Beqvez™ (fidanacogene elaparvovec-dzkt)
(Intravenous)

Document Number: IC-0755

Last Review Date: 01/06/2025
Date of Origin: 06/04/2024
Dates Reviewed: 06/2024, 01/2025

I. Length of Authorization

Coverage will be provided for one dose and may not be renewed.

II. Dosing Limits

Max Units (per dose and over time) [HCPCS Unit]:

- 1 billable unit for one dose

III. Initial Approval Criteria [1-12]

Submission of medical records (chart notes) related to the medical necessity criteria is REQUIRED on all requests for authorizations. Records will be reviewed at the time of submission. Please provide documentation related to diagnosis, step therapy, and clinical markers (i.e. genetic and mutational testing) supporting initiation when applicable. Please provide documentation via direct upload through the PA web portal or by fax.

Coverage is provided in the following conditions:

Hemophilia B (Congenital Factor IX Deficiency) †

- Patient is at least 18 years of age; **AND**
- Patient has a diagnosis of moderate to severe congenital factor IX deficiency (i.e., ≤2% of normal circulating factor IX), as confirmed by blood coagulation testing, for which the subject is on continuous routine factor IX prophylaxis, unless there is a contraindication or intolerance *(Note: Continuous routine prophylaxis is defined as the intent of treating with an a priori defined frequency of infusions (e.g., twice weekly, once every two weeks, etc.) as documented in the medical records)*; **AND**
- Patient has not received prior hemophilia AAV-vector–based gene therapy (e.g., etranacogene dezaparvovec); **AND**
- Patient has one or more of the following:
    - Currently use Factor IX prophylaxis therapy (e.g., AlphaNine SD, Alprolix, BeneFIX, Idelvion, Ixinity, Mononine, Profilnine, Rebinyn, Rixubis, etc.); **OR**
    - Have current or historical life-threatening hemorrhage; **OR**

- Have repeated, serious spontaneous bleeding episodes, *(e.g., intramuscular hematomas requiring hospitalization, hemarthrosis, central nervous system (CNS) bleeding (including intracranial hemorrhage), pulmonary hemorrhage, life-threatening gastrointestinal (GI) hemorrhage and umbilical cord bleeding)*; **AND**

- Patient has been tested and found negative for Factor IX inhibitor titers (i.e., <0.6 Bethesda Units) and does not have a prior history of inhibitors *(Note: if test result is positive, re-test within approximately 2 weeks. If re-test is also positive, fidanacogene elaparvovec should not be given)*; **AND**
- Patient Factor IX activity will be monitored periodically (e.g., weekly for 3 months) as well as presence of inhibitors if bleeding is not controlled *(Note: patients will continue to require exogenous Factor IX until response to fidanacogene elaparvovec occurs)*; **AND**
- Patient will discontinue Factor IX prophylaxis therapy upon achieving FIX levels of 5% from fidanacogene elaparvovec treatment; **AND**
- Patient is adeno-associated virus serotype Rh74var capsid (AAVRh74var) neutralizing antibody negative as determined by an FDA-approved or CLIA-compliant test❖; **AND**
- Patient will have baseline liver function assessed prior to and after therapy according to the monitoring schedule outlined in the product labeling with corticosteroids administered in response to elevations; **AND**
- Patients with preexisting risk factors for hepatocellular carcinoma *(e.g., patients with cirrhosis, advanced hepatic fibrosis, hepatitis C or B, non-alcoholic fatty liver disease (NAFLD), chronic alcohol consumption, non-alcoholic steatohepatitis (NASH), and advanced age)* will have abdominal ultrasound screenings and be monitored regularly (e.g., annually) for alpha-fetoprotein (AFP) elevations following administration; **AND**
- Patient does not have current liver-related coagulopathy, hypoalbuminemia, persistent jaundice, or cirrhosis), portal hypertension, splenomegaly, hepatic encephalopathy, hepatic fibrosis, or active viral hepatitis; **AND**
- Patient has been tested for HIV and does not have an active infection (*i.e., either CD4+ cell count <200 mm^3 or viral load ≥20 copies/mL in cases of serological evidence of HIV-1 or HIV-2 infection)*; **AND**
- Patient has been counseled on avoidance of potentially hepatoxic substances (e.g., alcohol) which may reduce the efficacy of fidanacogene elaparvovec

Notes:

- Monitor Factor IX activity levels as outlined in the prescribing information to confirm adequate endogenous Factor IX activity levels to support discontinuation of pre-infusion Factor IX prophylaxis therapy.
- Exogenous Factor IX or other hemostatic products may also be required in case of surgery, invasive procedures, trauma, or bleeds in the event that fidanacogene elaparvovec-derived Factor IX activity is deemed insufficient for adequate hemostasis in such situations.
- Use of exogenous Factor IX concentrates before and after fidanacogene elaparvovec administration may impede assessment of endogenous, fidanacogene elaparvovec-derived Factor IX activity.

❖ If confirmed using an immunotherapy assay-*http://www.fda.gov/companiondiagnostics*

† FDA Approved Indication(s); ‡ Compendia Recommended Indication(s); Φ Orphan Drug

IV. Renewal Criteria

- Duration of authorization has not been exceeded (refer to Section I)

V. Dosage/Administration [1]

Indication	Dose
Hemophilia B (Congenital Factor IX Deficiency)	The recommended dose of Beqvez is a single-dose intravenous infusion of 5×10^{11} vector genomes per kg (vg/kg) of body weight. **Calculate patient's dose weight:** – Dosing is based on the patient's body mass index (BMI) in kg/m^2 **Patient's BMI** / **Patient's Dose Weight** ≤30 kg/m^2 / Dose Weight=Actual body weight >30 kg/m^2 / Determine using the following calculation: Dose Weight (kg) = 30 kg/m^2 x [Height (m)]^2 **Calculation of patient's dose volume in mL:** – Dose weight in kilograms (kg) divided by 20 = dose in mL (The division factor 20 represents the amount of vector genomes per mL of the Beqvez suspension (1×10^{13} vg/mL) divided by the per kilogram dose (5×10^{11} vg/kg))
• *Beqvez contains genetically modified vectors. Personal protective equipment (including gloves, safety goggles, laboratory coat and sleeves) should be worn while preparing or administering.* • *Confirm that the patient's identity matches the patient-specific identifier number on the outer carton.* • *Store in the original package to avoid direct sunlight and ultraviolet light exposure.* • *Thaw Beqvez vials for 1 hour at room temperature 15 °C to 30 °C (59 °F to 86 °F) in the upright orientation in the inner carton. Vials may be gently swirled but not shaken or inverted.* • *DO NOT administer as an intravenous push or bolus.* • *DO NOT infuse the diluted suspension in the same intravenous line with any other products.* • *DO NOT use a central line or port.*	

VI. Billing Code/Availability Information

HCPCS code(s):

- J1414 – Injection, fidanacogene elaparvovec-dzkt, per therapeutic dose; 1 billable unit = 1 kit (based on weight chart below) *(Effective 01/01/2025)*
- J3590 – Unclassified biologics *(Discontinue use on 01/01/2025)*
- C9172 – Injection, fidanacogene elaparvovec-dzkt, per therapeutic dose; 1 billable unit = 1 kit (based on weight chart below) *(Discontinue use on 01/01/2025)*

NDC(s):

Beqvez Multi-Vial kit sizes:

Patient Dose Weight (kg)	Total number of vials per Kit	NDC

≤75	4	00069-2004-04
>75 to ≤95	5	00069-2005-05
>95 to ≤115	6	00069-2006-06
>115 to ≤135	7	00069-2007-07

VII. References

1. Beqvez [package insert]. New York, NY; Pfizer, Inc., April 2024. Accessed November 2024.
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3. Guidelines for the Management of Hemophilia. 3rd Edition. World Federation of Hemophilia 2020. Available at: https://www1.wfh.org/publications/files/pdf-1863.pdf. Accessed November 2024.
4. Graham A1, Jaworski K. Pharmacokinetic analysis of anti-hemophilic factor in the obese patient. Haemophilia. 2014 Mar;20(2):226-9.
5. Croteau SE1, Neufeld EJ. Transition considerations for extended half-life factor products. Haemophilia. 2015 May;21(3):285-8.
6. Mingot-Castellano, et al. Application of Pharmacokinetics Programs in Optimization of Haemostatic Treatment in Severe Hemophilia a Patients: Changes in Consumption, Clinical Outcomes and Quality of Life. Blood. 2014 December; 124 (21).
7. MASAC Recommendation Concerning Prophylaxis For Hemophilia A And B With And Without Inhibitors. National Hemophilia Foundation. MASAC Document #267 (Replaces Document #241); April 2022. Available at: https://www.bleeding.org/sites/default/files/document/files/267_Prophylaxis.pdf. Accessed November 2024.
8. Rayment R, Chalmers E, Forsyth K, et al. Guidelines on the use of prophylactic factor replacement for children and adults with Haemophilia A and B. B J Haem:190;5, Sep 2020. https://doi.org/10.1111/bjh.16704.
9. Peyvandi F, Palla R, Menegatti M, et al. Coagulation factor activity and clinical bleeding severity in rare bleeding disorders: results from the European Network of Rare Bleeding Disorders. *J Thromb Haemost*. 2012;10:615-621.
10. MASAC Recommendations on Hemophilia Treatment Center Preparedness for Delivering Gene Therapy for Hemophilia. National Hemophilia Foundation. MASAC Document #282. October 2023. Available at: https://www.bleeding.org/healthcare-professionals/guidelines-on-care/masac-documents/masac-document-282-masac-recommendations-on-hemophilia-treatment-center-preparedness-for-delivering-gene-therapy-for-hemophilia. Accessed November 2024.
11. Thornburg, C.D., Simmons, D.H., von Drygalski, A. Evaluating gene therapy as a potential paradigm shift in treating severe hemophilia. BioDrugs. 2023. DOI: 10.1007/s40259-023-00615-4.

12. Klamroth R, Cuker A, Alzahrani H, et al. Efficacy and Safety of Fidanacogene Elaparvovec in Adults with Moderately Severe or Severe Hemophilia B: Results from the Phase 3 BENEGENE-2 Gene Therapy Trial. Hamostaseologie 2024; 44(S 01): S81-S82. DOI: 10.1055/s-0044-1779185.

Appendix 1 – Covered Diagnosis Codes

ICD-10	ICD-10 Description
D67	Hereditary factor IX deficiency

Appendix 2 – Centers for Medicare and Medicaid Services (CMS)

The preceding information is intended for non-Medicare coverage determinations. Medicare coverage for outpatient (Part B) drugs is outlined in the Medicare Benefit Policy Manual (Pub. 100-2), Chapter 15, §50 Drugs and Biologicals. In addition, National Coverage Determinations (NCDs) and/or Local Coverage Determinations (LCDs) may exist and compliance with these policies is required where applicable. Local Coverage Articles (LCAs) may also exist for claims payment purposes or to clarify benefit eligibility under Part B for drugs which may be self-administered. The following link may be used to search for NCD, LCD, or LCA documents https://www.cms.gov/medicare-coverage-database/search.aspx. Additional indications, including any preceding information, may be applied at the discretion of the health plan.

Medicare Part B Covered Diagnosis Codes (applicable to existing NCD/LCA/LCD): N/A

Medicare Part B Administrative Contractor (MAC) Jurisdictions		
Jurisdiction	**Applicable State/US Territory**	**Contractor**
E (1)	CA, HI, NV, AS, GU, CNMI	Noridian Healthcare Solutions, LLC
F (2 & 3)	AK, WA, OR, ID, ND, SD, MT, WY, UT, AZ	Noridian Healthcare Solutions, LLC
5	KS, NE, IA, MO	Wisconsin Physicians Service Insurance Corp (WPS)
6	MN, WI, IL	National Government Services, Inc. (NGS)
H (4 & 7)	LA, AR, MS, TX, OK, CO, NM	Novitas Solutions, Inc.
8	MI, IN	Wisconsin Physicians Service Insurance Corp (WPS)
N (9)	FL, PR, VI	First Coast Service Options, Inc.
J (10)	TN, GA, AL	Palmetto GBA
M (11)	NC, SC, WV, VA (excluding below)	Palmetto GBA
L (12)	DE, MD, PA, NJ, DC (includes Arlington & Fairfax counties and the city of Alexandria in VA)	Novitas Solutions, Inc.
K (13 & 14)	NY, CT, MA, RI, VT, ME, NH	National Government Services, Inc. (NGS)
15	KY, OH	CGS Administrators, LLC