Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy

CENTENE® Corporation

Clinical Policy: Mozafancogene Autotemcel (RP-L102)

Reference Number: CP.PHAR.719
Effective Date: FDA Approval Date
Last Review Date: 05.25
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

Mozafancogene autotemcel (RP-L102®/™) is an autologous hematopoietic stem cell-based gene therapy with a lentiviral vector encoding a functional copy of the Fanconi anemia complementation group A (*FANCA)* gene.

FDA Approved Indication(s) [Pending]

RP-L102 is indicated for the treatment of Fanconi anemia (FA) complementation group A.

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 medical director review**.

It is the policy of health plans affiliated with Centene Corporation® that RP-L102 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*
**Only for initial treatment dose; subsequent doses will not be covered.*

A. Fanconi Anemia (must meet all)**:**

1. Diagnosis of FA complementation group A as evidenced by both of the following (a and b);*
   a. *FANCA* gene mutation confirmed by genetic testing;
   b. One of the following (i or ii):
      i. Positive chromosome breakage test in peripheral blood;
      ii. Documentation of potential somatic mosaicism (e.g., negative, or equivocal chromosome breakage test) and medically significant decrease in at least one blood cell lineage over time (*see Appendix D*);
2. Prescribed by or in consultation with a transplant specialist, hematologist, or geneticist;
3. Age ≥ 1 year and ≤ 12 years;*
4. Weight ≥ 8 kg;*
5. One of the following (a or b):
   a. Member has no available human leukocyte antigen (HLA)-matched (i.e., full HLA-matching of all evaluated alleles) donor;

b. Member has an available HLA-matched donor, and both of the following (i and ii):
   i. Provider submits medical rationale that allogeneic hematopoietic stem cell transplantation (HSCT) is not feasible (e.g., donor unable to undergo donation procedure because of medical impairments);
   ii. Member understands the risks and benefits of alternative therapeutic options such as allogeneic HSCT;

6. Transplant specialist attestation that member is clinically stable and eligible to undergo myeloablative conditioning and HSCT;
7. Member has not received prior allogeneic HSCT;
8. Member has not received prior gene therapy;
9. Both of the following (a and b):
   a. Dose does not exceed a single administration;
   b. Dose contains a minimum of 3 x 10^5 CD34+ cells/kg.*

Approval duration: 6 months (one time infusion per lifetime)

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*

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

A. Fanconi Anemia

1. Continued therapy will not be authorized as RP-L102 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

CBT: chromosome breakage test
FA: Fanconi anemia
FANCA: Fanconi anemia complementation group A
FDA: Food and Drug Administration
HLA: human leukocyte antigen
HSCT: hematopoietic stem cell transplantation

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

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

Appendix D: General Information

- Somatic mosaicism can occur in T-lymphocytes and hematopoietic stem cells due to the reversion of an inherited variant in an FA gene. Mosaicism may be suspected if peripheral blood chromosome breakage tests were reported as negative or equivocal.
- Examples of medically significant decrease in at least one blood lineage over time: bone marrow failure, bone marrow hypocellular, disseminated intravascular coagulation, febrile neutropenia, leukopenia

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
FA complementation group A*	Minimum recommended dose: 3 x 10^5 CD34+ cells/kg*	Not applicable*

VI. Product Availability [Pending]

Pending

VII. References

1. Moxafancogene Autotemcel Prescribing Information.
2. ClinicalTrials.gov. Lentiviral-mediated gene therapy of Fanconi anemia patients subtype A (FANCOLEN-1). Available at: https://clinicaltrials.gov/study/NCT03157804. Accessed January 17, 2025.
3. ClinicalTrials.gov. A clinical trial to evaluate the safety of RP-L102 in pediatric subjects with Fanconi anemia subtype A. Available at: https://clinicaltrials.gov/study/NCT03814408. Accessed January 17, 2025.
4. Fanconi Anemia Research Fund. Fanconi Anemia Clinical Care Guidelines, Fifth Edition. Fanconi Anemia Research Fund. 2020. Available at: https://www.fanconi.org/images/uploads/other/Fanconi_Anemia_Clinical_Care_Guidelines_5thEdition_web.pdf. Accessed January 17, 2025.
5. Lasaga M, Río P, Vilas-Zornoza A, et al. Gene therapy restores the transcriptional program of hematopoietic stem cells in Fanconi anemia. *Haematologica*. 2023 Oct 1;108(10):2652-2663. doi: 10.3324/haematol.2022.282418.

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	02.11.25	05.25

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.