the health plan is accountable for contracting with providers and paying for services for both medical and pharmacy care. Patients are required to enroll in a managed health plan, and the state pays a capitated rate to the plan that should cover both medical and pharmacy costs. This approach has the greatest risk to health plans, as the capitated rate may not account for a patient with hemophilia or a patient with inhibitors.
- Managed care with carve out pharmacy costs: Patients are required to enroll in a managed health care plan, but the costs of pharmaceuticals are not included in the capitated rate. The state may contract with a pharmacy benefits manager directly to manage pharmacy costs or may pay community pharmacists on a “fee for service” basis. The state often receives a financial rebate on the cost of pharmaceuticals.
- Managed care with hemophilia carve out: The state generally uses managed care for the Medicaid population, but members with hemophilia are excluded from the managed care requirement.
- Hybrid: States have the option to carve out just some portion of the care, for example, factor replacement products, while carving-in other services such as other pharmaceuticals and services.

According to the Plasma Protein Therapeutics Association (PPTA, a trade organization of biologic therapy manufacturers), Florida, Kansas and New Jersey carve out hemophilia therapies from Medicaid health plan pharmacy benefits. Florida, Louisiana and South Carolina have stand-alone programs for management of hemophilia therapies.<sup>24</sup> <sup>†</sup> The state of Florida requires Medicaid beneficiaries with hemophilia to enroll in a disease management program with one of two contracted hemophilia specialty pharmacy providers. In addition to securing volume pricing for factor, this approach ensures that specialty pharmacy providers have experience to effectively address needs of member with hemophilia.

New Jersey has enacted comprehensive legislation to ensure access to hemophilia treatment addressing access to necessary materials and supplies for home infusion. New Jersey requires health insurance organizations to cover blood products and home infusion equipment, and to ensure that contracted organizations deliver high quality infusion services and medical waste disposal.<sup>25</sup>

While states continue to wrestle with strategies to effectively finance and manage the financial risk of insuring hemophilia in Medicaid, patient advocacy groups have developed a number of studies and position papers. The National Hemophilia Foundation recommends not including blood clotting factors in Medicaid capitation rates (e.g. carving it out),<sup>26</sup> and has produced an actuarial analysis in at least one state showing the financial risks of doing so. This strategic advocacy was effective in Indiana, where factor costs are carved out of Indiana Medicaid managed care.

## **Hemophilia and Medicaid Managed Care**

Medicaid health plans can expect increased enrollment of members with hemophilia in the coming years. The Medicaid program pays for almost half of all births in the U.S., making it likely that children born with this hereditary disorder may be enrolled in Medicaid health plans at birth.<sup>27</sup> In addition, the majority of Medicaid eligible members with hemophilia currently qualify under disability eligibility rules, a population less frequently served by managed care. As more states turn to health plans to serve disabled and Dual Eligible populations, plans will encounter more members with hemophilia. In addition, the number of Medicaid members with hemophilia is expected to rise in general, as more people, particularly adult men and children, are covered by the Medicaid program.

Medicaid health plans offer the advantage of well-developed care coordination programs and quality reporting systems compared to fee-for-service Medicaid. Yet hemophilia care may present new challenges to health plans. The small number of members coupled with potentially extremely high costs makes it challenging to anticipate these members on an actuarial basis, and challenging to prepare to meet their needs. For example, health plans may have few hematologists in their networks, and fewer who specialize in hemophilia and offer care in conjunction with other multi-disciplinary services required. Health plans will need to proactively work with states to anticipate financing needs for members with hemophilia, and to develop network contracting agreements that ensure member access to comprehensive hemophilia care.

MHPA's inquiries and search for health plan's hemophilia activities found diverse approaches to care. Some Medicaid health plans have no formal program. These plans indicated that they could potentially identify members by high claims costs and address their needs through complex case management

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<sup>†</sup> This is not a comprehensive discussion of state policy, and the state information is not verified by MHPA. State hemophilia policies may have changed.

services. Other health plans have specialized programs for members with hemophilia. United Healthcare, for example, has partnerships in place with Hemophilia Treatment Centers and several specialized pharmacy management organizations. The program enables the organization to expand clinical programs, improve the member experience, and potentially improve the health of members.<sup>28</sup> Similarly, the AmeriHealth Mercy Family of Companies, a multi-state Medicaid managed care provider, has developed a program to coordinate health and pharmacy services with HTCs while providing care coordination oversight internally. (See sidebar on page 19 for more information)

Hemophilia and other rare diseases present unique challenges to Medicaid health plans. These include:

- Treatment Access And Quality: The nature of a rare disease makes it challenging to ensure availability of practitioners with expertise in treating the condition. Network management approaches need to ensure access to multi-disciplinary experts and comprehensive treatment. In addition, medical management protocols need to account for the unique clinical needs for members with hemophilia, for example, through an extended physical therapy benefit.
- Care Management: People with hemophilia have complex needs and may need support coordinating the services of multiple and multi-disciplinary providers, along with tests, home care services, and supplies. Patients served by HTCs have long standing care coordination service relationships provided through the HTC. The health plan's care coordination role may vary depending on whether the member has access to coordinated services from the HTC or other sources. Health plans could conduct their own care management, engage a specialty disease manager, or delegate to a Hemophilia Treatment Center for services.
- Cost Management: The cost of factor replacement products can be extraordinarily high, which can be difficult for health plans to absorb in a capitated pharmacy strategy. Health plans, states, patients and providers need to reach a common understanding of how to manage the catastrophic costs in a manner that preserves appropriate treatment access while protecting the health plan and patient from catastrophic financial exposure.
- Pharmacy Management: The majority of costs for members with hemophilia are in factor replacement. Thus, both price and capability are important in selecting pharmacy vendors for carved-in services. If carved in, pharmacy may be handled in-plan or delegated to a specialty pharmacy. Pharmacy benefit managers specializing in hemophilia and 340b pharmacies are the predominant pharmacy providers for hemophilia services. Plans must identify cost effective strategies and partners for delivery of routine and emergency factor replacement products.
- Risk Adjustment: As an inherited disease, hemophilia is clustered in families and not evenly distributed across the population. This means that some states or plans may have a cluster of members, while others do not encounter any members with this condition. States and plans can proactively develop solutions to mitigate the financial risk.
- Patient Involvement: Patients and families are responsible for many aspects of hemophilia care, including home infusion. This entails a significant teaching and assessment function for the pharmacy provider. Engaging and involving members and their families in coverage and care management decisions is also essential. In addition, connecting members to community and national support systems is important for quality of life.

Each of these issues is discussed below, and considerations for health plans are identified.

## Treatment Access and Quality

Clinical care for people with hemophilia is delivered through a network of specialized Hemophilia Treatment Centers. HTCs offer access to an array of professionals – hematologists, orthopedists, dentists, nurses, social workers and physical therapists among others – who treat hemophilia on a daily basis. HTCs also carry out clinical research, improving patient access to clinical trials. This “centers of excellence” approach helps to ensure comprehensive, high quality care and prevent long term disability.

From a health plan perspective, the nature of a rare disease makes it challenging to ensure availability of practitioners with expertise in treating the condition. The federal Hemophilia Treatment Center program has simplified the process by funding centers where providers can get specialized training and where patients can access the full array of services required for quality treatment. Health plans’ network contracting strategy should encompass a full range of multi-disciplinary hemophilia specialists, most easily accomplished through contracts with HTCs.

Another critical issue for plans is assuring treatment quality. Health plans may need to adapt medical management protocols to ensure that members have medically necessary services that might otherwise be subject to limitations, such as physical therapy or dental care. Patients may need to use an out of network provider for dental care or surgical services, in order to access a provider with expertise treating people with hemophilia. Health plans also have a role in anticipatory care management and assuring access to appropriate clotting factor for patients undergoing surgical procedures or inpatient stays.

### Health Plan Considerations: Treatment Access and Quality

- Identify and contract with specialized providers to deliver comprehensive services. Since 70% of patients with hemophilia are treated by HTCs, health plans should make it a priority to implement contracts with HTCs. HTCs can participate in health plan networks as contracted providers or through out of network agreements.
- Consider alternative agreements for non-contracted or out of state HTCs. HTCs that are part of a larger academic medical center or acute care facility that does not participate in a health plan network, may be able to provide services as an in-network provider based on a letter of agreement.
- Ensure access to the full scope of services required for comprehensive hemophilia care. Agreements with HTCs should address the scope of services that are covered under the in network agreement, including pharmacy, dental, physical therapy, and orthopedic services.
- For members who do not use an HTC, identify sources of expert specialty care including developing telemedicine programs for remote access or offering specialty consultation to treating providers.
- Regularly evaluate information on access and quality of care for members with hemophilia. This may be through evaluation of patient data or individualized interviews or surveys.
- Where appropriate, facilitate patient enrollment in clinical trials.

## Care Management

Patients with severe hemophilia can become catastrophically ill very quickly and may have comorbidities such as HIV or hepatitis. These members are logically candidates for complex case management services. Care coordination goals include ensuring access to essential medications, limiting waste, ensuring that patients have the education and preparation for home factor infusion, and connecting members to social support as needed. Health plans may be able to more effectively provide services by identifying and engaging members upon enrollment rather than waiting for a catastrophic event.

Health plans typically offer care coordination services to members who are at very high risk for high cost or poor clinical outcomes. But as one analysis of hemophilia issues in managed care noted, although hemophilia is a chronic condition with high individual costs, the very small percentage of the population affected may mean that plans do not flag this condition for significant attention.<sup>29</sup> Health plans with carved out pharmacy costs may not know of the hemophilia diagnosis until the member generates high medical claims. In fact one plan contacted by MHPA indicated that because pharmacy is carved out in their state, the plan would be unlikely to identify a member with hemophilia unless that member had a catastrophic event generating high medical claims.

Care coordination services may be available to patients with hemophilia from several sources. HTCs offer care coordination for patients treated in the HTC, including coordination of home infusion services and specialty physician services. Specialty pharmacy benefit managers, discussed in more detail below, also often coordinate home care, testing, and factor management. For patients enrolled in a health plan, therefore, care coordination may mean identifying the patient and ensuring that other care coordination services are functioning smoothly.

Health plan care coordinators can serve as a knowledgeable single point of contact to assist patients in addressing routine and urgent needs. The plan would not need to duplicate HTC and pharmacy care coordination services, but would likely want to be aware of the services being provided to a high risk patient. Rather than try to develop or hire the expertise needed to serve this very small population, health plans may find that it is more cost effective to provide umbrella oversight for specialized hemophilia care coordinators located in an HTC or specialty pharmacy provider.

### Health Plan Considerations: Care Coordination

- When HTCs are providing care, determine what wrap around care coordination services are needed in order to not duplicate services at added expense.
- Ensure appropriate access to providers and pharmacies with expertise in hemophilia, that results in effective preventive and acute care
- Ensure primary care access and timely primary preventive care, including vaccinations
- Coordinate care with other specialty providers
- Promote medication and testing adherence
- Facilitate home care services including infusion and port management
- Assist members to access social support for quality of school, home and work life
- Anticipate and plan for events that may trigger bleeding such as surgery
- Monitor treatment and performance indicators
- For women, ensure access to family planning, genetic counseling or high risk prenatal care.

## Cost Management

Hemophilia drugs make up 86% of the costs for people with hemophilia A. Variables in the cost of care include disease severity, frequency of bleeding, development of inhibitors, and the price of factor replacement products (biologics). Patients with complications or “inhibitors” can generate over a million in cost per year. A study of 2008 costs in a commercial population reported an average cost of \$155,000 (both hemophilia A and B); 3% of that population had an inhibitor and averaged \$697,000 annually.<sup>30</sup> A 2008 study of hemophilia and Medicaid found that the average cost of medications was almost \$143,000 per year.<sup>31</sup> A federal analysis of 2010 Medicare spending found that Medicare paid an average of \$217,000 annually for recombinant Factor VIII replacement for the 660 beneficiaries with hemophilia A – making it the most costly drug average per beneficiary.<sup>32</sup>

Estimating annual costs for hemophilia treatment is a challenge on several levels. Most studies on do not differentiate between type (Hemophilia A and B) and severity (mild, moderate, severe), instead yielding overall estimates for average cost that vary widely from study to study. In addition, experts in hemophilia note that average cost estimates may be misleading, since there is tremendous variability in individual cost. Many experts believe a two-year average represents a more accurate portrayal of costs. More studies are needed that analyze separately the costs associated with different types of hemophilia. Nevertheless, it is clear that treatment costs for members with severe hemophilia are routinely high, and can be catastrophic.

Factor replacement cost is significantly higher for members with inhibitors; these members cost almost four times the median of those without inhibitors. As noted, over 30 percent of the population with hemophilia develops “inhibitors” at some point. People with inhibitors may require a significantly higher factor dose until resolution of inhibitors,<sup>33</sup> which may cost in the millions of dollars. High levels of inhibitors can result in much more severe bleeding than that found in non-inhibitor patients. This, plus the costs of treatment and the problems resulting from patients’ immune suppression, leads to overall higher treatment costs, along with significant patient morbidity.

Since pharmacy cost is a driving factor for hemophilia costs, health plans need to understand variables and implement a cost effective factor purchasing strategy. This is discussed in more detail in the Pharmacy Management Section. Typically health plans use prior authorization and contracts with pharmacy benefits managers or specialty pharmacies – including HTCs with 340b pharmacies - to ensure appropriate dosing and inventory of related services such as infusion supplies.

Health plans also look at the total cost picture including use of preventable acute care services. Indirect costs include emergency and inpatient services that are influenced positively or negatively by appropriate factor management (e.g. preventing avoidable bleeds). Both underutilization and overutilization are problematic for members with hemophilia. Underutilization reflects under treatment of hemophilia, and could lead to avoidable bleeds. Overutilization can result in unnecessary factor infusion or wasted doses.

### Health Plan Considerations: Cost Management

- Work with state policy leaders to develop an effective purchasing strategy for factor under Medicaid (either through a 340b or with sufficient rebates)
- Monitor factor costs to identify the most cost effective purchasing route.

- Ensure that factor dosing is within recommended parameters and generates the appropriate clinical response for preventive and acute care (assay management)
- Ensure that pharmacy benefit managers or specialty pharmacy providers carry out the full scope of required factor management services, patient education, home care services and medical waste management
- Prevent wasted factor by ensuring appropriate pharmacy management and developing protocols for the number of doses kept in the patient homes
- Prevent acute or catastrophically expensive complications by coordinating with hospitals and other providers to plan for elective and emergency conditions
- Monitor and evaluate the total cost of care, including inpatient and emergency services, to evaluate use of avoidable acute care.

## Pharmacy Management

Pharmacy management is a critical treatment and cost management aspect of hemophilia care. Health plans managing the pharmacy benefit directly or through contract with a pharmacy benefit manager (PBM) need to carry out thorough due diligence with providers to ensure patients have access to experienced pharmacy providers who can deliver timely, safe, and effective products and supplies. Health plans may want to develop medical management parameters to ensure that patients receive the medically necessary dosing and in-home stock. Health plans also provide oversight of quality indicators relating to pharmacy and infusion services.

Pharmacy management has the

following goals:

- Ensure timely access to the appropriate clotting factor and other medications for the member
- Ensure appropriate supply chain management of the factor to ensure product quality (particularly when patients keep a stock of factor at home).
- Ensure access to home or outpatient center infusion services and management of ports
- Educate the patient on home IV administration if needed
- Ensure access to other medications that may include HIV and hepatitis treatment
- Deliver required infusion supplies including heparin and other materials to manage infusions
- Coordinate laboratory testing and reporting on factor use to ensure appropriate dosing
- Manage refills, including tracking and managing of home shipments of factor and infusion supplies
- Manage factor replacement and other biologics if the patient develops inhibitors
- Negotiate effective pricing of factor products

It is imperative that health plans work with specialty pharmacy and 340b providers with the expertise to manage the priorities cited above. Pharmacy benefits managers should be able to demonstrate proficiency in providing services prior to entering into a contract with the health plan. Contracted pharmacy vendors should have a robust program for monitoring quality indicators and reporting on them to the state or plan. Pharmacy services should be regularly reported to the health plan with information that includes factor utilization, number of patient bleeds, costs, timeliness of service and results of assay testing. Plans should carefully track use of other inpatient services and use this information to anticipate care management needs for the member.

There are clinical considerations in the choice of pharmacy providers. Clinical and product continuity are important. Hemophilia advocacy organizations note that factor products are not biologically interchangeable. Changing a product brand or switching from plasma based or recombinant product may trigger an allergic or inhibitor reaction. In addition, many patients have established relationships with factor providers and do not want to disrupt reliable and effective relationships. Patients new to coverage, relocating or transitioning from pediatric to adult service may need assistance identifying an appropriate specialized hemophilia treatment provider. Patients also need assurances that routine and emergency shipments of factor with related supplies will be delivered as needed. Plans can identify effective providers by engaging with the local patient advocacy organizations.

Reducing non-adherence to the prophylactic regimen is another key responsibility of the pharmacy management program. Patients on prophylaxis typically keep a supply of factor at home in order to self-infuse 2-3 times a week. Missing prophylactic doses is problematic in two ways. Most importantly, patients who do not receive the factor are at risk for bleeding – thus rendering the preventive program ineffective. Second, unused doses may accumulate and be wasted, representing a significant cost to the payer.<sup>34</sup> Effective pharmacy management programs collaborate with the patient to assess for non-adherence and address underlying factors.

Health plans need to evaluate their options for purchasing factor replacement products. States that carve out pharmacy services make the determination of where to purchase factor. For carved in products, options include use of specialty pharmacies, specialized pharmacy benefit managers, and HTC 340b pharmacies. State policy influences which approach is used.

An advantage of HTCs with 340b pharmacies is the co-location of medical and pharmacy services. Several states have developed innovative financing arrangements to take advantage of the cost savings achieved by 340b pharmacies to offer enhanced services. For example Kansas and Massachusetts pay a per-unit dispensing fee for factor obtained through the 340b pharmacy.<sup>35</sup> Costs are below what would be paid to a community pharmacy filling a Medicaid prescription, and the dispensing fee supports the comprehensive services of the parent HTC.

Plans need to evaluate options and negotiate the best price with a 340b pharmacy. Some states are less willing to allow use of 340b services for pharmacy dispensing in Medicaid due to availability of Medicaid drug rebates. Rebates cannot also be collected for drugs dispensed through 340bs. In addition, 340b pharmacies are also entitled to mark up the price charged to payer organizations, meaning that the 340b price is usually but not always the least expensive.

#### Health Plan Considerations: Pharmacy

- Contract with an experienced hemophilia pharmacy provider
- Ensure that pharmacy providers meet patient needs for consistent, timely services, products and infusion supplies
- Ensure that any vendor manages factor cost through appropriate assay testing and product management
- Develop policies to ensure correct dosing and stock in the member's home
- Monitor quality and accountability of pharmacy providers

#### Risk Adjustment

As an inherited disease, hemophilia is clustered in families and not evenly distributed in the population. Statistical modeling by Milliman suggests that the total number of members with hemophilia in any one health plan is likely to be small but unevenly distributed. Milliman estimates a plan with 5000 members could expect to have zero members, while plans with an enrollment of 100,000 would have an average of 8 members. Since hemophilia is clustered in families and care is regionalized (meaning that many families may cluster near regional treatment centers), some health plans will have no enrollees with hemophilia while others may have more than the expected number.

The cost of medications can be difficult to absorb in a capitated plan because costs dramatically from patient to patient. These factors result in unpredictable and unevenly distributed clinical needs and costs. A small plan providing coverage to a baby born with hemophilia or a member who develops inhibitors may generate catastrophic financial losses unless the plan's agreement with the state proactively addresses these contingencies. One possibility is that states offer stop-loss insurance if factor replacement is a carved-in service for members with hemophilia

#### Health Plan Considerations: Risk Adjustment

- Work with states to develop a statewide approach for financing care of members with hemophilia who may be enrolled in the future. This may include a risk pool or carve out.
- Develop protocols that explicitly call for management of patients with hemophilia by hemophilia treatment centers and ensure that all plans in the state have contracts with the HTC.
- Develop strategies for data sharing across vendors to ensure coordination of services for completely or partially carved-out services

- Identify financing arrangement for members with hemophilia work in conjunction with a capitated approach
- Ensure that states offer stop-loss insurance if factor replacement is a carved-in service for members with hemophilia

## Patient Involvement

As is the case for many rare diseases, the hemophilia community has developed a very active policy and support network. National and state based hemophilia organizations offer information, social support services, and often links with community resources. The groups also carry out fundraising for clinical services and research. Patient advocacy organizations for hemophilia – notably the National Hemophilia Foundation and the World Federation of Hemophilia have taken a leadership role in evaluating the research to promulgate clinical practice guidelines and other recommendations.

Patient organizations have outlined principles they believe are essential to appropriate treatment of members with hemophilia including access to factor and ancillary supplies, use of specialty pharmacy providers, and financing arrangements. The groups have raised concerns about managed care programs that disrupt established relationships with either pharmacy or medical providers and replace them with less costly and also less experienced providers.<sup>36</sup>

Health plans can benefit by recognizing the crucial role that advocacy has had in improving treatment for hemophilia, and recognizing patients and families as partners.

### Health Plan Consideration: Patient Involvement

- Ensure that the plan's treatment approach reflects best practices in hemophilia care (drawing on experienced providers and programs)
- Engage with state and local patient advocacy organizations early, to solicit input on program design and preferences
- Communicate early and often about health plan decisions that may impact any aspect of hemophilia treatment
- Provide opportunities for patient feedback on changes being considered by the health plan
- Carry out transition planning as members move from fee for service into managed care
- Be accountable to report on clinical quality and patient experience results of the hemophilia managed care program.

## Conclusion

Hemophilia is a lifelong chronic illness that requires complex clinical management. With appropriate care management and preventive care, people with hemophilia (diagnosed as children) can live full lives free of disability. The large majority of people with hemophilia are cared for through a well-established federally funded system of comprehensive Hemophilia Treatment Centers. HTCs integrate best practice specialized care and pharmacy services.

With implementation of health reform and Medicaid expansions, it is likely that Medicaid health plans will enroll more members with hemophilia over the coming years. Health plans are encouraged to

proactively plan for these members and develop systems of care that deliver multi-disciplinary services through experienced providers. Plans should provide oversight to ensure that the high costs of care for these members reflect dollars effectively and efficiently well spent.

Health plans should prioritize development of arrangements with HTCs to deliver care for members with hemophilia. Health plans should also examine opportunities to lower factor replacement drug costs through relationships with 340b pharmacies (while keeping in mind the loss of rebates on 340b products). If permissible through the state Medicaid program, acquisition of factor through a 340b may be the most cost effective way to purchase the drugs through an integrated medical and pharmacy provider offering comprehensive hemophilia care. Other specialized pharmacy providers also deliver accountable factor delivery programs.

As with any rare disease, plans should work closely with patients and their advocates to identify patient priorities and preferences and to address them when possible. A close collaboration and continued communication with the local and national hemophilia community will help promote seamless transitions that protect the health of members.

## Part 3: Resources<sup>‡</sup>

### Federal Organizations

#### **Centers for Disease Control and Prevention - Hemophilia Home Page**

[www.cdc.gov/ncbddd/hemophilia/index.html](http://www.cdc.gov/ncbddd/hemophilia/index.html)

CDC conducts surveillance and epidemiologic research on hemophilia. The CDC website includes information about prevalence and manifestations of hemophilia. Also provides information on Hemophilia Treatment Centers and links to regional hemophilia program contacts.

#### **Health Resources and Services Administration (HRSA)**

[www.mchb.hrsa.gov/programs/hemophilia/](http://www.mchb.hrsa.gov/programs/hemophilia/)

[www.hrsa.gov/opa/index.html](http://www.hrsa.gov/opa/index.html)

HRSA is the lead organization on the Hemophilia Treatment Center Program. HRSA is also responsible for management and oversight of the 340B program, which is open to HTCs and other federally funded clinics for the underserved. The HRSA 340b website includes regulatory information and FAQs on 340B operations.

#### **National Institutes of Health Office of Rare Disease Research (NIH ORDR)**

[www.rarediseases.info.nih.gov/GARD/Condition/10418/Hemophilia.aspx](http://www.rarediseases.info.nih.gov/GARD/Condition/10418/Hemophilia.aspx)

The ORDR offers web resources and responds to questions about rare diseases. The office tracks research on rare diseases, including biologic mechanisms and biologic and gene therapy treatments.

### Advocacy Organizations

#### **Hemophilia Federation of America (HFA)**

[www.hemophilafed.org/](http://www.hemophilafed.org/)

Hemophilia Federation of America (HFA) is a non-profit 501(c)(3) organization incorporated in 1994 to address the evolving needs of the bleeding disorders community. HFA serves as a consumer advocate for safe, affordable, and obtainable blood products and health coverage, as well as a better quality of life for all persons with bleeding disorders. HFA's ongoing consumer advocacy agenda includes product safety, as well as accessibility, affordability, and availability of the products the individuals of this community require.

#### **National Hemophelia Foundation (NHF)**

[www.hemophilia.org](http://www.hemophilia.org)

The NHF provides services and information to people with hemophilia, and is dedicated to improving treatments and finding a cure for hemophilia. The NHF resource center that includes research and resources for patients and families, and also helps to link individuals with appropriate treatments and services. The NHF offers talking points and briefings on insurance coverage for hemophilia, and sponsored an analysis of factors influencing capitation rates in Medicaid managed care. (linked in publications section below).

#### **The Hemophilia Alliance**

[www.hemoalliance.org](http://www.hemoalliance.org)

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<sup>‡</sup> This is a sample of organizations and resources available

## Hemophilia in Medicaid Managed Care - MHPA Issue Brief

The Hemophilia Alliance is a not-for-profit organization representing the interests of HTCs that operate or are seeking to have 340B programs. The purpose of the Alliance is to promote the common interests of these HTCs. The Alliance website offers information about the 340b program along with information for payers. The Hemophilia Alliance, a national organization, has developed a Medicaid Model Program for integrating clinical care and 340B pharmacy services covered by Medicaid.<sup>37</sup>

### Safety Net Hospitals for Pharmaceutical Access

[www.snhpa.org/](http://www.snhpa.org/)

SNHPA represents close to 1,000 public and private nonprofit hospitals and health systems that participate in the 340B drug discount program. SNHPA was formed in 1993 to increase the affordability and accessibility of pharmaceutical care for poor and underserved populations. It monitors, educates, and advocates on behalf of members with respect to federal and state laws and regulations pertaining to 340B and related pharmacy matters.

**Multiple states chapters and state organizations are also engaged in advocacy**

### Hemophilia Guidelines

#### NHF Medical and Scientific Advisory Council (MASAC)

[www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=157&contentid=347](http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=157&contentid=347)

The NHF MASAC is a multi-disciplinary advisory body that includes representatives from the provider community and public agencies such as CDC, NIH, and the Food and Drug Administration. The MASAC makes recommendations on hemophilia clinical treatment issues and other factors that impact access to treatment, based on review of evidence and professional consensus.

#### World Federation of Hemophilia

[www.wfh.org/en/page.aspx?pid=492](http://www.wfh.org/en/page.aspx?pid=492)

**Guidelines for the management of hemophilia.** [www.ncbi.nlm.nih.gov/pubmed/22776238](http://www.ncbi.nlm.nih.gov/pubmed/22776238)

The World Federation of Hemophilia (WFH) provide global leadership to improve and sustain care for people with inherited bleeding disorders, including hemophilia, von Willebrand disease, rare factor deficiencies, and inherited platelet disorders. The WFH has developed guidelines on a variety of aspects of hemophilia care.

### State Hemophilia Program Examples

#### Florida Agency for Health Care Administration

[www.fdhc.state.fl.us/Medicaid/MediPass/diseaseManagement.shtml](http://www.fdhc.state.fl.us/Medicaid/MediPass/diseaseManagement.shtml)

[www.medicaid.gov/Medicaid-CHIP-Program-Information/By-Topics/Waivers/1115/downloads/fl/fl-medicaid-reform-pa.pdf](http://www.medicaid.gov/Medicaid-CHIP-Program-Information/By-Topics/Waivers/1115/downloads/fl/fl-medicaid-reform-pa.pdf)

Disease Management is required for members with hemophilia. Members must enroll with one of two designated providers.

#### Illinois Department of Healthcare and Family Services - Medicaid hemophilia management program

<http://www.hfs.illinois.gov/pharmacy/hemo.html>

Save Medicaid Access and Resources Together (SMART) Act address utilization management, standards of care, pharmacy standards, and conflict of interest in dispensing of blood products for Medicaid beneficiaries with hemophilia.

## Hemophilia in Medicaid Managed Care - MHPA Issue Brief

### New Jersey

Described in bleeding disorder legislative analysis here: <http://www.cga.ct.gov/2010/rpt/2010-R-0001.htm>

### North Carolina Department of Health and Human Services - Hemophilia Specialty Pharmacy Program

<http://www.ncdhhs.gov/dma/bulletin/0113bulletin.htm#hemo>

The specialty pharmacy program for hemophilia drugs mandated by the General Assembly [Session Law 2012-142, Section 10.48 (a2)] will be implemented on January 31, 2013. Pharmacy providers furnishing hemophilia drugs and services to Medicaid and N.C. Health Choice (NCHC) beneficiaries should follow the clinically appropriate standards of care outlined in Clinical Coverage Policy No. 9B, Hemophilia Specialty Pharmacy Program.

### Health Plan Resources

#### Aetna Clinical Policy Bulletin – Clotting Factors

[http://www.aetna.com/cpb/medical/data/100\\_199/0131.html](http://www.aetna.com/cpb/medical/data/100_199/0131.html)

#### United Healthcare Hemophilia Management Program

[https://www.unitedhealthcareonline.com/ccmcontent/ProviderII/UHC/en-US/Assets/ProviderStaticFiles/ProviderStaticFilesPdf/Tools%20and%20Resources/Pharmacy%20Resources/hemophilia\\_FAQ.pdf](https://www.unitedhealthcareonline.com/ccmcontent/ProviderII/UHC/en-US/Assets/ProviderStaticFiles/ProviderStaticFilesPdf/Tools%20and%20Resources/Pharmacy%20Resources/hemophilia_FAQ.pdf)

#### Keystone Mercy

<http://www.keystonemercy.com/member/eng/health/carecoord/hemophilia/index.aspx>

### Publications Relevant to Medicaid Health Plans

Health care expenditures for Medicaid-covered males with haemophilia in the United States, 2008 (2012) [http://www.cdc.gov/ncbddd/hemophilia/documents/GuhMedicaidHaemophilia2012\\_sy5.pdf](http://www.cdc.gov/ncbddd/hemophilia/documents/GuhMedicaidHaemophilia2012_sy5.pdf)

Hemophilia and Managed Care: Partnering to Achieve Cost-Effective Care (2011)

Am J of Pharmacy Benefits - Full text, slide presentation and continuing education offering available here: [www.shbc.us/index.php?option=com\\_content&view=article&id=40:hemophilia&catid=1](http://www.shbc.us/index.php?option=com_content&view=article&id=40:hemophilia&catid=1)

Actuarial Opinion On Issues In The Capitation Of Hemophilia (2006)

<http://www.hemophilia.org/NHFWeb/MainPgs/MainNHF.aspx?menuid=157&contentid=347>

A Model for a Regional System of Care to Promote the Health and Well-Being of People with Rare Chronic Genetic Disorders

[http://hemoalliance.org/documents/HTC\\_Article-CompCareModel-Baker\\_et\\_al\\_D0085551.pdf](http://hemoalliance.org/documents/HTC_Article-CompCareModel-Baker_et_al_D0085551.pdf)

Mortality among males with hemophilia: relations with source of medical care

<http://bloodjournal.hematologylibrary.org/content/96/2/437.full.pdf+html>

Safety Net Hospitals for Pharmaceutical Access. "State 340b Medicaid Billing Best Practices."

[www.snhpa.org/public/documents/pdfs/state\\_340b\\_medicaid\\_billing\\_bp.pdf](http://www.snhpa.org/public/documents/pdfs/state_340b_medicaid_billing_bp.pdf)

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