RXADVANCE
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Clinical Policy Title:	tafamidis
Policy Number:	RxA.618
Drug(s) Applied:	Vyndaqel®, Vyndamax™
Original Policy Date:	03/06/2020
Last Review Date:	12/07/2020
Line of Business Policy Applies to:	All lines of business

Background

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

Dosing Information

Drug Name	Indication	Dosing Regimen	Maximum Dose
tafamidis meglumine (Vyndaqel®)	Cardiomyopathy of transthyretin-mediated amyloidosis	20 mg orally once daily	80 mg/day
tafamidis (Vyndamax™)		61 mg orally once daily	61 mg/day

Dosage Forms

- tafamidis meglumine (Vyndaqel®): capsules 20 mg
- tafamidis (Vyndamax™): capsules 61 mg

Clinical Policy

Provider must submit documentation (such as office chart notes, lab results or other clinical information) supporting that member has met all approval criteria.

I. Initial Approval Criteria

A. Transthyretin Amyloid Cardiomyopathy (must meet all):

1. Diagnosis of cardiomyopathy of wild-type or hereditary transthyretin-mediated amyloidosis;
2. Prescribed by or in consultation with a cardiologist or neurologist;
3. Age 18 years of age or older;
4. Cardiac involvement was confirmed by echocardiography or cardiac magnetic resonance imaging (e.g., end-diastolic interventricular septal wall thickness exceeding 12 mm);
5. One of the following (a or b):
   a. Biopsy is positive for amyloid deposits; or
   b. Technetium-labeled bone scintigraphy tracing results confirm presence of amyloid deposits;
6. One of the following (a or b):

a. For members with wild type ATTR-CM, presence of TTR precursor protein confirmed by immunohistochemistry, scintigraphy, or mass spectrometry); or
b. For members with hereditary ATTR-CM, presence of a TTR mutation confirmed by genetic testing;

7. Negative serum or urine test for amyloid light chains;
8. Member has not had a liver transplant;
9. Dose does not exceed either of the following (a or b):
   a. Vyndaqel®: 80 mg per day
   b. Vyndamax™: 61 mg per day

Approval Duration
Commercial: 6 months
Medicaid: 6 months

II. Continued Therapy Approval

A. Transthyretin Amyloid Cardiomyopathy (must meet all):

1. Member is currently receiving the medication that has been authorized by RxAdvance or the member has met initial approval criteria listed in this policy;
2. Member is responding positively to therapy, including but not limited to improvement or stabilization in any of the following parameters:
   a. Walking ability (e.g., 6-minute walk test);
   b. Cardiac related hospitalization;
   c. NYHA classification of heart failure;
   d. Left ventricular stroke volume;
   e. Cardiac procedures or laboratory tests (e.g., plasma BNP, NT-proBNP, serum troponin, Holter monitoring, echocardiography, or electrocardiogram);
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
Commercial: 12 months
Medicaid: 12 months

III. Appendices

APPENDIX A: Abbreviation/Acronym Key
ATTR-CM: Cardiomyopathy of Transthyretin-Mediated Amyloidosis
BNP: B-type Natriuretic Peptide
FDA: Food and Drug Administration
NT-proBNP: N-terminal B-type Natriuretic Peptide
NYHA: New York Heart Association

APPENDIX B: Therapeutic Alternatives
Not applicable

APPENDIX C: Contraindications/Boxed Warnings

- Contraindication(s):
  - None

- Boxed Warning(s):
  - None

APPENDIX D: General Information

- Transthyretin amyloid cardiomyopathy is a rare, life-threatening disease characterized by the build-up of abnormal deposits of misfolded protein called amyloid in the heart and is defined by restrictive cardiomyopathy and progressive heart failure.
- Previously, there were no medicines approved to treat ATTR-CM; the only available options included symptom management, and, in rare cases, heart (or heart and liver) transplant. It is estimated that the prevalence of ATTR-CM is approximately 100,000 people in the U.S. and only one to two percent of those patients are diagnosed today.

References

1. Vyndaqel, Vyndamax Prescribing Information. New York, NY; Pfizer, Inc., April 2020. Available at: http://labeling.pfizer.com/ShowLabeling.aspx?id=11685. Accessed October 15, 2020.
2. Maurer MS, et al. Tafamidis treatment for patients with transthyretin amyloid cardiomyopathy. N Engl J Med. 2018; 379(11): 1007-1016. Accessed October 15, 2020.
3. Ando Y, Coelho T, Berk JL, Cruz MW, Ericzon BG, Ikeda S, et al. Guideline of transthyretin-related hereditary amyloidosis for clinicians. Orphanet J Rare Dis. 2013; 8: 31. Accessed October 15, 2020.

Review/Revision History	Review/Revision Date	P&T Approval Date
Policy established.	01/01/2020	03/06/2020
Policy was reviewed 1. Policy formatting updated. 2. Policy applies to all lines of business. 3. Criteria for approval updated. 4. Continued therapy criteria II.A.1 was rephrased to “Currently receiving medication that has been authorized by RxAdvance…”. 5. Appendix D added and updated with general information. 6. References were reviewed and updated.	12/03/2020	12/07/2020