Effective Date: 5/2026
Reviewed: 2/2026
Scope: Medicaid

FORZINITY (elamipretide)

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
Forzinity is indicated to improve muscle strength in adults and pediatric members with Barth Syndrome weighing at least 30 kg.

This indication is approved under accelerated approval based on an improvement in knee extensor muscle strength, an intermediate clinical endpoint. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

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

II. CRITERIA FOR INITIAL APPROVAL

Barth Syndrome
Authorization of 6 months may be granted for management of Barth Syndrome when all the following criteria are met:

A. Member is 12 years of age or older.
B. Member weighs 30 kg or greater.
C. Documentation of current estimated glomerular filtration rate (eGFR) and prescribed dose
[Note: For adult members with eGFR < 30 mL/min/1.73 m² and not on dialysis, the FDA-approved dose is 20mg subcutaneously once daily]
D. Member meets either of the following criteria:
    a. Adult member has either an estimated glomerular filtration rate (eGFR) ≥ 30 mL/min/1.73 m² or eGFR < 30 mL/min/1.73 m² and is not on dialysis
    b. Pediatric member and is not renally impaired
E. Documentation of confirmed diagnosis of Barth Syndrome by either of the following:
    a. Genetic testing documenting a pathogenic variant in the TAFAZZIN gene.
    b. Increased monolysocardiolipin:cardiolipin (MLCL/CL) ratio.
F. Documentation with chart notes and/or medical records that member has completed a 6-minute walk test (6MWT) prior to the start of therapy and has been found to be ambulatory and impaired per provider.
G. Member does not have uncontrolled hypertension despite appropriate treatment in the opinion of the provider (i.e., blood pressure consistently elevated above 160 mmHg systolic or 100 mmHg diastolic).
H. Member has not previously undergone or plans to undergo heart transplantation.
I. Member meets either of the following criteria:
    a. Member does not have an implantable cardioverter defibrillator (ICD) and is not planning to undergo implantation of ICD.

b. Member has an ICD but with no known occurrence of ICD discharge in the past three months.

J. Member is not currently receiving treatment with chemotherapeutic agents and has not received prior radiation therapy to the chest.

K. Member has not received stem cell or gene therapy and is not currently being treated by a therapeutic investigational device.

L. The requested medication is prescribed by or in consultation with a cardiologist or physician who specializes in the treatment of metabolic or neuromuscular disorders.

III. CONTINUATION OF THERAPY

Authorization of 6 months may be granted for management of Barth Syndrome requesting continuation of therapy when all the following criteria are met:

A. Member continues to meet all initial criteria.

B. Documentation with chart notes and/or medical records that the member has demonstrated a response to therapy [e.g., improvement in rate of disease progression as demonstrated by improvement in distance walked on the 6-minute walk test (6MWT), the Barth Syndrome Symptom Assessment (BTHS-SA) score, muscle strength as measured by handheld dynamometry (HHD), Five Times Sit-To-Stand (5XSST) time, SWAY Application Balance Assessment, Patient Global Impression Scales of Symptoms (PGI), and/or Clinician Global Impression (CGI)]

IV. QUANTITY LIMIT

Forzinity: Four 280 mg/3.5 mL vials per 28 days (daily dose of 0.5 mL)

V. REFERENCES

1. Forzinity [package insert]. Needham, MA: Stealth BioTherapeutics Inc.; September 2025.
2. FDA Grants Accelerated Approval to Forzinity. FDA News Release; Sept 19, 2025.
3. Marjoram L, Huang Y, Koenig MK, Cohen BH, Anderson E. Real-world disease burden and health care resource utilization for patients with Barth syndrome. J Med Econ. 2025 Dec;28(1):2010-2026. doi: 10.1080/13696998.2025.2588729. Epub 2025 Nov 21. PMID: 41268908.