Washington State Health Care Authority

Antihemophilic Products – Etranacogene dezaparvovec-drlb (Hemgenix)

Medical policy no. 85.10.25.AA-1 Effective Date: Month, 1, Year

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: https://www.hca.wa.gov/assets/billers-and-providers/apple-health-preferred-drug-list.xlsx

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 deficiency) etranacogene dezaparvovec (Hemgenix)	etranacogene dezaparvovec (Hemgenix) may be approved when all of the following criteria are met: 1. Patient is 18 years of age or older; **AND** 2. Prescribed by, or in consultation with, a hematologist or specialist in hemophilia; **AND** 3. Patient has not received prior gene therapy; **AND**

	4. Diagnosis of moderately severe or severe congenital Factor IX deficiency (hemophilia B) defined by factor IX < 2 IU/dL or ≤ 2% of normal, as confirmed by blood coagulation testing; **AND** 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: a. Current or historical life-threatening hemorrhage; **OR** b. History of repeated, serious spontaneous bleeding episodes; **AND** 6. Documentation demonstrating patient is negative for Factor IX inhibitor titers; **AND** 7. Patient has a baseline anti-AAV5 antibody titer of ≤ 1:678 measured by ELISA; **AND** 8. Factor IX prophylaxis therapy will be discontinued upon achieving Factor IX levels of at least 5% following treatment; **AND** 9. Documentation is submitted that includes the following: a. The client's current weight; **AND** 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 **12 months** of the date of approval**.**
	Criteria (Reauthorization) Etranacogene dezaparvovec (Hemgenix) may be approved for **one dose** only and **cannot be renewed**.

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^{13} 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:

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.
2. 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
4. MASAC recommendations concerning products licensed for the treatment of hemophilia and other bleeding disorders. National Hemophilia Foundation. MASAC Document #263; August 2020. Available at: http://www.hemophilia.org.
5. Guidelines for the Management of Hemophilia. 3rd Edition. World Federation of Hemophilia 2020. Available at: https://www1.wfh.org/publications/files/pdf-1863.pdf.
6. MASAC recommendation concerning prophylaxis. 2016 National Hemophilia Foundation. MASAC Document #241; February 2016. Available at: http://www.hemophilia.org.

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

16	76-80	160	00053-0160-16
17	81-85	170	00053-0170-17
18	86-90	180	00053-0180-18
19	91-95	190	00053-0190-19
20	96-100	200	00053-0200-20
21	101-105	210	00053-0210-21
22	106-110	220	00053-0220-22
23	111-115	230	00053-0230-23
24	116-120	240	00053-0240-24
25	121-125	250	00053-0250-25
26	126-130	260	00053-0260-26
27	131-135	270	00053-0270-27
28	136-140	280	00053-0280-28
29	141-145	290	00053-0260-29
30	146-150	300	00053-0300-30
31	151-155	310	00053-0301-31
32	156-160	320	00053-0302-32
33	161-165	330	00053-0303-33
34	166-170	340	00053-0304-34
35	171-175	350	00053-0305-35
36	176-180	360	00053-0306-36
37	181-185	370	00053-0307-37
38	186-190	380	00053-0480-38
39	191-195	390	00053-0260-39
40	196-200	400	00053-0400-40
41	201-205	410	00053-0410-41
42	206-210	420	00053-0420-42
43	211-215	430	00053-0430-43
44	216-220	440	00053-0440-44
45	221-225	450	00053-0450-45
46	226-230	460	00053-0460-46
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 request:	Reference #:	MAS:	
Patient	Date of birth	ProviderOne ID	
Pharmacy name	Pharmacy NPI	Telephone number	Fax number
Prescriber	Prescriber NPI	Telephone number	Fax number
Medication and strength	Directions for use	Qty/Days supply	

1. Indicate patient's diagnosis:
   - ☐ Moderately severe or severe congenital Factor IX deficiency (hemophilia B) defined by factor IX < 2 IU/dL or ≤ 2% of normal (confirmed by blood coagulation testing)
   - ☐ Other. Specify: _____
2. Has patient received prior gene therapy? ☐ Yes ☐ No
3. Is this prescribed by or in consultation with a hematologist or specialist in hemophilia? ☐ 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