Preemptive policy: This is a P&T approved policy and can be used after the drug is FDA approved until it is superseded by an updated policy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

Reference Number: CP.PHAR.710
Effective Date: FDA Approval Date
Last Review Date: 02.26
Line of Business: Commercial, HIM, Medicaid

Revision Log

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

Description

Ataluren (Translarna™) is a protein restoration therapy.

FDA Approved Indication(s) [Pending]

Translarna is indicated for the treatment of nonsense mutation Duchenne muscular dystrophy (nmDMD).

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 Translarna may be **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 nmDMD (*see Appendix D*) confirmed by genetic testing;*
2. Prescribed by or in consultation with a neurologist;
3. Age ≥ 5 years;*
4. Member has all of the following assessed within the last 30 days (a, b, and c):
   a. Ambulatory function (e.g., ability to walk with or without assistive devices, not wheelchair dependent) with a 6-minute walk test (6MWT) distance ≥ 150 m;
   b. Stable cardiac function with left ventricular ejection fraction (LVEF) > 40%;
   c. Stable pulmonary function with predicted forced vital capacity (FVC) ≥ 50%;
5. Inadequate response (as evidenced by a significant decline in 6MWT, LVEF, or FVC) despite adherent use of an oral corticosteroid (e.g., prednisone, deflazacort [Emflaza®], Agamree®)* for ≥ 12 months, unless contraindicated or clinically significant adverse effects are experienced;^
   **Prior authorization is required for Emflaza and Agamree*
   ^For Illinois HIM requests, the step therapy requirement above does not apply as of 1/1/2026 per IL HB 5395
6. Translarna is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;

7. Translarna is not prescribed concurrently with exon-skipping therapies (e.g., Amondys 45®, Exondys 51®, Vyondys 53®, Viltepso®);
8. Member has not previously received gene replacement therapy for DMD (e.g., Elevidys®);
9. Documentation of member's current body weight in kg;
10. Dose does not exceed 40 mg/kg per day.*

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

1. Currently receiving medication for nmDMD or member has previously met initial approval criteria;
2. Member is responding positively to therapy as evidenced by one of the following (a or b):
   a. All of the following assessed within the last 6 months (i, ii, and iii):
      i. Ambulatory function (e.g., ability to walk with or without assistive devices, not wheelchair dependent) with a 6MWT distance ≥ 150 m;
      ii. Stable cardiac function with LVEF > 40%;
      iii. Stable pulmonary function with predicted FVC ≥ 50%;
   b. Member has received this medication via a healthcare insurer without meeting the requirements above (see criterion 2a), and medical record shows improved or stable LVEF and FVC, assessed within the last 6 months;
3. Member has been assessed by a neurologist within the last 6 months;
4. Translarna is prescribed concurrently with an oral corticosteroid, unless contraindicated or clinically significant adverse effects are experienced;
5. Translarna is not prescribed concurrently with exon-skipping therapies (e.g., Amondys 45, Exondys 51, Vyondys 53, Viltepso);

6. Member has not previously received gene replacement therapy for DMD (e.g., Elevidys);
7. Documentation of member's current body weight in kg;
8. If request is for a dose increase, new dose does not exceed 40 mg/kg per day.*

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) 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

6MWT: 6-minute walk test
DMD: Duchenne muscular dystrophy
FDA: Food and Drug Administration
FVC: forced vital capacity
LVEF: left ventricular ejection fraction
mRNA: messenger ribonucleic acid
nmDMD: nonsense mutation DMD

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
prednisone*	0.3-0.75 mg/kg/day or 10 mg/kg/weekend PO	Based on weight
deflazacort (Emflaza®)	0.9 mg/kg/day PO QD	Based on weight

Drug Name	Dosing Regimen	Dose Limit/ Maximum Dose
Agamree® (vamorolone)	6 mg/kg/day PO QD (up to a maximum of 300 mg/day) • If member has mild (Child-Pugh A) to moderate (Child-Pugh B) hepatic impairment: 2 mg/kg/day PO QD (up to a maximum of 100 mg/day) • If co-administered with strong CYP3A4 inhibitors (e.g., itraconazole): 4 mg/kg/day PO QD (up to a maximum of 200 mg/day)	See regimen

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.
**Off-label*

Appendix C: Contraindications/Boxed Warnings [Pending]
- Contraindication(s): pending
- Boxed warning(s): pending

Appendix D: General Information
- nmDMD affects approximately 11-13% of DMD patients. A nonsense mutation is a type of point mutation where a single letter is changed that stops the gene from being read. It results in a premature stop codon within messenger ribonucleic acid (mRNA). This premature stop codon in the mRNA causes disease by terminating translation before a full-length protein is generated. Gene sequencing can confirm the presence of a nonsense mutation in the dystrophin gene.
- 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 while Translarna (approved in Europe in August 2014, with authorization for use later withdrawn in March 2025 due to lack of confirmed effectiveness) is mentioned only as an emerging treatment.
- 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.
- The inclusion criteria for Study 041 (NCT03179631), the pivotal study used to support the FDA approval of Translarna, enrolled patients at least 5 years of age with a 6MWT distance ≥ 150 m at baseline.
- Having an LVEF below 40% may indicate presence of cardiomyopathy or heart failure, while a predicted FVC below 50% may indicate presence of severe pulmonary disease.

V. Dosage and Administration [Pending]

Indication	Dosing Regimen	Maximum Dose
nmDMD*	PO TID: 10 mg/kg in the morning, 10 mg/kg in the afternoon, and 20 mg/kg at night*	40 mg/kg/day*

VI. Product Availability [Pending]

Granules for oral suspension: 125 mg, 250 mg, 1,000 mg*

VII. References

1. European Medicines Agency: Translarna: EPAR – Product Information; April 2025. Available at: https://www.ema.europa.eu/en/medicines/human/EPAR/translarna. Accessed November 3, 2025.
2. 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.
3. Gloss D, Moxley RT, Ashwal S, Oskoui M. Practice guideline update summary: corticosteroid treatment of Duchenne muscular dystrophy. Neurology. 2016; 86: 465-472. Reaffirmed on January 22, 2022.
4. ClinicalTrials.gov. Long-term outcomes of ataluren in Duchenne muscular dystrophy. Available at: https://www.clinicaltrials.gov/study/NCT03179631. Accessed November 3, 2025.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created pre-emptively.	01.14.25	02.25
1Q 2026 annual review: no significant changes as drug is not FDA-approved; added step therapy bypass for IL HIM per IL HB 5395; references reviewed and updated.	11.03.25	02.26

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.