

Clinical Policy: Tafamidis (Vyndaqel, Vyndamax)

Reference Number: CP.PHAR.432

Effective Date: 09.01.19

Last Review Date: 05.20

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

Tafamidis meglumine (Vyndaqel[®]) and tafamidis (Vyndamax[™]) are transthyretin stabilizers.

FDA Approved Indication(s)

Vyndaqel and Vyndamax are indicated for the treatment of the cardiomyopathy of wild type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) in adults to reduce cardiovascular mortality and cardiovascular-related hospitalization.

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.

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

I. Initial Approval Criteria

A. Transthyretin Amyloid Cardiomyopathy (must meet all):

1. Diagnosis of ATTR-CM;
2. Prescribed by or in consultation with a cardiologist;
3. Age \geq 18 years;
4. Diagnosis is supported by one of the following (a or b):
 - a. Tissue biopsy amyloid protein is identified as transthyretin via mass spectrometry or immunohistochemistry, and (i or ii):
 - i. Tissue biopsy is of endomyocardial origin;
 - ii. Tissue biopsy is of extra-cardiac origin and echocardiography (Echo), cardiac magnetic resonance imaging (CMR), or positron emission tomography (PET) findings are consistent with cardiac amyloidosis;
 - b. Member meets all of the following (i, ii, and iii):
 - i. Echo, CMR, or PET findings are consistent with cardiac amyloidosis;
 - ii. Cardiac uptake is Grade 2 or 3 on a radionuclide scan utilizing one of the following radiotracers (a, b, or c):
 - a) ^{99m}Tc-labeled 3,3-diphosphono-1,2-propanodicarboxylic acid (DPD);
 - b) ^{99m}Tc-labeled pyrophosphate (PYP);
 - c) ^{99m}Tc-labeled hydroxymethylene diphosphonate (HMDP);
 - iii. Each of the following laboratory tests is negative for monoclonal protein (a, b, and c):

- a) Serum kappa/lambda free light chain ratio analysis;
 - b) Serum protein immunofixation;
 - c) Urine protein immunofixation;
5. Member has not had a liver transplant;
6. Dose does not exceed either of the following (a or b):
- a. Vyndaqel: 80 mg (4 capsules) per day;
 - b. Vyndamax: 61 mg (1 capsule) per day.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid.

II. Continued Therapy

A. Transthyretin Amyloid Cardiomyopathy (must meet all):

1. Currently receiving medication via Centene benefit or member has previously met initial approval criteria;
2. Member is responding positively to therapy, including but not limited to improvement or stabilization in any of the following parameters:
 - a. Walking ability;
 - b. Nutrition (e.g., body mass index);
 - c. Cardiac related hospitalization;
 - d. Cardiac procedures or laboratory tests (e.g., Holter monitoring, echocardiography, electrocardiogram, plasma BNP or NT-proBNP, serum troponin);
3. Dose does not exceed either of the following (a or b):
 - a. Vyndaqel: 80 mg (4 capsules) per day;
 - b. Vyndamax: 61 mg (1 capsule) per day.

Approval duration: 12 months

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

1. Currently receiving medication via Centene benefit and documentation supports positive response to therapy.
Approval duration: Duration of request or 6 months (whichever is less); or
2. Refer to the off-label use policy for the relevant line of business if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): CP.CPA.09 for commercial, HIM.PHAR.21 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.PHAR.21 for health insurance marketplace, and CP.PMN.53 for Medicaid or evidence of coverage documents.**

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key

ATTR-CM: cardiomyopathy of transthyretin-mediated amyloidosis

FDA: Food and Drug Administration

Appendix B: Therapeutic Alternatives

Not applicable

Appendix C: Contraindications/Boxed Warnings

None reported

V. Dosage and Administration

Drug Name	Dosing Regimen	Maximum Dose
Tafamidis (Vyndaqel)	20 mg (4 capsules) PO QD	80 mg/day
Tafamidis (Vyndamax)	61 mg (1 capsule) PO QD	61 mg/day

VI. Product Availability

Drug Name	Availability
Tafamidis (Vyndaqel)	Capsules: 20 mg
Tafamidis (Vyndamax)	Capsules: 61 mg

VII. References

1. Vyndaqel, Vyndamax Prescribing Information. New York, NY; Pfizer, Inc.; August 2019. Available at: https://www.accessdata.fda.gov/drugsatfda_docs/label/2019/211996s000,212161s0001bl.pdf. Accessed February 14, 2020.
2. Maurer MS, Schwartz JH, Gundapaneni B, et al. Tafamidis treatment for patients with transthyretin amyloid cardiomyopathy. *N Engl J Med*. 2018; 379(11): 1007-1016.
3. Ando Y, Coelho T, Berk JL, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. *Orphanet Journal of Rare Diseases*. 2013; 8:31.
4. Gillmore JD, Maurer MS, Falk RH, et al. Nonbiopsy diagnosis of cardiac transthyretin amyloidosis. *Circulation*. 2016;133(24):2404. Epub 2016 Apr 22.
5. Dorbala S, Ando Y, Bokhari S, et al. ASNC/AHA/ASE/EANM/HFSA/ISA/SCMR/SNMMI expert consensus recommendations for multimodality imaging in cardiac amyloidosis: Part 1 of 2 - Evidence base and standardized methods of imaging. *J Cardiac Failure*; 2019: 24(11): e2-e39.
6. Dorbala S, Ando Y, Bokhari S, et al. ASNC/AHA/ASE/EANM/HFSA/ISA/SCMR/SNMMI expert consensus recommendations for multimodality imaging in cardiac amyloidosis: Part 2 of 2-Diagnostic criteria and appropriate utilization. *Journal of Cardiac Failure*; 2019: 25(11): 854-865.
7. Witteles RM, Bokhari S, Damy T, et al. Screening for transthyretin amyloid cardiomyopathy in everyday practice. *JACC*, August 2019; 7(8): 709-16.

Reviews, Revisions, and Approvals	Date	P&T Approval Date
Policy created	06.18.19	08.19
Finalized HIM line of business on policy per SDC and prior clinical guidance.	01.16.20	
Cardiac scintigraphy added as a tissue biopsy alternative for ATTR-CM; references reviewed and updated.	02.11.20	05.20

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.

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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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