Generic Name: etranacogene dezaparvovec-drlb

Applicable Drugs: Hemgenix

Preferred: N/A

Non-preferred: etranacogene dezaparvovec-drlb (Hemgenix)

Date of Origin: 5/12/2024

Date Last Reviewed / Revised: 05/12/2024

PRIOR AUTHORIZATION CRITERIA

(May be considered medically necessary when criteria I through X are met)

I. Natal male aged ≥ 18 years old with a diagnosis of congenital hemophilia with moderately severe or severe factor IX (FIX) deficiency with documentation of one of the following (A, B, or C):
   A. Current use of FIX prophylaxis therapy with stable therapy for ≥ 2 months before request.
   B. Current or historical life-threatening hemorrhage.
   C. Repeated, serious spontaneous bleeding episodes.

II. Documented history of > 150 previous exposure days of FIX protein treatment.

III. No history of inhibitors to FIX (≥ 0.3 Bethesda units).

IV. No history of previous gene therapy for hemophilia B.

V. Documentation of baseline labs/testing results (A through G):
   A. Liver function assessments including liver enzyme testing (AST, ALT, ALP, and total bilirubin) hepatic ultrasound, and elastography to rule out significant hepatic fibrosis (stage 3 or 4) or cirrhosis.
   B. Hepatitis B serology to rule out active Hepatitis B infection.
   C. Hepatitis C serology to rule out active Hepatitis C infection.
   D. HIV screening and HIV-RNA levels (if applicable) to rule out active and virally unsuppressed HIV infection.
   E. FIX activity level ≤ 2% indicating moderately severe or severe FIX deficiency.
   F. Negative FIX inhibitor titer screening (< 0.3 Bethesda units).

VI. Must be prescribed by a board-certified hematologist.

VII. Must be administered by a qualified treatment site trained by CSL Behring.

VIII. Indication, dose, and monitoring plan are consistent with FDA labeling. The FDA-recommended dose of etranacogene dezaparvovec-drlb is 2x1013 genome copies per kg of body weight or 2mL/kg of body weight administered by IV infusion.

EXCLUSION CRITERIA

- Uncontrolled HIV infection.
- Advanced liver fibrosis (Metavir fibrosis score F3 or F4).
- Active hepatitis B infection or currently receiving Hepatitis B antiviral therapy.
- Active hepatitis C infection or currently receiving Hepatitis C antiviral therapy.

OTHER CRITERIA

- Documentation of an anti-adeno-associated viral vector serotype V5 (AAV5) titers ≤ 1:678 is strongly suggested. The test is accessible through CSL Behring.

QUANTITY / DAYS SUPPLY RESTRICTIONS

- Kit that contains the number of vials corresponding to the dosing requirement based on body weight/ 1 day (see Appendix Table 1).

APPROVAL LENGTH

- **Authorization:** Approve for 1 treatment course for a 6-month duration. Limit 1 treatment course per lifetime.
- **Re-Authorization:** N/A

APPENDIX

Table 1. Hemgenix Multi-Vial Kits

Total Number of Vials per Kit	Patient Body Weight (kg)	Total Volume per Kit (mL)	NDC Number
10	46-50	100	0053-0100-10
11	51-55	110	0053-0110-11
12	56-60	120	0053-0120-12
13	61-65	130	0053-0130-13
14	66-70	140	0053-0140-14
15	71-75	150	0053-0150-15
16	76-80	160	0053-0160-16
17	81-85	170	0053-0170-17
18	86-90	180	0053-0180-18
19	91-95	190	0053-0190-19
20	96-100	200	0053-0200-20
21	101-105	210	0053-0210-21
22	106-110	220	0053-0220-22
23	111-115	230	0053-0230-23

24	116-120	240	0053-0240-24
25	121-125	250	0053-0250-25
26	126-130	260	0053-0260-26
27	131-135	270	0053-0270-27
28	136-140	280	0053-0280-28
29	141-145	290	0053-0290-29
30	146-150	300	0053-0300-30
31	151-155	310	0053-0310-31
32	156-160	320	0053-0320-32
33	161-165	330	0053-0330-33
34	166-170	340	0053-0340-34
35	171-175	350	0053-0350-35
36	176-180	360	0053-0360-36
37	181-185	370	0053-0370-37
38	186-190	380	0053-0380-38
39	191-195	390	0053-0390-39
40	196-200	400	0053-0400-40
41	201-205	410	0053-0410-41
42	206-210	420	0053-0420-42
43	211-215	430	0053-0430-43
44	216-220	440	0053-0440-44
45	221-225	450	0053-0450-45
46	226-230	460	0053-0460-46
47	231-235	470	0053-0470-47
48	236-240	480	0053-0480-48

REFERENCES

1. Hemgenix. Prescribing information. CSL Behring LLC 2022. Accessed October 11, 2023. https://labeling.cslbehring.com/PI/US/Hemgenix/EN/Hemgenix-Prescribing-Information.pdf
2. Pipe SW, Leebeek FWG, Recht M, et al. Gene Therapy with etranacogene dezaparvovec for Hemophilia B. *N Engl J Med*. 2023;388(8):706-718. doi: 10.1056/NEJMoa2211644
3. Srivastava A, Santagostino E, Dougall A, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition. *Haemophilia.* 2020: 26(Suppl 6): 1-158. doi.org/10.1111/hae.14046
4. Hemgenix. Resources to support your practice. Hemgenix.com. Accessed October 11, 2023. https://www.hemgenix.com/hcp/support-and-resources/your-practice