

VYND AQEL (tafamidis meglumine) VYNDAMAX (tafamidis)

POLICY

I. INDICATIONS

The indications below including FDA-approved indications and compendial uses are considered a covered benefit provided that all the approval criteria are met and the member has no exclusions to the prescribed therapy.

FDA-Approved Indication

Vyndaqel and Vyndamax are transthyretin stabilizers 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.

All other indications are considered experimental/investigational and not medically necessary.

II. DOCUMENTATION

Submission of the following information is necessary to initiate the prior authorization review:

- A. Echocardiography or cardiac magnetic resonance imaging results confirming cardiac involvement
- B. For members with hereditary ATTR-CM: results confirming a mutation of the transthyretin (TTR) gene
- C. For biopsy proven disease:
 - 1. Tissue biopsy confirming the presence of the transthyretin amyloid deposition
 - 2. Immunohistochemical analysis, mass spectrometry, tissue staining, or polarized light microscopy results confirming transthyretin precursor proteins
- D. For technetium-labeled bone scintigraphy proven disease:
 - 1. A serum kappa/lambda free light chain ratio, serum protein immunofixation or urine protein immunofixation test result showing the absence of monoclonal proteins
 - 2. Scintigraphy tracing results confirming presence of amyloid deposits
- E. For continuation of therapy: Medical record documentation confirming the member demonstrates a beneficial response to treatment (e.g., improvement in rate of disease progression as demonstrated by distance walked on the 6-minute walk test, the Kansas City Cardiomyopathy Questionnaire–Overall Summary (KCCQ-OS) score, cardiovascular-related hospitalizations, NYHA classification of heart failure, left ventricular stroke volume, NT-proBNP level)

III. PRESCRIBER SPECIALTIES

This medication must be prescribed by, or in consultation with, a cardiologist.

IV. CRITERIA FOR INITIAL APPROVAL

Cardiomyopathy of Wild Type or Hereditary Transthyretin-mediated Amyloidosis

Authorization of 6 months may be granted for treatment of cardiomyopathy of wild type or hereditary transthyretin-mediated amyloidosis (ATTR-CM) when all of the following criteria are met:

- A. The member exhibits clinical symptoms of cardiomyopathy and heart failure (e.g., dyspnea, fatigue, orthostatic hypotension, syncope, peripheral edema).
- B. Cardiac involvement was confirmed by echocardiography or cardiac magnetic resonance imaging (e.g., end-diastolic interventricular septal wall thickness exceeding 12 mm).
- C. The diagnosis is confirmed by one of the following:
 1. The member meets both of the following:
 - i. Presence of transthyretin amyloid deposits on analysis of biopsy from cardiac or noncardiac sites.
 - ii. Presence of transthyretin precursor proteins was confirmed by immunohistochemical analysis, mass spectrometry, tissue staining, or polarized light microscopy.
 2. The member meets both of the following:
 - i. Positive technetium-labeled bone scintigraphy tracing.
 - ii. Systemic light chain amyloidosis is ruled out by a test showing absence of monoclonal proteins (serum kappa/lambda free light chain ratio, serum protein immunofixation, or urine protein immunofixation).
- D. For members with hereditary ATTR-CM, presence of a mutation of the TTR gene was confirmed.
- E. The member is not a liver transplant recipient.
- F. The requested medication will not be used in combination with inotersen (Tegsedi), patisiran (Onpattro), or vutrisiran (Amvuttra).

V. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for the continued treatment of ATTR-CM when all of the following criteria are met:

- A. The member must meet all initial authorization criteria.
- B. The member must have demonstrated a beneficial response to treatment with tafamidis therapy [e.g., improvement in rate of disease progression as demonstrated by distance walked on the 6-minute walk test, the Kansas City Cardiomyopathy Questionnaire—Overall Summary (KCCQ-OS) score, cardiovascular-related hospitalizations, NYHA classification of heart failure, left ventricular stroke volume, N-terminal B-type natriuretic peptide (NT-proBNP) level]. Documentation from the medical record must be provided.

Effective date: 4/1/2020

Reviewed: 1/2020, 01/2021, 01/2022, 3/2023 Scope: Medicaid
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V. QUANTITY LIMIT

Vyndaqel has a quantity limit of 4 capsules per day.

Vyndamax has a quantity limit of 1 capsule per day.

VI. REFERENCES

1. Vyndaqel and Vydamax [package insert]. New York, NY: Pfizer Labs.; May 2022.
2. Maurer MS, Schwartz JH, Gundapaneni B, et al. Tafamidis treatment for patients with transthyretin amyloid cardiomyopathy. *N Engl J Med*. 2018 Sep 13; 379(11):1007-1016.
3. Maurer MS, Sabahat B, Thibaud D, et al. Expert Consensus Recommendations for the Suspicion and Diagnosis of Transthyretin Cardiac Amyloidosis. *Circulation: Heart Failure*. 2019 Sep 4;12:9.
4. Ruberg FL, Grogan M, et al. Transthyretin Amyloid Cardiomyopathy. *J Am Coll Cardiol*. 2019;73:2872-91.