Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy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: Deramiocel (CAP-1002)

Reference Number: CP.PHAR.716
Effective Date: FDA Approval Date
Last Review Date: 05.26
Line of Business: Commercial, HIM/ICHRA, Medicaid

Coding Implications
Revision Log

See Important Reminder at the end of this policy for important regulatory and legal information.

Description

Deramiocel (CAP-1002) is an allogenic cardio-sphere-derived cell (CDC) therapy.

FDA Approved Indication(s) [Pending]

CAP-1002 is indicated for the treatment of patients with Duchenne muscular dystrophy (DMD) cardiomyopathy.

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 may **require medical director review.**

It is the policy of health plans affiliated with Centene Corporation® that CAP-1002 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. Duchenne Muscular Dystrophy (must meet all)**:**

1. Diagnosis of DMD confirmed by genetic testing;*
2. One of the following (a or b):
   a. Prescribed by or in consultation with a neurologist;
   b. Member is being treated at a certified Duchenne care center or an MDA care center (*see Appendix D*);*
3. Age ≥ 10 years;*
4. Member has a performance upper limb (PUL 2.0) entry item score of 2 – 5;*
5. One of the following (a or b):*
   a. Member is non-ambulatory;
   b. If member is late ambulatory (i.e., ability to walk with or without assistive devices, not wheelchair dependent) defined as a 10-meter walk time of > 10 seconds, age is < 18 years;
6. Member meets both of the following assessed within the last 30 days (a and b):*
   a. Stable cardiac function with left ventricular ejection fraction (LVEF) ≥ 35%;
   b. Stable pulmonary function with predicted forced vital capacity (FVC) ≥ 35%;

7. Member has been on a stable dose of an oral corticosteroid (e.g., prednisone, Emflaza®*, Agamree®*) for ≥ 6 months, unless contraindicated or clinically significant adverse effects are experienced;*
   **Prior authorization is required for Emflaza and Agamree*
8. CAP-1002 is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;*
9. Member has not received prior stem cell therapy;*
10. Member has not previously received gene replacement therapy for DMD (e.g., Elevidys®);*
11. If member is currently on an exon-skipping therapy (e.g., Amondys 45™, Exondys 51®, Viltepso™, Vyondys 53™), member must discontinue therapy prior to CAP-1002;
12. Dose does not exceed 150 million cells every 3 months.*

Approval duration: 6 months

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/ICHRA) 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/ICHRA, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) 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/ICHRA, 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/ICHRA, and CP.PMN.53 for Medicaid.

II. Continued Therapy*

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

A. Duchenne Muscular Dystrophy (must meet all)**:**

1. Member meets one of the following (a or b):
   a. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
   b. Member is currently receiving medication and is enrolled in a state and product with continuity of care regulations (*refer to state specific addendums for CC.PHARM.03A and CC.PHARM.03B)*;
2. Member is responding positively to therapy as evidenced by, including but not limited to, improvement or stabilization in member's PUL score, LVEF, and FVC ;*
3. Member has been assessed by a neurologist within the last 6 months;
4. CAP-1002 is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
5. Member has not received prior stem cell therapy;*

6. Member has not previously received gene replacement therapy for DMD (e.g., Elevidys);*
7. CAP-1002 is not prescribed concurrently with exon-skipping therapies (e.g., Amondys 45, Exondys 51, Viltepso, Vyondys 53);*
8. If request is for a dose increase, new dose does not exceed 150 million cells every 3 months.*

Approval duration: 6 months

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/ICHRA) 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/ICHRA, and CP.PMN.255 for Medicaid; or
   b. For drugs NOT on the formulary (commercial, health insurance marketplace/ICHRA) 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/ICHRA, 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/ICHRA, 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/ICHRA, and CP.PMN.53 for Medicaid or evidence of coverage documents.

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

CDC: cardio-sphere-derived cell
FDA: Food and Drug Administration
FVC: forced vital capacity
LVEF: left ventricular ejection fraction
PUL: performance of upper limb

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings [Pending]

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

Appendix D: General Information

- Corticosteroids are routinely used in DMD management with established efficacy in slowing decline of muscle strength and function (including motor, respiratory, and cardiac). They are recommended for all DMD patients per the American Academy of Neurology (AAN) and DMD Care Considerations Working Group; in addition, the AAN guidelines have been endorsed by the American Academy of Pediatrics, the American Association of Neuromuscular & Electrodiagnostic Medicine, and the Child Neurology Society.
  - The DMD Care Considerations Working Group guidelines, which were updated in 2018, continue to recommend corticosteroids as the mainstay of therapy.
- Prednisone is the corticosteroid with the most available evidence. A second corticosteroid commonly used is deflazacort, which was FDA approved for DMD in February 2017. On October 2023, a third corticosteroid, vamorolone, was approved by the FDA for DMD.
- Parent Project Muscular Dystrophy (PPMD)'s certified Duchenne care center program helps to ensure that centers comply with the standards of care and services established in the Duchenne care guidelines. The full list of certified Duchenne care centers can be found at: https://www.parentprojectmd.org/care/find-a-certified-duchenne-care-center/.
- The Muscular Dystrophy Association (MDA) care centers offers individuals with muscular dystrophy, ALS and other neuromuscular diseases to access expert multidisciplinary care, clinical trials, and to connect with MDA and the neuromuscular community. The full list of MDA care centers can be found at: https://www.mda.org/care/care-center-list.
- The performance of upper (PUL 2.0) scale includes an entry item to define the broad starting functional level, and 22 items subdivided into shoulder level (6 items), mid-level (9 items), and distal level (7 items).
  - The score on the PUL entry item determines the starting point for subsequent tests (whether to start at the shoulder- or middle-level), typically ranges from 0 to 6, with a lower score indicating greater upper limb impairment and a higher score signifying better function; a score of 0 represents "no useful hand function" while a score of 6 indicates "full shoulder abduction without weakness."
  - The 22-item scale grades performance of various functional skills on a scale of 0 (unable), 1 (completes independently but with modifications), and 2 (completed without compensation). Total score 0 – 42 with a higher score denoting a higher level of function. Entry item is not included in this total score.

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
DMD*	150 million cells IV every 3 months*	150 million cells/3 months *

VI. Product Availability [Pending]

Pending

VII. References

1. ClinicalTrials.gov. A study of CAP-1002 in ambulatory and non-ambulatory patients with Duchenne muscular dystrophy (HOPE-2). Available at: https://www.clinicaltrials.gov/study/NCT03406780. Accessed January 30, 2026.

2. ClinicalTrials.gov. Open-label extension of the HOPE-2 trial (HOPE-2-OLE). Available at: https://www.clinicaltrials.gov/study/NCT04428476. Accessed January 30, 2026.
3. McDonald CM, Marban E, Hendrix S, et al. Repeated intravenous cardiosphere-derived cell therapy in late-stage Duchenne muscular dystrophy (HOPE-2): A multicenter, randomized, double-blind, placebo-controlled, phase 2 trial. Lancet 2022;39:1049-58.
4. Birnkrant DJ, Bushby K, Bann CM, et al. Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management. Lancet Neurol. 2018; 17: 251-267.
5. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. Report of the guideline development subcommittee of the American Academy of Neurology. Neurology. 2016; 86:465-472. Reaffirmed January 22, 2022.

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	03.11.25	05.25
2Q 2026 annual review: no significant changes as drug is not yet FDA-approved; references reviewed and updated. Added ICHRA line of business.	03.30.26	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.