Blue Cross Blue Shield Blue Care Network of Michigan

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Effective Date: 06/10/2021

Hemophilia Class Policy

FDA approval: Multiple
HCPCS: Multiple
Benefit: Medical

Policy:

Requests must be supported by submission of chart notes and patient specific documentation.

A. Coverage of the requested drug is provided when all the following are met:
   a. Factor VIII products
      i. Diagnosis of hemophilia A, established by or in consultation with a hematologist
      AND
      ii. The requested dose and frequency are within the limits detailed in Table 1 (reflecting FDA labeled dosing) OR the provider has documented clinical reasoning for higher dosing
      AND
      iii. Patient weight, age, history of bleeds (both spontaneous and trauma) and inhibitor status- testing has been completed within the last 12 months and provided to plan
      AND
      iv. Medication is dispensed by a treatment center associated with hemophilia that provides high quality hemophilia care with outcome based results (ie: hemophilia treatment centers)
   b. Factor IX products
      i. Diagnosis of hemophilia B, established by or in consultation with a hematologist
      AND
      ii. The requested dose and frequency are within the limits detailed in Table 1 (reflecting FDA labeled dosing) OR the provider has documented clinical reasoning for higher dosing
      AND
      iii. Patient weight, age, history of bleeds (both spontaneous and trauma) and inhibitor status- testing has been completed within the last 12 months and provided to plan
      AND
      iv. Medication is dispensed by a treatment center associated with hemophilia that provides high quality hemophilia care with outcome based results (ie: hemophilia treatment centers)
   c. Hemlibra
      i. For prophylaxis of bleeding episodes in patients diagnosed with congenital hemophilia A with inhibitors
         1. Prescribed and dispensed by a specialist that works in a hemophilia treatment center

2. Documentation of a historical or current high titer for factor VIII inhibitors measuring > 5 Bethesda Units per milliliter (BU/mL)
3. Will not be used in combination with Immune Tolerance Induction (ITI)
4. Medication is dispensed by a treatment center associated with hemophilia that provides high quality hemophilia care with outcome based results (ie: hemophilia treatment centers)

ii. For prophylaxis of spontaneous bleeding episodes in patients diagnosed with congenital hemophilia A without inhibitors
1. Prescribed and dispensed by a specialist that works in a hemophilia treatment center
2. Documentation of severe hemophilia A with factor VIII level <1% OR moderate hemophilia A with factor VIII level between 1%-5%
3. Documentation of optimally dosed prophylactic factor VIII product is ineffective for the prevention of spontaneous bleeding events (such as: continuing to have bleeding events or arthroscopic changes within a target joint)
4. Documentation of the number of bleeds experienced within the past 12 months
5. Medication is dispensed by a treatment center associated with hemophilia that provides high quality hemophilia care with outcome based results (ie: hemophilia treatment centers)

B. Quantity Limitations, Authorization Period and Renewal Criteria
a. Quantity Limit: Align with FDA recommended dosing with a maximum 30 day supply
b. Initial Authorization Period: 6 months
c. Renewal Criteria:
i. Continuation of coverage will be provided when treatment has been proven successful through a decrease in the number of bleeds
ii. No development of anti-drug antibodies that impact the clearance or efficacy (Hemlibra only)
d. Renewal Authorization Period: 1 year

***Note: Coverage may differ for Medicare Part B members based on any applicable criteria outlined in Local Coverage Determinations (LCD) or National Coverage Determinations (NCD) as determined by Center for Medicare and Medicaid Services (CMS). See the CMS website at http://www.cms.hhs.gov/. Determination of coverage of Part B drugs is based on medically accepted indications which have supported citations included or approved for inclusion determined by CMS approved compendia.

Therapeutic considerations:

A. **FDA approved indication/Diagnosis**

**Please refer to most recent prescribing information.*

B. **Background Information**

a. There are two types of hemophilia: hemophilia A and hemophilia B
i. Hemophilia A is defined as a deficiency in factor VIII clotting factor
ii. Hemophilia B is defined as a deficiency in factor IX clotting factor

b. There are three main levels of severity
i. Mild hemophilia patients have a factor activity of 5 to 40% and usually do not experience any major problems in everyday life. It often goes unnoticed until puberty or adulthood when bleeding after surgery or a deep cut lasts longer than normal. These patients do not typically need prophylactic therapy and only require on-demand factor for injuries or surgeries

ii. Moderate hemophilia patients have a factor activity of 1 to 5% and may have occasional bleeding, but usually only following surgery or an injury. Only rarely will there be no apparent cause
iii. Severe hemophilia patients have a factor level of less than 1% and often have bleeding for no known reason, especially in the joints and muscles. From infancy, patients bruise easily and as they become more active, learn to walk and put more strain on their joints and muscles, bleeding starts to occur

c. There are two types of factor products available to treat hemophilia, plasma derived factor which is entirely made of plasma from human donations and recombinant factor which is made by genetically engineered technology, both with standard and extended half-life products. All factor products have demonstrated to have similar safety and efficacy in clinical studies treating or reducing bleeding episodes with the apparent difference in the frequency of administration: up to three times weekly injections for standard and weekly or every two weeks for extended half-life products

d. Patients with severe hemophilia may develop an inhibitor sometime in their lives. Inhibitors most often develop during childhood, especially during the first 50 exposure days

e. Inhibitors are classified into two categories
   i. Those with a 5 or higher Bethesda unit result are classified as having a "high responding" inhibitor level
   ii. Those who measure below 5 units are classified as having a "low responding" inhibitor level

f. Depending on the inhibitor level, different therapeutic options are available to patients.
   i. Bypassing agents are used in the treatment of inhibitors. These contain factors that can stimulate the formation of a clot and stop bleeding. While these treatments are effective, many limitations exist including potential for bleeding or over-production of clots, and the need for frequent doses
   ii. Hemlibra (emicizumab) is a recombinant, humanized, bispecific monoclonal antibody that bridges activated factor IX and factor X to restore the function of missing activated factor VIII in hemophilia A patients to restore hemostasis. Hemlibra has only been studied in patients with inhibitors greater than 5 BU/mL and has not been studied in combination with Immune Tolerance Induction therapy.

C. **Efficacy**

**Please refer to most recent prescribing information.*

D. **Medication Safety Considerations**

**Please refer to most recent prescribing information.*

E. **Dosing and administration**

**Please refer to most recent prescribing information.*

F. **How supplied**

**Please refer to most recent prescribing information.*

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Policy History		
#	Date	Change Description
1.4	Effective Date: 06/10/2021	Annual review of criteria was performed, no changes were made
1.3	Effective Date: 06/11/2020	Added Sevenfact
1.2	Effective Date: 12/05/2019	Updated Wilate indication to include hemophilia A
1.1	Effective Date: 02/03/2020	PA added to BCNA and MAPPO for Hemlibra
1.0	Effective Date: 05/09/2019	New coverage criteria PA added to Hemlibra for BCBS and BCN

** The prescribing information for a drug is subject to change. To ensure you are reading the most current information it is advised that you reference the most updated prescribing information by visiting the drug or manufacturer website or http://dailymed.nlm.nih.gov/dailymed/index.cfm.*