

# Antihemophilic Products – Etranacogene dezaparvovec-drlb (Hemgenix)

## Medical policy no. 85.10.25.AA-1 Year

# Effective Date: Month, 1,

## **Related medical policies:**

Policy Name	Indications
N/A	N/A

**Note:** New-to-market drugs included in this class based on the Apple Health Preferred Drug List are non-preferred and subject to this prior authorization (PA) criteria. Non-preferred agents in this class require an inadequate response or documented intolerance due to severe adverse reaction or contraindication to at least TWO preferred agents. If there is only one preferred agent in the class documentation of inadequate response to ONE preferred agent is needed. If a drug within this policy receives a new indication approved by the Food and Drug Administration (FDA), medical necessity for the new indication will be determined on a case-by-case basis following FDA labeling.

To see the list of the current Apple Health Preferred Drug List (AHPDL), please visit: <u>https://www.hca.wa.gov/assets/billers-and-providers/apple-health-preferred-drug-list.xlsx</u>

### Medical necessity

Drug	Medical Necessity
etranacogene dezaparvovec (Hemgenix)	etranacogene dezaparvovec (Hemgenix) may be considered medically necessary in patients who meet the criteria described in the clinical policy below.
	If all criteria are not met, the clinical reviewer may determine there is a medically necessary need and approve on a case-by-case basis. The clinical reviewer may choose to use the reauthorization criteria when a patient has been previously established on therapy and is new to Apple Health.

### **Clinical policy:**

Clinical Criteria			
Hemophilia B (congenital factor IX	etranacogene dezaparvovec (Hemgenix) may be approved when all of the		
deficiency)	following criteria are met:		
etranacogene dezaparvovec	1. Patient is 18 years of age or older; AND		
(Hemgenix)	2. Prescribed by, or in consultation with, a hematologist or		
	specialist in hemophilia; AND		
	<ol><li>Patient has <u>not</u> received prior gene therapy; AND</li></ol>		

Antihemophilic Products- Hemgenix

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<ol> <li>Diagnosis of moderately severe or severe congenital Factor IX deficiency (hemophilia B) defined by factor IX &lt; 2 IU/dL or ≤ 2% of normal, as confirmed by blood coagulation testing; AND</li> </ol>
<ul> <li>5. Contraindication, intolerance, or history of failure to continuous routine factor IX prophylaxis with greater than 150 prior exposure days to factor IX therapy. Failure to continuous routine factor IX prophylaxis is defined as one of the following: <ul> <li>a. Current or historical life-threatening hemorrhage;</li> <li>OR</li> <li>b. History of repeated, serious spontaneous bleeding</li> </ul> </li> </ul>
episodes; <b>AND</b> 6. Documentation demonstrating patient is <u>negative</u> for Factor IX inhibitor titers; <b>AND</b>
<ol> <li>Patient has a baseline anti-AAV5 antibody titer of ≤ 1:678 measured by ELISA; AND</li> </ol>
<ol> <li>Factor IX prophylaxis therapy will be discontinued upon achieving Factor IX levels of at least 5% following treatment; AND</li> </ol>
<ol> <li>Documentation is submitted that includes the following:</li> <li>a. The client's current weight; AND</li> </ol>
b. Liver function tests within the past 3 months.
If ALL criteria are met, the request will be authorized for a single one- time dose within <b>12 months</b> of the date of approval.
Criteria (Reauthorization)
Etranacogene dezaparvovec (Hemgenix) may be approved for <b>one dose</b>
only and <u>cannot be renewed</u> .

## Dosage and quantity limits

Drug	Indication	Approved Dose	Dosage Form and Quantity Limit
etranacogene dezaparvovec (Hemgenix)	Hemophilia B (congenital factor IX deficiency)	2 x 10 <sup>13</sup> genome copies (gc)/kg (or 2 mL/kg) administered as an intravenous infusion	1 kit per lifetime (see weight chart in Appendix)

## Coding:

HCPCS Code	Description
J1411	Injection, etranacogene dezaparvovec-drlb, per therapeutic dose

## Background:

Antihemophilic Products- Hemgenix

Congenital Factor IX deficiency, also known as hemophilia B, is an X-linked disorder that predominantly impacts males and manifests as bleeding from impaired hemostasis and bleeding-related complications. Hemophilia B is categorized by disease severity, which is determined by the amount of clotting factor in the blood. Severe disease is defined as Factor IX levels < 1 IU/dL or <1% of normal. Moderate disease is defined by a Factor IX level of 1-5 IU/dL or 1%-5% of normal and mild disease is 5-40 IU/dL or 5%-40% of normal. The current standard of care for hemophilia B is to replace the deficient coagulation factor either through episodic ("on demand") treatment given at the time of bleeding, or through continuous prophylaxis to prevent bleeding. Those who are on a prophylactic regimen may still require on-demand treatment. Gene therapy for hemophilia B aims to eliminate the need for Factor IX replacement. Etranacogene dezaparvovec (Hemgenix) is given as a single dose by intravenous infusion that delivers an AAV5 vector containing a copy of the gene that encodes for Factor IX. Although short-term results are promising and demonstrate a reduction in Factor IX replacement doses post-infusion, the durability of response has not been established and patients still may need periodic Factor IX treatment. Long-term extension trials are ongoing to establish the durability of response.

### References

- 1. Hemgenix [prescribing information]. King of Prussia, PA; CSL Behring, LLC., November 2022.
- Von Drygalski A, Giermasz A, Castaman G, et al. Etranacogene dezaparvovec (AMT-061 phase 2b): normal/near normal FIX activity and bleed cessation in hemophilia B. Blood Adv. 2019;3(21):3241-3247. doi:10.1182/bloodadvances.2019000811
- 3. activity and bleed cessation in hemophilia B. Blood Adv. 2019;3(21):3241-3247. doi:10.1182/bloodadvances.2019000811
- MASAC recommendations concerning products licensed for the treatment of hemophilia and other bleeding disorders. National Hemophilia Foundation. MASAC Document #263; August 2020. Available at: <u>http://www.hemophilia.org</u>.
- 5. Guidelines for the Management of Hemophilia. 3rd Edition. World Federation of Hemophilia 2020. Available at: <u>https://www1.wfh.org/publications/files/pdf-1863.pdf</u>.
- 6. MASAC recommendation concerning prophylaxis. 2016 National Hemophilia Foundation. MASAC Document #241; February 2016. Available at: <u>http://www.hemophilia.org</u>.

### History

Approved Date	Effective Date	Version	Action and Summary of Changes
MM/DD/YYY	MM/DD/YYYY	XX.XX.XX-X	New policy created Pending Approval (draft/unpublished version)

## Appendix

Multi-Vial Kits			
Total Number of Vials per Kit	Patient Body Weight (Kg)	Total Volume per Kit (mL)	NDC Number
10	46-50	100	00053-0100-10
11	51-55	110	00053-0110-11
12	56-60	120	00053-0120-12
13	61-65	130	00053-0130-13
14	66-70	140	00053-0140-14
15	71-75	150	00053-0150-15

Antihemophilic Products- Hemgenix

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<b>48</b> 236-240 480 00053-0480-48	47	231-235	470	00053-0470-47
	48	236-240	480	00053-0480-48

# Washington State Health Care Authority Antihemophilic (Hemgenix)

Please provide the information below, please print your answer, attach supporting documentation, sign, date, and return to our office as soon as possible. Without this information, we may deny the request in seven (7) working days.

Date of requ	lest:	Reference #:	ference #: MAS:			
Patient		Date of birth	ProviderOne ID			
Pharmacy na	ame	Pharmacy NPI	Teleph	one number	Fax number	
Prescriber		Prescriber NPI	Teleph	one number	Fax number	
Medication	and strength		Dire	ections for use		Qty/Days supply
<ol> <li>Indicate patient's diagnosis:</li> <li>Moderately severe or severe congenital Factor IX deficiency (hemophilia B) defined by factor IX &lt; 2 IU/dL or ≤ 2% of normal (confirmed by blood coagulation testing)</li> <li>Other. Specify:</li> </ol>					B) defined by factor IX < 2	
2.	Has patient received	prior gene therapy?	Yes [	No		
3.	Is this prescribed by o	or in consultation with a	hemato	ologist or spe	cialist in hemo	philia? 🗌 Yes 🗌 No
4.	Does patient have a contraindication tofactor IX prophylaxis? 🗌 Yes 🗌 No					
5.	Does patient have an intolerance or failure using continuous routine factor IX prophylaxis with more than 150 prior exposure days to factor IX therapy? 🗌 Yes 🗌 No					
6.	Has patient had one or more of the following while receiving continuous routine factor IX prophylaxis? Check all that apply: Current or historical life-threatening hemorrhage History of repeated, serious spontaneous bleeding episodes					
7.	Does the patient have clinical documentation demonstrating the following? Check all that apply: Patient is negative for Factor IX inhibitor titers Patient has a baseline anti-AAV5 antibody titer of ≤ 1:678 measured by ELISA					
8.	. Will Factor IX prophylaxis therapy be discontinued once the patient has achieved Factor IX levels of at least 5% following treatment? Yes No					
9.	. Has patient had a liver function test within the last 3 months? 🗌 Yes 🗌 No					
10.	Patient weight (kg): _		Date	taken:		_

The following are required with this request:			
Liver function test within the last 3 months			
Chart notes, labs and testing documenting diagnosis			
Prescriber signature	Prescriber specialty	Date	