

# Specialty Guideline Management

## Vyndaqel-Vyndamax

### Products Referenced by this Document

Drugs that are listed in the following table include both brand and generic and all dosage forms and strengths unless otherwise stated. Over-the-counter (OTC) products are not included unless otherwise stated.

| Brand Name | Generic Name        |
|------------|---------------------|
| Vyndaqel   | tafamidis meglumine |
| Vyndamax   | tafamidis           |

### 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<sup>1</sup>

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.

### Documentation

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

## Initial requests

- Chart notes or medical record documentation of prior hospitalization of heart failure or confirming the member demonstrates clinical symptoms of heart failure at baseline.
- For biopsy proven disease:
  - Tissue biopsy from cardiac or noncardiac sites confirming the presence of the transthyretin amyloid deposition.
  - Immunohistochemical analysis, mass spectrometry, tissue staining, or polarized light microscopy results confirming the presence transthyretin precursor proteins.
- For technetium-labeled bone scintigraphy proven disease:
  - Scintigraphy tracing results confirming the presence of amyloid deposits.
  - Serum kappa/lambda free light chain ratio, serum protein immunofixation, and urine protein immunofixation test results showing the absence of monoclonal proteins.
- For hereditary ATTR-CM: testing or analysis confirming a pathogenic or likely pathogenic variant in the transthyretin (TTR) gene.

## Continuation requests

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, New York Heart Association [NYHA] classification of heart failure, left ventricular stroke volume, N-terminal B-type natriuretic peptide [NT-proBNP] level).

## Prescriber Specialties

This medication must be prescribed by or in consultation with a geneticist, cardiologist, or a physician specializing in the treatment of amyloidosis.

## Coverage Criteria

### Cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis (ATTR-CM)<sup>1-6</sup>

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:

- Member is 18 years of age or older.

- Member has medical history of heart failure with at least one prior hospitalization for heart failure (not due to arrhythmia or a conduction system disturbance treated with a permanent pacemaker), OR exhibits clinical symptoms of heart failure (e.g., volume overload, dyspnea, fatigue, orthostatic hypotension, syncope, peripheral edema) at baseline.
- Diagnosis is confirmed by either of the following criteria:
  - Member meets both of the following criteria for biopsy proven disease:
    - Presence of transthyretin amyloid deposits on analysis of biopsy from cardiac or noncardiac sites.
    - Presence of transthyretin precursor proteins was confirmed by immunohistochemical analysis, mass spectrometry, tissue staining, or polarized light microscopy.
  - Member meets both of the following criteria for technetium-labeled bone scintigraphy proven disease:
    - Presence of amyloid deposits confirmed by technetium-labeled bone scintigraphy tracing.
    - Systemic light chain amyloidosis is ruled out by showing the absence of monoclonal proteins by all of the following tests: a) serum kappa/lambda free light chain ratio, b) serum protein immunofixation, and c) urine protein immunofixation.
- For members with hereditary ATTR-CM, the diagnosis is confirmed by detection of a pathogenic or likely pathogenic variant in the TTR gene.
- Member does not have either of the following:
  - A history of liver or heart transplant
  - Implantation of left-ventricular assist device
- The requested medication will not be used in combination with inotersen (Tegsedi), patisiran (Onpattro), vutrisiran (Amvuttra), eplontersen (Wainua), or acoramidis (Attruby).

## Continuation of Therapy

Authorization of 12 months may be granted for continued treatment of ATTR-CM when both of the following criteria are met:

- Member must meet all requirements in the coverage criteria.
- 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).

## References

1. Vyndaqel and Vyndamax [package insert]. New York, NY: Pfizer Labs; October 2023.

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. Maurer MS, Sabahat B, Thibaud D, et al. Expert consensus recommendations for the suspicion and diagnosis of transthyretin cardiac amyloidosis. *Circ Heart Fail*. 2019;12(9):e006075.
4. Kittleson MM, Maurer MS, Ambardekar AV, et al. Cardiac amyloidosis: Evolving diagnosis and management: A scientific statement from the American Heart Association. *Circulation*. 2020;142(1):e7-e22.
5. Yadav JD, Othee H, Chan KA, et al. Transthyretin amyloid cardiomyopathy-Current and future therapies. *Ann Pharmacother*. 2021;55(12):1502-1514.
6. Kittleson MM, Ruberg FL, Ambardekar AV, et al. 2023 ACC expert consensus decision pathway on comprehensive multidisciplinary care for the patient with cardiac amyloidosis: A report of the American College of Cardiology solution set oversight committee. 2023;88(11):1076-1126.