Clinical Policy: Elamipretide (Forzinity)

Reference Number: PA.CP.PHAR680
Effective Date: 02/2026
Last Review Date: 01/2026

Description

Elamipretide (Forzinity™) is a mitochondrial-targeting agent peptide that binds to cardiolipin.

FDA Approved Indication(s)

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(s).

Policy/Criteria

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

It is the policy of PA Health & Wellness® that Forzinity is **medically necessary** when the following criteria are met:

I. Initial Approval Criteria

A. Barth Syndrome (must meet all)**:**

1. Diagnosis of Barth syndrome confirmed by DNA testing for the presence of a mutation in the tafazzin (TAZ) gene;
2. Prescribed by or in consultation with a clinical geneticist, metabolic disease specialist, endocrinologist, cardiologist, hematologist, or neurologist;
3. Weight ≥ 30 kg;
4. Documentation of impaired muscle strength (e.g., knee extensor muscle strength measured by handheld dynamometry);
5. Dose does not exceed both of the following (a and b):
   a. 40 mg per day;
   b. 1 vial per 7 days.

Approval duration: 6 months

B. Other diagnoses/indications

1. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

II. Continued Therapy

A. Barth Syndrome (must meet all)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies;

2. Member is responding positively to therapy as evidenced by improvement in muscle strength; (e.g. knee extensor muscle strength measured by handheld dynamometry);.
3. If request is for a dose increase, new dose does not exceed both of the following (a and b):
   a. 40 mg per day;
   b. 1 vial per 7 days.

Approval duration: 12 months

B. Other diagnoses/indications (must meet 1 or 2)**:**

1. Currently receiving medication via PA Health & Wellness benefit and documentation supports positive response to therapy or the Continuity of Care policy (PA.PHARM.01) applies.
   Approval duration: Duration of request or 12 months (whichever is less); or
2. Refer to the off-label use policy if diagnosis is NOT specifically listed under section III (Diagnoses/Indications for which coverage is NOT authorized): PA.CP.PMN.53

III. Diagnoses/Indications for which coverage is NOT authorized:

A. Non-FDA approved indications, which are not addressed in this policy, unless there is sufficient documentation of efficacy and safety according to the off label use policies – PA.CP.PMN.53

IV. Appendices/General Information

Appendix A: Abbreviation/Acronym Key
FDA: Food and Drug Administration
TAZ: tafazzin gene

Appendix B: Therapeutic Alternatives
Not applicable

Appendix C: Contraindications/Boxed Warnings

- Contraindication(s): serious hypersensitivity to any of the ingredients
- Boxed warning(s): none reported

V. Dosage and Administration

Indication	Dosing Regimen	Maximum Dose
Barth syndrome	40 mg SC QD	40 mg/day

VI. Product Availability

Vial: 280 mg/3.5 mL (80 mg/mL)

VII. References

1. Forzinity Prescribing Information. Needham, MA: Stealth BioTherapeutics Inc. September 2025. Available at https://www.accessdata.fda.gov/drugsatfda_docs/label/2025/215244s000lbl.pdf. Accessed October 6, 2025.

2. Thompson WR, Manuel R, Abbruscato A, et al. Long-term efficacy and safety of elamipretide in patients with Barth syndrome: 168-week open-label extension results of TAZPOWER. Genet Med. 2024 Jul;26(7):101138. doi: 10.1016/j.gim.2024.101138.
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 Mar;23(3):471-478. doi: 10.1038/s41436-020-01006-8.
4. Clarke SL, Bowron A, Gonzalez IL, et al. Barth syndrome. *Orphanet journal of rare diseases*. 2013 Feb 12;8(1):1. doi: 10.1186/1750-1172-8-23
5. Van Werkhoven MA, Thorburn DR, et al. Monolysocardiolipin in cultured fibroblasts is a sensitive and specific marker for Barth Syndrome. *J Lipid Res.* 2006 Oct;47(10):2346-51. doi: 10.1194/jlr.D600024-JLR200.
6. Kulik W, van Lenthe H, Stet FS, et al. Bloodspot assay using HPLC-tandem mass spectrometry for detection of Barth syndrome. *Clin Chem*. 2008 Feb;54(2):371-8. doi: 10.1373/clinchem.2007.095711.
7. Vaz FM, van Lenthe H, Vervaart MAT, et al. An improved functional assay in blood spot to diagnose Barth syndrome using the monolysocardiolipin/cardiolipin ratio. *J Inherit Metab Dis*. 2022 Jan;45(1):29-37. doi: 10.1002/jimd.12425.

Coding Implications

Codes referenced in this clinical policy are for informational purposes only. Inclusion or exclusion of any codes does not guarantee coverage. Providers should reference the most up-to-date sources of professional coding guidance prior to the submission of claims for reimbursement of covered services.

HCPCS Codes	Description
C9399	Unclassified drugs or biologicals
J3490	Unclassified drugs

Reviews, Revisions, and Approvals	Date
Policy created	01/2026