Original Effective Date: 03/04/2026
Current Effective Date: 03/04/2026
Last P&T Approval/Version: 01/28/2026
Next Review Due By: 07/2026
Policy Number: C30297-A

Forzinity (elamipretide) NC

PRODUCTS AFFECTED

Forzinity (elamipretide)

COVERAGE POLICY

Coverage for services, procedures, medical devices and drugs are dependent upon benefit eligibility as outlined in the member's specific benefit plan. This Coverage Guideline must be read in its entirety to determine coverage eligibility, if any. This Coverage Guideline provides information related to coverage determinations only and does not imply that a service or treatment is clinically appropriate or inappropriate. The provider and the member are responsible for all decisions regarding the appropriateness of care. Providers should provide Molina Healthcare complete medical rationale when requesting any exceptions to these guidelines.

Documentation Requirements:
Molina Healthcare reserves the right to require that additional documentation be made available as part of its coverage determination; quality improvement; and fraud; waste and abuse prevention processes. Documentation required may include, but is not limited to, patient records, test results and credentials of the provider ordering or performing a drug or service. Molina Healthcare may deny reimbursement or take additional appropriate action if the documentation provided does not support the initial determination that the drugs or services were medically necessary, not investigational or experimental, and otherwise within the scope of benefits afforded to the member, and/or the documentation demonstrates a pattern of billing or other practice that is inappropriate or excessive.

DIAGNOSIS:
Barth Syndrome

REQUIRED MEDICAL INFORMATION:
Forzinity (elamipretide) is considered not medically necessary for all indications, including but not limited to Barth Syndrome, due to insufficient evidence of therapeutic value since clinical benefit has not been established. Data from clinical studies of elamipretide in a small number of people with Barth Syndrome demonstrated safety and tolerability. However, the pivotal trial failed to achieve statistically significant change in the primary function and fatigue assessments. In a subsequent open label trial, improvements were seen. Ultimately the approval was based on a secondary endpoint of knee extensor strength as measured by handheld dynamometry. Continued approval is contingent upon verification and description of clinical benefit in a confirmatory trial.

Molina Healthcare will continue to evaluate and update this policy as relevant clinical evidence becomes available to determine whether Forzinity (elamipretide) provides clear clinical benefit or slows progression of the disease.

CONTINUATION OF THERAPY:
NA

DURATION OF APPROVAL:
NA

PRESCRIBER REQUIREMENTS:
NA

AGE RESTRICTIONS:
NA

QUANTITY:
NA

PLACE OF ADMINISTRATION:
NA

DRUG INFORMATION

ROUTE OF ADMINISTRATION:
Subcutaneous

DRUG CLASS:
Mitochondrial Cardiolipin Binder

FDA-APPROVED USES:
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).

COMPENDIAL APPROVED OFF-LABELED USES:
None

APPENDIX

APPENDIX:
Appendix 1:
Stealth BioTherapeutics will evaluate expanded access requests submitted by a treating physician on a case-by-case basis for patients affected with Barth syndrome that is genetically confirmed and provided that the initial request meets the criteria or otherwise set forth in their Expanded Access protocol (see www.ClinicalTrials.gov with identifier NCT04689360).
https://stealthbt.com/expanded-access/

BACKGROUND AND OTHER CONSIDERATIONS

BACKGROUND:
Barth Syndrome is an ultra rare, X-linked genetic disorder that primarily affects males. It is caused by a mutation in the rafazzin gene (TAZ, also G4.5), resulting in an inborn error of phospholipid

metabolism, affecting many systems of the body. Though not always present, cardinal characteristics of this multi-system disorder often include combinations and varying degrees of cardiomyopathy, neutropenia, low muscle mass and muscle weakness, growth delay, exercise intolerance, feeding problems, cardiolipin abnormalities, and 3-methylglutaconic aciduria. Not all manifestations may be simultaneously present or apparent.

Forzinity is the first FDA approved treatment for Barth Syndrome. Treatment for Barth Syndrome primarily consists of treating the patient's specific symptoms.

Forzinity (elamipretide) is a mitochondria-targeting peptide designed to improve the function of mitochondria and energy production in the cells. It is a once daily, subcutaneous injection. It is thought to work by selectively targeting and stabilizing a key mitochondrial lipid called cardiolipin, which plays a crucial role in maintaining mitochondrial structure and function. Forzinity was evaluated in a randomized, double-blind, placebo-controlled, crossover trial and its 192-week, open-label, single-arm extension period. The randomized trial evaluated the efficacy and safety of once daily Forzinity 40 mg injected subcutaneously for 12 weeks in 12 subjects ≥12-years-old and >30 kg with genetically confirmed Barth syndrome. The primary endpoints for the randomized trial were distance walked during 6-minute walk test and Total Fatigue Score on the Barth syndrome Symptom Assessment. Forzinity was not superior to placebo on these primary endpoints. Ten subjects completed the randomized trial and entered the extension period designed to evaluate long-term safety and tolerability of Forzinity. Eight of these 10 subjects participated through Week 168 of the extension period. Knee extensor muscle strength measured by handheld dynamometry was evaluated as one of the secondary endpoints in the randomized trial and in the extension period. Increases in knee extensor muscle strength were not observed during the randomized trial but were observed during the extension period. Adverse reactions occurring more commonly on Forzinity than on placebo include injection site reactions such as injection site erythema, pain, induration, pruritus, bruising, and urticaria. Forzinity is approved under accelerated approval based on an improvement in knee extensor muscle strength, which is an intermediate clinical endpoint. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial.

CONTRAINDICATIONS/EXCLUSIONS/DISCONTINUATION:
All other uses of Forzinity (elamipretide) are considered experimental/investigational. Contraindications to Forzinity (elamipretide) include: Serious hypersensitivity to any of the ingredients, do not use in neonates.

Exclusions/Discontinuation:
There is insufficient information to recommend a dosage regimen in adults with eGFR less than 30 mL/minute and on dialysis.
There is insufficient information to recommend a dosage regimen in pediatric patients weighing 30 kg or greater with renal impairment.
Forzinity was not studied in patients going through pubertal growth spurt, patients with uncontrolled hypertension, patient with history of heart transplant or on the waiting list for heart transplant, or patients with an implantable cardioverter defibrillator (ICD) with recent discharge or patient expected to undergo ICD placement.

OTHER SPECIAL CONSIDERATIONS:
If a dose is missed, skip the dose and take the next dose of Forzinity at the scheduled time. Do not take a double dose of Forzinity.

CODING/BILLING INFORMATION

CODING DISCLAIMER. *Codes listed in this policy are for reference purposes only and may not be all-inclusive or applicable for every state or line of business. Deleted codes and codes which are not effective at the time the service is rendered may not be eligible for reimbursement. Listing of a service or device code in this policy does not guarantee coverage. Coverage is determined by the benefit document. Molina adheres to Current Procedural Terminology (CPT®), a registered trademark of the American Medical Association (AMA). All CPT codes and descriptions are copyrighted by the AMA; this information is included for informational purposes only. Providers and facilities are expected to utilize industry-standard coding practices for all submissions. Molina has the right to reject/deny the claim and recover claim payment(s) if it is determined it is not billed appropriately or not a covered benefit. Molina reserves the right to revise this policy as needed.*

HCPCS CODE	DESCRIPTION
NA	

AVAILABLE DOSAGE FORMS:
Forzinity SOLN 280MG/3.5ML single-patient-use vial

REFERENCES

1. Forzinity (elamipretide) injection, for subcutaneous use [prescribing information]. Needham, MA: Stealth BioTherapeutics Inc.; September 2025.
2. United Mitochondrial Disease Foundation. (2025). FORZINITY™ (elamipretide) UMDF. Retrieved from Umdf.org website: https://umdf.org/forzinity-elamipretide/
3. Barth Syndrome Foundation. (2019). Barth Syndrome Foundation: Barth Syndrome: What is Barth Syndrome? Retrieved from www.barthsyndrome.org website: https://www.barthsyndrome.org/barthsyndrome/
4. National Organization for Rare Disorders. (2025, September). Barth Syndrome - NORD (National Organization for Rare Disorders). Retrieved from NORD (National Organization for Rare Disorders) website: https://rarediseases.org/rare-diseases/barth-syndrome/

SUMMARY OF REVIEW/REVISIONS	DATE
NEW CRITERIA CREATION	Q1 2026