

Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy



Clinical Policy: Dalnacogene Ponparvovec (BBM-H901)

Reference Number: CP.PHAR.771

Effective Date: **FDA Approval Date**

Last Review Date: 05.26

Line of Business: Commercial, HIM, Medicaid

[Coding Implications](#)

[Revision Log](#)

See [Important Reminder](#) at the end of this policy for important regulatory and legal information.

Description

Dalnacogene ponparvovec (BBM-H901^{®/™}) is an adeno-associated virus (AAV) vector-based gene therapy.

FDA Approved Indication(s) **[Pending]**

BBM-H901 is indicated for the treatment of adults with hemophilia B (congenital factor IX deficiency).

Policy/Criteria

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

All requests reviewed under this policy **require Precision Drug Action Committee (PDAC) Utilization Management Review**. Refer to CC.PHAR.21 for process details.

It is the policy of health plans affiliated with Centene Corporation[®] that BBM-H901 is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria*

**Criteria will mirror the clinical information from the prescribing information once FDA-approved*

A. Congenital hemophilia B (must meet all):

1. Diagnosis of congenital hemophilia B (factor IX deficiency);*
2. Prescribed by or in consultation with a hematologist;
3. Age \geq 18 years;*
4. Member has severe or moderately severe hemophilia (defined as a factor IX level of \leq 2%);*
5. Member meets one of the following (a, b, or c):
 - a. Adherence to current use of a factor IX product* (e.g., Alprolix[®], Benefix[®], Idelvion[®], Ixinity[®], Rebinyn[®], Rixubis[®]) for routine prophylaxis as assessed and documented by prescriber;
 - b. Has current or historical life-threatening hemorrhage;
 - c. Has repeated, serious spontaneous bleeding episodes (*see Appendix D*);
6. Member has been treated with factor IX product for a minimum of 100 exposure days (*see Appendix D*);*

7. Member meets all of the following (a, b, and c):*
 - a. No previous documented history of a detectable factor IX inhibitor;
 - b. Documentation of inhibitor level assay < 0.6 Bethesda units (BU) within the last 12 months;
 - c. If member had an initial positive test result for factor IX inhibitor, member has documentation of a subsequent negative test within 2 weeks;
8. Member has had all of the following baseline liver assessments within the last 3 months (a, b, and c):*
 - a. Documentation of liver enzymes $\leq 1.5x$ the upper limit of normal (i.e., alanine aminotransferase [ALT], aspartate aminotransferase [AST], alkaline phosphatase [ALP], and total bilirubin);
 - b. Documentation of normal hepatic ultrasound and elastography;
 - c. If member has evidence of radiological liver abnormalities and/or sustained liver enzyme elevations, attestation from hepatologist that member is eligible for BBM-H901;
9. Member does not have a history of allergic reactions to immunoglobulin administration;*
10. Member has not received prior gene therapy;
11. Member has been tested for neutralizing anti-adenovirus serotype 843 (AAV843) antibodies and is deemed a suitable candidate for treatment;*
12. Documentation of member's body weight in kg;
13. Dose does not exceed a single IV infusion of 5×10^{12} vector genomes (vg) per kg.*

Approval duration: 3 months (1 dose only)

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Congenital Hemophilia B

1. Continued therapy will not be authorized as BBM-H901 is indicated to be dosed one time only.

Approval duration: Not applicable

B. Other diagnoses/indications (must meet 1 or 2):

1. If this drug has recently (within the last 6 months) undergone a label change (e.g., newly approved indication, age expansion, new dosing regimen) that is not yet reflected in this policy, refer to one of the following policies (a or b):
 - a. For drugs on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the no coverage criteria policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.33 for health insurance marketplace, and CP.PMN.255 for Medicaid; or
 - b. For drugs NOT on the formulary (commercial, health insurance marketplace) or PDL (Medicaid), the non-formulary policy for the relevant line of business: CP.CPA.190 for commercial, HIM.PA.103 for health insurance marketplace, and CP.PMN.16 for Medicaid; or
2. If the requested use (e.g., diagnosis, age, dosing regimen) is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized) AND criterion 1 above does not apply, refer to the off-label use policy for the relevant line of business: CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid.

III. Diagnoses/Indications for which coverage is NOT authorized:

- A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – CP.CPA.09 for commercial, HIM.PA.154 for health insurance marketplace, and CP.PMN.53 for Medicaid, or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

AAV: adeno-associated virus	BU: Bethesda units
ALP: alkaline phosphatase	ED: exposure day
ALT: alanine aminotransferase	FDA: Food and Drug Administration
AST: aspartate aminotransferase	vg: vector genomes

Appendix B: Therapeutic Alternatives

This table provides a listing of preferred alternative therapy recommended in the approval criteria. The drugs listed here may not be a formulary agent for all relevant lines of business and may require prior authorization.

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Factor IX recombinant products for routine prophylaxis		
Alprolix [®]	50 IU/dL/kg IV once weekly or 100 IU/dL/kg IV once every 10 days	100 IU/dL/kg/dose
BeneFIX [®]	100 IU/kg IV once weekly	100 IU/kg/dose
Idelvion [®]	25-40 IU/kg IV every 7 days followed by 50-75 IU/kg IV every 14 days once well-controlled	40 IU/kg/week
Ixinity [®]	40 to 70 IU/kg IV twice weekly	140 IU/kg/week

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Rebinyln [®]	40 IU/kg IV once weekly	40 IU/kg/week
Rixubis [®]	40-60 IU/kg IV twice weekly	60 IU/kg/dose

Therapeutic alternatives are listed as Brand name[®] (generic) when the drug is available by brand name only and generic (Brand name[®]) when the drug is available by both brand and generic.

Appendix C: Contraindications/Boxed Warnings [Pending]

- Contraindication(s): **pending**
- Boxed warning(s): **pending**

Appendix D: General Information

- Serious bleeding episodes include bleeds in the following sites: intracranial; neck/throat; gastrointestinal; joints (hemarthrosis); muscles (especially deep compartments such as the iliopsoas, calf, forearm), or mucous membranes of the mouth, nose, and genitourinary tract.
- Spontaneous bleed is defined as a bleeding episode that occurs without apparent cause and is not the result of trauma.
- Exposure day (ED): An ED is a day on which a person with hemophilia has been infused with factor concentrate to treat or prevent a bleed. The number of EDs consists only of those days on which factor was infused. A higher level of cumulative treatment increases the likelihood of immunologic stability – a decreased risk of producing inhibitors.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
Hemophilia B*	Recommended dose: 5 x 10 ¹² vg/kg of body weight by IV infusion*	5 x 10 ¹² vg/kg*

VI. Product Availability [Pending]

Pending

VII. References

1. BBM-H901 Prescribing Information. Pharma City, ST: Belief BioMed; Mth Yr. Available at: www.website.com.pdf. Accessed Month Day, Year.
2. Xue F, Ju M, Zhu T, et al. Factor IX-Padua AAV gene therapy in hemophilia B: phases 1/2 and 3 trials. *Nature Medicine*. January 2026;32:93-102.
3. Xue F, Li H, Wu X, et al. Safety and activity of an engineered, liver-tropic adeno-associated virus vector expressing a hyperactive Padua factor IX administered with prophylactic glucocorticoids in patients with haemophilia B: a single-centre, single-arm, phase 1, pilot trial. *Lancet Haematology*. July 2022;9(7):e504-13.
4. Srivastava A, Santagostino E, Dougall A, et al. WFH Guidelines for the Management of Hemophilia, 3rd edition. *Haemophilia*. 2020 Aug;26 Suppl 6:1-158.
5. Carcao M and Goudemand J. Inhibitors in hemophilia: A primer, 5th edition. World Federation of Hemophilia. Available at: <https://www1.wfh.org/publication/files/pdf-1122.pdf>.

6. Medical and Scientific Advisory Council (MASAC) of the National Bleeding Disorders Foundation (formerly National Hemophilia Foundation): Database of treatment guidelines. Available at: <https://www.hemophilia.org/healthcare-professionals/guidelines-on-care/masac-documents>.
7. Rezende SM, Neumann I, Angchaisuksiri P, et al. International Society on Thrombosis and Haemostasis clinical practice guideline for treatment of congenital hemophilia A and B based on the Grading of Recommendations Assessment, Development, and Evaluation methodology. *J Thromb Haemost.* 2024;22(9):2629-52.

Coding Implications [Pending]

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
Pending	Pending

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively	02.24.26	05.26

Important Reminder

This clinical policy has been developed by appropriately experienced and licensed health care professionals based on a review and consideration of currently available generally accepted standards of medical practice; peer-reviewed medical literature; government agency/program approval status; evidence-based guidelines and positions of leading national health professional organizations; views of physicians practicing in relevant clinical areas affected by this clinical policy; and other available clinical information. The Health Plan makes no representations and accepts no liability with respect to the content of any external information used or relied upon in developing this clinical policy. This clinical policy is consistent with standards of medical practice current at the time that this clinical policy was approved. “Health Plan” means a health plan that has adopted this clinical policy and that is operated or administered, in whole or in part, by Centene Management Company, LLC, or any of such health plan’s affiliates, as applicable.

The purpose of this clinical policy is to provide a guide to medical necessity, which is a component of the guidelines used to assist in making coverage decisions and administering benefits. It does not constitute a contract or guarantee regarding payment or results. Coverage decisions and the administration of benefits are subject to all terms, conditions, exclusions, and limitations of the coverage documents (e.g., evidence of coverage, certificate of coverage, policy, contract of insurance, etc.), as well as to state and federal requirements and applicable Health Plan-level administrative policies and procedures.

This clinical policy is effective as of the date determined by the Health Plan. The date of posting may not be the effective date of this clinical policy. This clinical policy may be subject to

applicable legal and regulatory requirements relating to provider notification. If there is a discrepancy between the effective date of this clinical policy and any applicable legal or regulatory requirement, the requirements of law and regulation shall govern. The Health Plan retains the right to change, amend or withdraw this clinical policy, and additional clinical policies may be developed and adopted as needed, at any time.

This clinical policy does not constitute medical advice, medical treatment, or medical care. It is not intended to dictate to providers how to practice medicine. Providers are expected to exercise professional medical judgment in providing the most appropriate care, and are solely responsible for the medical advice and treatment of members. This clinical policy is not intended to recommend treatment for members. Members should consult with their treating physician in connection with diagnosis and treatment decisions.

Providers referred to in this clinical policy are independent contractors who exercise independent judgment and over whom the Health Plan has no control or right of control. Providers are not agents or employees of the Health Plan.

This clinical policy is the property of the Health Plan. Unauthorized copying, use, and distribution of this clinical policy or any information contained herein are strictly prohibited. Providers, members, and their representatives are bound to the terms and conditions expressed herein through the terms of their contracts. Where no such contract exists, providers, members and their representatives agree to be bound by such terms and conditions by providing services to members and/or submitting claims for payment for such services.

Note:

For Medicaid members, when state Medicaid coverage provisions conflict with the coverage provisions in this clinical policy, state Medicaid coverage provisions take precedence. Please refer to the state Medicaid manual for any coverage provisions pertaining to this clinical policy.

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