Georgia Hemophilia Advisory Board

Report to the Office of Health Strategy and Coordination

Hemophilia and Other Bleeding Disorders: Standards of Care

October 2021

Table of Contents

Background

In 2011, House Bill 214 was enacted in Georgia establishing a Hemophilia Advisory Board to provide expert advice to the state on health and insurance policies, plans, and programs that impact individuals with hemophilia and other congenital bleeding disorders. The Board was directed to "establish standards of care and treatment for persons living with hemophilia and other congenital bleeding disorders, taking into consideration the federal and state standards of care guidelines developed by state and national organizations, including, but not limited to, the Medical and Scientific Advisory Council of the National Hemophilia Foundation." In 2019, this law was amended by HB 186 so that the Hemophilia Advisory Board would submit its annual report on October 1 to the Office of Health Strategy and Coordination instead of to the governor and General Assembly on January 1.

Members of the Hemophilia Advisory Board

The following individuals serve on the Hemophilia Advisory Board:

Hikie Allen, Consumer, Board Member, Hemophilia of Georgia
Pamela Bryant, Administrative Director, Hemophilia of Georgia Center for Bleeding & Clotting Disorders of Emory
Leigh Carpenter, RPh, Vice President of Pharmacy Services, Hemophilia of Georgia
Julia Conde, Consumer
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Amanda Greene, RN, MSN, Lead Pediatric Nurse Coordinator, Hemophilia of Georgia Center for Bleeding & Clotting Disorders of Emory
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Overview of Hemophilia and Other Inherited Bleeding Disorders

Hemophilia is a rare, hereditary disorder in which a person's blood does not clot properly due to an insufficient amount of a protein, called clotting factor, in the blood. The missing clotting factors are denoted with Roman numerals, either factor VIII (FVIII) in hemophilia A or factor IX (FIX) in hemophilia B. The person with hemophilia does not bleed faster than the average person and will not have a problem with a superficial cut or scrape. However, persons with hemophilia (depending on the severity of the disease) often experience internal bleeding into joints, muscles, and vital organs; without adequate treatment, these bleeding episodes can lead to disability and even death.

Although hemophilia is lifelong, it is treatable. Preferred treatment is replacement of the missing clotting factor through intravenous infusion of either a highly purified or recombinant blood clotting factor (protein). In 2018, the U.S. Food and Drug Administration (FDA) approved Hemlibra® (emicizumab-kxwh), a non-factor coagulation product, for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults and children, ages newborn and older, with hemophilia A without factor VIII inhibitors. It is injected subcutaneously (under the skin) and had been previously approved by the FDA for hemophilia A patients with inhibitors. Clotting factor products and non-factor coagulation products are extremely expensive, with costs reaching as high as $300,000 to over $1,000,000 per year, depending on the severity of disease, the adequacy and expediency of medical care received, and whether a patient develops an inhibitor to the standard factor replacement products.

Optimal medical oversight is offered by hemophilia experts at federally designated Hemophilia Treatment Centers (HTCs). Georgia's HTCs are located at Emory University (Atlanta), Augusta University, and The Children's Hospital at Memorial University Medical Center (Savannah). Timely access to an HTC and clotting factor reduces morbidity and mortality in a person with hemophilia. [*Blood*. 2000 Jul; 96(2):437-42 and *Am J Public Health.* 1984 Jun; 74(6):616-7.] Instruction in self-administration of medication and disease education for the patient and community providers are integral to managing the disorder and contribute to a patient's quality of life and independent, productive living.

There are other inherited bleeding disorders treated at HTCs, including von Willebrand Disease (which is believed to occur in as many as 1 in every 100 people), rare bleeding disorders such as Glanzmann thrombasthenia and Bernard-Soulier Syndrome, and qualitative platelet disorders such as delta storage pool deficiency. In Georgia, over1,600 people have been identified with hemophilia or other inherited bleeding disorders.

Standards for providers of clinical care of patients with inherited bleeding disorders

Since the 1970s, the Hemophilia Treatment Center (HTC) has been the optimal model for the delivery of integrated, multi-disciplinary comprehensive care and has been shown to reduce mortality and morbidity in patients with inherited bleeding disorders including hemophilia. (Soucie, JM, B.L. Evatt and the Hemophilia Surveillance System, Hematologic Diseases Branch, Centers for Disease Control and Prevention. Care in HTCs is Associated with Lower Mortality Risk among Persons with Hemophilia in the United States, *Haemophilia*, Volume 4, Number 3, May 1998).
Providers of clinical care at an HTC, or their designees if appropriate, shall:

a) Have an integrated core team which includes the medical director (typically a hematologist), a bleeding disorder nurse who has been trained in bleeding disorders and infusion skills, a social worker for assessment, referral, and guidance in payment/insurance issues, a program coordinator (may be the nurse) and a physical therapist who can offer joint range of motion assessment and rehabilitation from possible muscular or joint injuries.
b) Offer comprehensive evaluations which include medical history, physical examination, nursing evaluation, physical therapy evaluation, psychosocial evaluation, and periodic laboratory evaluation and surveillance.
c) Provide comprehensive clinics including pre-clinic and post-clinic conferences designed to create and implement individualized treatment plans for the patients.
d) Offer a sufficient number of clinics to meet the recommendation of a patient's physician and what their condition dictates. (The suggested minimum number of comprehensive care clinic appointments for moderate and severe hemophilia, Glanzmann thrombasthenia, Bernard-Soulier syndrome, and other severe factor deficiency disorders is annual. Comprehensive care visits are recommended for non-severe bleeding disorders every other year, including certain types of VWD, platelet disorders, mild hemophilia, and mild/moderate factor deficiencies.
e) Collaborate with expert clinical care providers who are needed to address complications of bleeding disorders.
f) Include as extended team members or provide referrals to the following as needed: genetic counseling, dental services, nutrition counseling, orthopedics, gynecology and obstetrics, infectious disease oversight, and vocational guidance; ensure that these providers have knowledge and experience in the care of persons with bleeding disorders.
g) Have access to specialized laboratories that can provide timely, reliable, and accurate factor assays and platelet function assessment for the diagnosis and management of patients.
h) Partner with pharmacies providing clotting factor products to monitor correct usage of products by the patients, including reviewing order patterns.
i) Consider for patients the demonstrated benefits of early intervention and prophylaxis (regular administration of clotting factor to prevent bleeding).

j) Coordinate care with the patient's primary care physician, including having the HTC send the physician a summary letter following the patient's annual comprehensive examination.
k) Maintain confidential integrated patient records and adhere to HIPAA regulations.
l) Ensure educational or training opportunities (including professional meetings specific for bleeding disorders) for the core team to maintain expert knowledge of inherited bleeding disorders and evidenced-based treatment regimens.
m) Provide physicians, who are knowledgeable in the management of persons with inherited bleeding disorders, for consultation on a 24-hour basis.
n) Provide educational resources, home infusion training, and instruction in maintenance of home therapy records.
o) Provide services in a culturally sensitive manner, including providing interpreters when needed and literature that is appropriate for targeted audiences.
p) Partner with patients to facilitate self-management and independence, including plans for emergencies and acquisition of emergency medical identification devices.
q) Have a formal policy in place designed to provide patients and families with the education and training required to develop independent self-care as they transition from pediatric care to the adult care setting.
r) Participate in a national database/s that supports the development of clinical outcomes research projects for bleeding disorders, such as the American Thrombosis and Hemostasis Network, in as much as this research is critical to identify safer, more efficacious, and cost-effective ways to manage these disorders and their complications.
s) Offer, when available, information and access to clinical trials or therapeutic research to benefit people with bleeding disorders, because many of these trials provide access to cutting-edge therapies while saving the patient and their insurance company (or Medicaid or Medicare) the cost of providing routine visits and treatment (clotting factor/non-factor coagulation product) for up to several years.
t) Offer education about bleeding disorders to the patients' communities, including the primary care providers and local emergency departments.
u) Participate in a formal Quality Improvement program (as guided by the HRSA/MCHB Healthy People 2030 measures published for persons with inherited bleeding disorders) that includes the acquisition of patient and caregiver input and feedback.
v) Provide information on appropriate questions that patients should ask when choosing a pharmacy to provide medications for inherited bleeding disorders.

Standards for pharmacies providing clotting factor and non-factor coagulation products for home use by patients with bleeding disorders

A pharmacy providing clotting factor or non-factor coagulation products for home use by patients with bleeding disorders shall:

a) Be licensed and accredited.
b) Have sufficient training, knowledge and understanding of bleeding disorders to accurately follow the instructions of the prescribing physician.
c) Have access to providers with sufficient clinical experience in caring for people with bleeding disorders.
d) Provide for the shipment of prescribed clotting factor or non-factor coagulation products to the patient within twelve hours in an emergency and otherwise within 72 hours.
e) Provide access to an on-call pharmacist 24/7 for emergency requests.
f) Have the ability to obtain all brands of clotting factor and non-factor coagulation products approved by the FDA. The products will be offered in multiple assay ranges and vial sizes and include products manufactured both from human plasma and with recombinant biotechnology (provided manufacturer supply exists and payer authorization can be obtained).
g) Supply all ancillary infusion equipment and supplies required to administer the clotting factor or non-factor coagulation products.
h) Store and ship, or otherwise deliver, products in conformity with state and federally mandated standards, including the standards listed in the product's package insert.
i) Not make any substitutions of clotting factor or non-factor coagulation products without the prior approval of the prescribing physician.
j) Fill prescriptions to meet a MASAC recommended goal of 10% over and 5% under for assay management but complying with each payer's requirements for assay management, to ensure proper management of shipments to meet patient's needs as requested.
k) When necessary, provide home nursing services within a timely manner, either directly or through a third party, utilizing licensed nurses with formal training and experience in treating bleeding disorders and the ability to provide home infusion and home infusion training for the patients as well as port care consistent with the protocol utilized by the HTC.
l) Provide a contact number for patients to report delivery problems.
m) Participate in the National Patient Notification System for clotting factor or non-factor coagulation product recalls and provide patients with notification of recalls of clotting factor or non-factor coagulation products and ancillary infusion equipment within twenty-four hours.
n) Provide language interpretive services over the phone or in person.
o) Have a plan for fulfilling orders in the event of a disaster.
p) Provide patients with a sharp needles disposal container and instructions on how to dispose of hazardous medical wastes according to state law. (However, the

provider of blood clotting products shall not be liable for any acts or omissions of the patient in the handling and disposal of medical waste.)

q) Provide appropriate and necessary recordkeeping and documentation including the retention of copies of the patient's prescriptions and product dispensing records and be able to provide records to the prescribing provider on at least a quarterly basis in support of Item r).
r) Partner with clinical care providers to monitor the patient's product utilization, including reviewing order patterns, in an effort to identify new bleeding patterns, monitor for inhibitor development, and monitor compliance with prophylaxis regimens.
s) Comply with the requirements of the Health Insurance Portability and Accountability Act of 1996 (HIPAA).
t) Provide patients with the information to report pharmacy delivery complaints directly to an accrediting agency.

Standards for insurers/managed care providers who cover clinical care (including health care services for home treatment) or drug benefits for individuals with bleeding disorders

The insurer shall:

a) Provide payment for all FDA-approved brands of clotting factor products and substitutes for clotting factors (along with pharmaceuticals that stimulate clotting factor release or diminish the breakdown of blood clots) in multiple assay ranges (low, medium, and high) as applicable, including products manufactured from human plasma and those manufactured with recombinant biotechnology techniques.
b) Provide to the covered person a choice of more than one clinical provider, including a federally funded HTC, that adheres to the standards of clinical care listed above.
c) Provide to the covered person a choice of more than one participating pharmacy that adheres to the standards listed above; one of these should be a designated 340B pharmacy.
d) Provide pre-approval or pre-authorization, if required, of a prescription for clotting factor products prior to the dispensing of the same within 72 hours or less if the treating physician deems the need to be urgent and requests a shorter time and consider extending pre-authorizations for one year in cases deemed appropriate by a provider.
e) Provide to the covered person coverage of coagulation laboratory services considered medically necessary for the screening, diagnosis, and treatment of bleeding disorders.
f) Provide that patient's prior approval of a prescription is valid for one year, instead of having to renew every three months.
g) Provide approval of a prescription for at least three days of therapy using recombinant factor for patients with Glanzmann thrombasthenia when the treating physician deems it necessary.

Standards for consumers of clinical or pharmacy services

Persons with bleeding disorders and the medical providers who offer them care and treatment shall adhere to the National Hemophilia Foundation's Consumer Bill of Rights and Responsibilities for Healthcare Services. (National Hemophilia Foundation's "Consumer Bill of Rights and Responsibilities for Healthcare Services," approved by NHF Board of Directors, July 1994.)

The consumer shall:

a) Adhere to use of a treatment calendar reporting system (paper or electronic) agreed upon by the patient and the patient's medical care team ensuring the ability of the hemophilia treatment center clinical staff to accurately assess the efficacy of treatment regimens and to adjust when necessary.
b) Give correct and complete information regarding insurer and health status to the patient's health care provider.
c) Access insurance, if it is available and affordable to them, or accept responsibility for declining such insurance.
d) Report to the Pharmacy Board of Georgia and the Georgia Office of the Inspector General their concerns about questionable practices of pharmacies that offer patients non-pharmaceutical-related kickbacks, show or no-show jobs, or other incentives that require patients to restrict their purchases to the pharmacy offering the incentive.

Definitions

Adjunctive treatment products - non-clotting factor therapies for treatment of bleeding disorders that include, but are not limited to, intravenous and intranasal pharmaceuticals that stimulate the release of factor VIII and/or von Willebrand factor, and oral and intravenous pharmaceuticals that slow the breakdown of formed clot at the site of injury/healing.

Ancillary infusion equipment and supplies - the equipment and supplies required to infuse clotting factor into a human vein. Includes, but is not limited to, syringes, needles, sterile gauze, gloves, alcohol swabs, and biohazardous waste containers.

Assay - the amount of a particular constituent of a mixture or of the biological or pharmacological potency of a drug.

Bleeding disorder - a medical condition characterized by a deficiency, absence, or defective production in an individual's blood of one or more essential blood-clotting proteins (clotting factors); includes hemophilia, von Willebrand Disease, and other inherited bleeding disorders. Platelet function disorders, defined below, also result in abnormal bleeding related to dysfunction.

Case management - coordination of needed medical, social, and support services for a patient, which includes, but is not limited to, coordination with Hemophilia Treatment Center care team, preparation and maintenance of needed documentation, assessment of patient psychosocial needs, and significant educational components for other providers.

Clotting factor - a medicine made from human plasma or by recombinant biotechnology which is approved by the FDA for the treatment and prevention of symptoms associated with bleeding disorders. The products may include, but are not limited to, recombinant factors VIII, IX, XIII and recombinant von Willebrand factor (including recombinant factor VIII and IX products with an extended half-life); plasma-derived factors VIII and IX; von Willebrand factor-containing plasma-derived products; products for patients with inhibitors, including activated prothrombin complex concentrates, recombinant factor VIIa, and recombinant porcine factor VIII; and factor X and factor XIII.

Coagulation laboratory - a laboratory able to diagnose bleeding disorders and perform specialized coagulation studies for patients with bleeding disorders to monitor the efficacy of treatment interventions.

Covered person - an individual who receives health care benefits or coverage for clinical care or drugs.

Healthcare insurer - an entity that issues an individual or a group health insurance policy or coverage.

Hemophilia - a bleeding disorder caused by an inherited deficiency of the factor VIII or factor IX blood clotting protein in the person's blood; may be classified as mild, moderate, or severe, depending on the amount of clotting factor present.

Hemophilia inhibitor - antibody that prevents normal function of either FVIII or FIX clotting factors rendering it useless as therapy and requiring the use of bypassing agents.

Hemophilia treatment center (HTC) - a federally designated specialty program in which a multi-disciplinary team of experts delivers diagnosis and treatment of congenital bleeding disorders; the core team includes a hematologist, a nurse coordinator, a social worker and a physical therapist.

Home infusion - infusion of a blood clotting product in a place other than an HTC, hospital, emergency room, physician's office, or clinic.

Home nursing services - instruction and assistance in the home by a nurse with formal training in the care of patients with inherited bleeding disorders; services include education in bleeding disorders and instruction in the reconstitution and intravenous administration of clotting factor.

Non-factor coagulation products - a medicine used to correct the hemostatic imbalance created in the laboratory using mechanisms to modify the coagulation cascade which is approved by the FDA for the treatment and prevention of symptoms associated with bleeding disorders. The products may include, but are not limited to, humanized bispecific (factor IXa/X) antibody replicating the function of FVIII.

Participating pharmacy - an entity which enters into an agreement with a health care insurer to dispense and educate the patient/care giver in the administration of blood clotting products, ancillary infusion equipment and supplies for home use by individuals with bleeding disorders.

Platelet function defects - a range of disorders characterized by abnormalities of the function, and sometimes the number of, blood platelets which are the cells in the blood that help it clot. The most common is called delta storage pool deficiency.

VWD or von Willebrand Disease - a bleeding disorder caused by an inherited deficiency or abnormality of the von Willebrand factor in the blood.

340B program - a federal program created under the authority of the Veterans Health Care Act of 1991 (Public Law 102-585, 106 Stat. 4943), which enacted section 340B of the Public Health Service Act (58 Stat. 682, 42 U.S.C. 256B) allowing designated covered entities, including federally-funded hemophilia treatment programs with pharmacy licenses, to purchase outpatient pharmaceuticals (such as clotting factor) at specified discounts so that they could sell the clotting factor to patients at a reduced price while offering them enhanced services.

Bibliography

Recommendation # 188 of the National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) "MASAC Recommendations Regarding Standards of Service for Pharmacy Providers of Clotting Factor Concentrates for Home Use to Patients with Bleeding Disorders"

Recommendation # 132 of the National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) "Standards and Criteria for the Care of Persons with Congenital Bleeding Disorders"

Recommendation # 202 of the National Hemophilia Foundation's Medical and Scientific Advisory Council (MASAC) "MASAC Recommendations Concerning Products Licensed for the Treatment of Hemophilia and other Bleeding Disorders"

National Hemophilia Foundation's consumer Bill of Rights and Responsibilities for Healthcare Services, Approved by NHF Board of Directors

Soucie, JM, B.L. Evatt and the Hemophilia Surveillance System, Hematologic Diseases Branch, Centers for Disease Control and Prevention. Care in Hemophilia Treatment Centers (HTC) is Associated with Lower Mortality Risk among Persons with Hemophilia in the United States, *Haemophilia*, Volume 4, Number 3, May 1998.

Georgia Resources

The Hemophilia of Georgia Center for Bleeding & Clotting Disorders of Emory

Pediatric Clinic Location:
Egleston Children's Hospital, 4th Floor
1405 Clifton Road NE
Atlanta, Georgia 30322
(404) 785-3523

Adult Clinic Location:
Emory Clinic Midtown
Medical Office Tower, 10th Floor, Suite 1090
550 Peachtree Street NE
Atlanta, GA 30308
(404) 778-7062

Augusta University

Pediatric Clinic Location:
Department of Pediatric Hematology/Oncology
1446 Harper Street, BG-2011
Augusta, Georgia 30912-3730
(706) 721-3626
Adult Clinic Location:
Adult Center for Blood Disorders
989 St. Sebastian Way
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