Reference number(s)
3076-A

SPECIALTY GUIDELINE MANAGEMENT

VYNDAQEL (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. For initial requests:
   1. Chart notes or medical record documentation confirming the member demonstrates clinical symptoms of cardiomyopathy and heart failure
   2. For members with hereditary ATTR-CM: results confirming a mutation of the transthyretin (TTR) gene
   3. For biopsy proven disease:
      i. Tissue biopsy confirming the presence of the transthyretin amyloid deposition
      ii. Immunohistochemical analysis, mass spectrometry, tissue staining, or polarized light microscopy results confirming transthyretin precursor proteins
   4. For technetium-labeled bone scintigraphy proven disease:
      i. A serum kappa/lambda free light chain ratio, serum protein immunofixation or urine protein immunofixation test result showing the absence of monoclonal proteins
      ii. Scintigraphy tracing results confirming presence of amyloid deposits

B. For continuation of therapy: Chart notes or 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. CRITERIA FOR INITIAL APPROVAL

Cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis
Authorization of 12 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. The diagnosis is confirmed by one of the following:
    1. The member meets either 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).
C. For members with hereditary ATTR-CM, presence of a mutation of the TTR gene was confirmed.
D. The member is not a liver transplant recipient.
E. The requested medication will not be used in combination with inotersen (Tegsedi), patisiran (Onpattro), vutrisiran (Amvuttra), or eplontersen (Wainua).

IV. CONTINUATION OF THERAPY

Authorization of 12 months may be granted for the continued treatment of ATTR-CM when both 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.

V. REFERENCES

1. Vyndaqel and Vyndamax [package insert]. New York, NY: Pfizer Labs; April 2023.
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. *Circ Heart Fail*. 2019 Sep 4;12:9.
4. Ruberg FL, Grogan M, et al. Transthyretin amyloid cardiomyopathy. *J Am Coll Cardiol*. 2019;73:2872-91.
5. Yadav JD, Othee H, Chan KA, Man DC, Belliveau PP, Towle J. Transthyretin Amyloid Cardiomyopathy-Current and Future Therapies. *Ann Pharmacother*. 2021;55(12):1502-1514.