

Elamipretide

Goal(s):

- Promote evidence-based standard of care in patients diagnosed with Barth Syndrome and with symptoms of cardiomyopathy and heart-related conditions.
- Limit to populations in which elamipretide has been studied and approved by the Food and Drug Administration.

Length of Authorization:

- Up to 12 months

Requires PA:

- Elamipretide

Covered Populations: FFS and CCO populations beginning 1/1/26 (pharmacy or provider administered claims)

Covered Alternatives:

- Current PMPDP preferred drug list per OAR 410-121-0030 at www.orpdl.org
- Searchable site for Oregon FFS Drug Class listed at www.orpdl.org/drugs/

Table 1. Recommended adjunct treatments for heart-related conditions

Population	Adjunct treatment
Ejection fraction ≤ 40%	ACE inhibitor, ARB or angiotensin receptor/neprolysin inhibitor (ARNI) AND Beta blocker
Arrhythmias (atrial fibrillation or flutter) or non-compaction cardiomyopathy	Aspirin or anticoagulation
Current edema related to heart failure	Diuretics to achieve a euvolemic state (chronic or as needed)

Table 2. Target doses for Common HFrEF therapies

ACE inhibitor/ARB/ARNI	Target Dose	Beta Blockers	Target Dose
Candesartan	32 mg once daily	Bisoprolol	10 mg once daily
Captopril	50 mg three times daily	Carvedilol	25 mg twice daily
Enalapril	10 mg twice daily	Carvedilol CR	80 mg once daily
Fosinopril	40 mg once daily	Metoprolol	200 mg once daily
Lisinopril	20 mg once daily		
Losartan	50 mg once daily		
Quinapril	20 mg twice daily		
Ramipril	10 mg once daily		
Sacubitril-valsartan	97-103 mg twice daily		
Valsartan	160 mg twice daily		

Approval Criteria

1. What diagnosis is being treated?

Record ICD10 code.

Approval Criteria

2. Is the request for a patient with a prior FFS approval for the requested drug?	Yes: Go to Renewal Criteria	No: Go to #3
3. Is there documentation of genetically-confirmed Barth Syndrome (e.g., pathogenic mutation in the TAZ gene)?	Yes: Go to #4	No: Pass to RPh. Deny; medical appropriateness
4. Is the request for a patient who weighs at least 30 kg?	Yes: Go to #5	No: Pass to RPh. Deny; medical appropriateness
5. Is the drug prescribed by a pediatric cardiologist or other specialist with experience in mitochondrial disorders?	Yes: Go to #6	No: Pass to RPh. Deny; medical appropriateness
6. Are there documented baseline assessments for all the following within the past year: <ul style="list-style-type: none"> • Cardiac function (e.g., ejection fraction) • Ambulatory motor function (e.g., 6MWT) • Muscle strength (e.g., physical therapy assessment)? 	Yes: Go to #7	No: Pass to RPh. Deny; medical appropriateness
7. Is there documentation or provider attestation that the patient has impaired ambulatory function but is still able to complete a 6-minute walk test?	Yes: Go to #8	No: Pass to RPh. Deny; medical appropriateness
8. Is there documentation or provider attestation that medication therapy for heart failure has been optimized when appropriate (Table 1 and 2)?	Yes: Go to #9	No: Pass to RPh. Deny; medical appropriateness
9. Is there documentation or provider attestation that a follow-up visit is scheduled with 4 weeks of initiation to assess tolerability and adherence?	Yes: Go to #10	No: Pass to RPh. Deny; medical appropriateness
10. Has the provider documented patient-specific goals for this therapy over the next 6 months? Note: Goals of therapy can vary from improvement in fatigue, motor function, quality of life disease, burden reduction, or disease stabilization.	Yes: Go to #11	No: Pass to RPh. Deny; medical appropriateness

Approval Criteria

11. Has the provider defined objective criteria to evaluate unsuccessful treatment or lack of response based on individual patient goals and current symptoms (i.e., when would the provider consider discontinuing therapy)?

To qualify for treatment coverage, the patient and provider must have a documented discussion about when risks of the therapy outweigh the benefits and a knowledge of the realistic expectations of treatment efficacy. Care must always take place in the context of the patient's support systems, overall health, and core values.

Yes: Pass to RPh;
Pend. Refer to DMAP
for secondary review.

Initial approval duration:
12 weeks

No: Pass to RPh.
Deny; medical
appropriateness

Renewal Criteria

1. Is there documented evidence of adherence and tolerance to therapy based on claims history and provider assessment?

Yes: Go to #2

No: Pass to RPh.
Deny; medical
appropriateness

2. Has the provider re-evaluated the following baseline assessments within the past 2 months?

- Motor function (e.g., 6MWT)
- Muscle strength (e.g., physical therapy assessment)

Yes: Go to #3

No: Pass to RPh.
Deny; medical
appropriateness

3. Is there documentation that muscle strength has improved from baseline?

Note: FDA-approval was based on improvements in knee extensor muscle strength after treatment over 12 weeks which continued to improve with over 3 years of follow-up.

Yes: Go to #4

No: Pass to RPh.
Deny; medical
appropriateness

4. Is there documentation that the patient's goals of therapy established prior to treatment have been met?

Yes: Pass to RPh;
Pend. Refer to DMAP
for secondary review.

Approval duration: 12
months

No: Go to #5

Renewal Criteria

5. Is there documentation that pre-established criteria for unsuccessful treatment or lack of response have been met?	Yes: Pass to RPh. Deny; medical appropriateness	No: Go to #6
6. Have the patient and provider had a documented discussion about when benefits of the therapy outweigh the potential risks?	Yes: Pass to RPh; Pend. Refer to DMAP for secondary review. Approval duration: 12 months	No: Pass to RPh. Deny; medical appropriateness

*P&T/DUR Review: 2/26 (SS)
Implementation: 3/1/26*