Reference number(s)
7225-A

Specialty Guideline Management Forzinity

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
Forzinity	elamipretide

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 Indications[1]

Forzinity is indicated to improve muscle strength in adult and pediatric patients 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.

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 documenting both of the following:

- Monolysocardiolipin:cardiolipin (MLCL/CL) ratio assay or genetic testing results supporting diagnosis.
- Medical records (e.g. chart notes) of the baseline assessment for the 6-minute walk test (6MWT) to establish baseline results.

Continuation Requests

Chart notes and/or medical records documenting a response to therapy [e.g., improvement in rate of disease progression as demonstrated by 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), Clinician Global Impression (CGI)].

Prescriber Specialties

This medication must be prescribed by or in consultation with a cardiologist or a physician who specializes in the treatment of metabolic or neuromuscular disorders.

Coverage Criteria

Barth Syndrome[1-3]

Authorization of 12 months may be granted for treatment of Barth syndrome when all of the following criteria are met:

- Member is 12 years of age or older.
- Member weighs 30 kg or greater.
- Member meets either of the following criteria:
    - Member is an adult and meets either of the following criteria:
        - Member has an estimated glomerular filtration rate (eGFR) greater than or equal to 30 mL/min/1.73 m^2.
        - Member has an eGFR less than 30 mL/min/1.73 m^2 and is not on dialysis.
    - Member is a pediatric patient and is not renally impaired.
- The diagnosis of Barth syndrome was confirmed by either of the following:
    - Genetic testing documenting a pathogenic variant in the TAFAZZIN gene.
    - Increased monolysocardiolipin:cardiolipin (MLCL/CL) ratio.

- 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.
- Member does not have uncontrolled hypertension in the opinion of the provider (i.e., blood pressure consistently elevated above 160 mmHg systolic or 100 mmHg diastolic despite appropriate treatment).
- Member has not previously undergone and is not planned to undergo heart transplantation.
- Member meets either of the following criteria:
    - Member does not have an implantable cardioverter defibrillator (ICD) and is not planned to undergo an implantation of an ICD.
    - Member has an ICD but no known occurrence of ICD discharge in the past three months.
- Member is not currently receiving treatment with chemotherapeutic agents and has not received prior radiation therapy to the chest.
- Member has not received stem cell or gene therapy and is not currently being treated by a therapeutic investigational device.

Continuation of Therapy[3]

Authorization of 12 months may be granted for members requesting continuation of therapy when the member has demonstrated a response to therapy [e.g., improvement in rate of disease progression as demonstrated by 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), Clinician Global Impression (CGI)].

References

1. Forzinity [package insert]. Needham, MA: Stealth BioTherapeutics Inc.; September 2025.
2. Ferreira C, Pierre G, Thompson R, et al. Barth Syndrome. 2014 Oct 9 [Updated 2020 Jul 9]. In: Adam MP, Feldman J, Mirzaa GM, et al., editors. GeneReviews® [Internet]. Seattle (WA): University of Washington, Seattle; 1993-2025. Available from: https://www.ncbi.nlm.nih.gov/books/NBK247162/.
3. Reid Thompson W, Hornby B, Manuel R, et al. A phase 2/3 randomized clinical trial followed by an open-label extension to evaluate the effectiveness of elamipretide in Barth syndrome, a genetic disorder of mitochondrial cardiolipin metabolism. Genet Med. 2021;23(3):471-478. doi:10.1038/s41436-020-01006-8