Medical Policy

Utilization Management and Clinical Medical Policy

Policy Name:	Policy Number:	Scope:	Origination Date:	Effective Date:
Fidanacogene elaparvovec-dzkt (BEQVEZ)	**MP-RX-FP-170-25**	☒ **MMM MA** ☒ **MMM MultiHealth**	**8/8/2025** **Last Review Date:** **5/6/2026**	**5/6/2026** **Frequently Revision:** **Annual**

Service Category:

☐ Anesthesia
☐ Surgery
☐ Radiology Procedures
☐ Pathology and Laboratory Procedures
☐ Medicine Services and Procedures
☐ Evaluation and Management Services
☐ DME/Prosthetics or Supplies
☒ Other: Part B Drugs

Service Description:

This document addresses the use of Fidanacogene elaparvovec-dzkt (BEQVEZ), a drug approved by the Food and Drug Administration (FDA) for the treatment of adults with moderately severe to severe hemophilia B (congenital factor IX deficiency), who currently use factor IX prophylaxis therapy, or have current or historical life-threatening hemorrhage, or have repeated, serious spontaneous bleeding episodes, and do not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid as detected by an FDA-approved test. Select patients for therapy based on an FDA-approved companion diagnostic for BEQVEZ.

Background Information:

Fidanacogene elaparvovec-dzkt is an adeno-associated virus (AAV) vector-based gene therapy designed to introduce a functional copy of the factor IX gene encoding a high-activity factor IX variant (FIX-R338L, also known as FIX-Padua), resulting in continuous endogenous factor IX expression after a one-time intravenous infusion. In the pivotal phase 3 BENEGENE-2 study, fidanacogene elaparvovec was superior to prophylaxis, with the annualized rate of bleeding for all bleeding episodes decreasing by 71%, from 4.42 at baseline to 1.28 after gene therapy. The most common adverse reaction (incidence ≥5%) reported in clinical studies was increased transaminases.

In the pivotal phase 3 open-label trial (BENEGENE-2), a single intravenous dose of 5×10^{11} vector genomes/kg led to a 71% reduction in annualized bleeding rate (from 4.42 to 1.28 episodes/year) compared to prior prophylaxis, demonstrating both noninferiority and superiority. Mean factor IX activity at 15 months was 26.9% (median 22.9%), with most patients achieving levels in the mild hemophilia range. No thrombotic events, inhibitor development, or malignancies were observed. The most common adverse event was transient elevation of aminotransferases, managed with glucocorticoids in 62% of participants.[2]

Long-term follow-up (median 5.5 years, range 3–6) confirms durable efficacy and safety, with sustained factor IX activity, low annualized bleeding rates (<1), and no new safety signals. Liver enzyme elevations and hepatic steatosis were observed, primarily in patients with metabolic risk factors, but no cases of hepatocellular carcinoma or vector-related malignancy have been reported. Exogenous factor IX was used perioperatively without unexpected bleeding.[3]

Assay variability in measuring FIX-R338L activity is recognized, with one-stage silica-based assays yielding higher values than chromogenic or ellagic acid-based assays. This should be considered in clinical monitoring.[4]

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Overall, fidanacogene elaparvovec-dzkt offers a single-administration, durable gene therapy option for eligible adults with hemophilia B, significantly reducing bleeding and treatment burden, with a favorable safety profile to date. [2-3][5][4]

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Medical Necessity Guidelines:

When a drug is being reviewed for coverage under a member's medical benefit plan or is otherwise subject to clinical review (including prior authorization), the following criteria will be used to determine whether the drug meets any applicable medical necessity requirements for the intended/prescribed purpose.

BEQVEZ (Fidanacogene elaparvovec-dzkt)

A. Criteria For Initial Approval (*Provider must submit documentation [such as office chart notes, lab results, pathology reports, imaging studies, and any other pertinent clinical information] supporting the patient's diagnosis for the drug and confirming that the patient has met **all** approval criteria.)*

i. Individual is 18 years of age or older; **AND**

ii. Individual is diagnosed with moderate to severe hemophilia B (congenital factor IX deficiency), and meets at least one of the following:
   a. Currently use factor IX prophylaxis therapy; **OR**
   b. Has current or historical life-threatening hemorrhage; **OR**
   c. Has repeated, serious spontaneous bleeding episodes; **AND**
   d. Does not have neutralizing antibodies to adeno-associated virus serotype Rh74var (AAVRh74var) capsid as detected by an FDA-approved test; **AND**
   e. Individual does not have active factor IX inhibitors (defined as a positive test of ≥0.6 Bethesda Units [BU]) and does not have a prior history of factor IX inhibitors; **AND**
   f. Individual does not have hypersensitivity to factor IX replacement product; **AND**
   g. Individual does not currently experience liver-related coagulopathy, hypoalbuminemia, persistent jaundice, or cirrhosis), portal hypertension, splenomegaly, hepatic encephalopathy, hepatic fibrosis, or active viral hepatitis; **AND**

iii. Individual has had the appropriate liver health assessment which includes:
   a. Liver function tests (alanine transaminase [ALT], aspartate transaminase [AST], alkaline phosphatase [ALP], bilirubin, albumin); **AND**
   b. Laboratory tests for active hepatitis B or C; **AND**
   c. Elastography and/or ultrasound and other laboratory assessments for liver fibrosis; **AND**

iv. Individual must not have either a CD4+ cell count <200mm^3 or viral load ≥20 copies/mL in case of serological evidence of HIV-1 or HIV-2 infection.

B. Criteria For Continuation of Therapy

i. Fidanacogene elaparvovec-dzkt is intended as a one-time therapy and repeat or continuous dosing is not supported or recommended.

C. Authorization Duration

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i. Initial Approval Duration: One time approval.
ii. Reauthorization Approval Duration: N/A

D. Conditions Not Covered

Any other use is considered experimental, investigational, or unproven, including the following (this list may not be all inclusive):

i. Requests for *BEQVEZ* may not be approved when the above criteria (Section A: Criteria for Initial Approval) are not met and for all other indications.

ii. Fidanacogene elaparvovec-dzkt (BEQVEZ) should not be administered to patients with either CD4+ cell count <200mm3 or viral load ≥20 copies/mL in case of serological evidence of HIV-1 or HIV-2 infection.

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Limits or Restrictions:

A. Therapeutic Alternatives

The list below includes preferred alternative therapies recommended in the approval criteria and may be subject to prior authorization.

i. N/A

B. Quantity Limitations

Approvals may be subject to dosing limits in accordance with FDA-approved labeling, accepted compendia, and/or evidence-based practice guidelines. The chart below includes dosing recommendations as per the FDA-approved prescribing information.

Drug	Recommended Dosage
BEQVEZ (fidanacogene elaparvovec-dzkt) injection	The recommended dose of BEQVEZ is 5×10^{11} vector genomes per kg (vg/kg) of body weight. Dose based on adjusted body weight. - For individuals with a BMI >30 kg/m^2 dosing should be based on dose weight calculated as 30 kg/m2 × [height (m)]^2

Codes Information:

The following list(s) of procedure and/or diagnosis codes is provided for reference purposes only and may not be all inclusive. Inclusion or exclusion of a procedure, diagnosis or device code(s) does not constitute or imply member coverage or provider reimbursement policy. Benefit coverage for health services is determined by the member specific benefit plan document and applicable laws that may require coverage for a specific service. The inclusion of a code does not imply any right to reimbursement or guarantee claim payment. Other Policies and Guidelines may apply.

ICD-10 Diagnostic Codes:

Codes	Description
D67	Hereditary factor IX deficiency

HCPCS Codes:

Codes	Description
J1414	Injection, fidanacogene elaparvovec-dzkt, per therapeutic dose

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Reference Information:

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2. Cuker, A., Kavakli, K., Frenzel, L., Wang, J., Astermark, J., Cerqueira, M. H., Iorio, A., Katsarou-Fasouli, O., Klamroth, R., Shapiro, A. D., Hermans, C., Ishiguro, A., Leavitt, A. D., Oldenburg, J. B., Ozelo, M. C., Teitel, J., Biondo, F., Fang, A., Fuiman, J., . . . Rupon, J. (2024). Gene Therapy with Fidanacogene Elaparvovec in Adults with Hemophilia B. New England Journal of Medicine, 391(12), 1108–1118. https://doi.org/10.1056/nejmoa2302982
3. Rasko, J. E., Samelson-Jones, B. J., George, L. A., Giermasz, A., Ducore, J. M., Teitel, J. M., McGuinn, C. E., High, K. A., De Jong, Y. P., Chhabra, A., O'Brien, A., Smith, L. M., Winburn, I., & Rupon, J. (2025). Fidanacogene elaparvovec for hemophilia B — a multiyear follow-up study. New England Journal of Medicine, 392(15), 1508–1517. https://doi.org/10.1056/nejmoa2307159
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5. Northington MW, Rice SE, Holmes AL, Watts Alexander CS. Gene-ius at work: Hemophilia B treatment enters a new era. Am J Health Syst Pharm. 2025 Jan 27:zxaf005. doi: 10.1093/ajhp/zxaf005. Epub ahead of print. PMID: 39868419.
6. Cuker, A., Kavakli, K., Frenzel, L., Wang, J., Astermark, J., Cerqueira, M. H., Iorio, A., Katsarou-Fasouli, O., Klamroth, R., Shapiro, A. D., Hermans, C., Ishiguro, A., Leavitt, A. D., Oldenburg, J. B., Ozelo, M. C., Teitel, J., Biondo, F., Fang, A., Fuiman, J., . . . Rupon, J. (2024). Gene therapy with fidanacogene elaparvovec in adults with hemophilia B. *New England Journal of Medicine, 391*(12), 1108–1118. doi:10.1056/NEJMoa2302982.
7. Pfizer Inc. (2024, April). *BEQVEZ (fidanacogene elaparvovec-dzkt) injection, for intravenous infusion: Full prescribing information*. Retrieved March 31, 2026.

Federal and state laws or requirements, contract language, and Plan utilization management programs or polices may take precedence over the application of this clinical criteria.

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Policy History:

Type of Review	Summary of Changes	P&T Approval Date	UM/CMPC Approval Date
Annual Review	Updated the policy to align with the current BEQVEZ prescribing information by refining the FDA indication language, adding the FDA-approved companion diagnostic requirement, strengthening eligibility criteria to exclude members with active or prior FIX inhibitors and hypersensitivity to FIX products, tightening the liver and HIV screening language, updating monitoring and safety details in the background section, and revising the dosing language to the current one-time IV dose with calculated dose weight for members with BMI greater than 30 kg/m². Administrative update to incorporate new policy template. Updated references.	5/1/2026	5/6/2026
Policy Inception	MMM Developed Medical Policy.	7/17/2025	8/8/2025