PHARMACY POLICY STATEMENT
Indiana Medicaid

DRUG NAME	Forzinity (elamipretide)
BENEFIT TYPE	Pharmacy
STATUS	Prior Authorization Required

Forzinity, granted accelerated approval by the FDA in 2025, is a mitochondrial cardiolipin binder indicated to improve muscle strength in adult and pediatric patients with Barth syndrome weighing at least 30 kg. The accelerated approval is based on an improvement in knee extensor muscle strength, an intermediate clinical endpoint, observed in the open-label extension (OLE) period of the TAZPOWER clinical trial.
Forzinity did not meet either of its primary endpoints in part 1 of the study. Knee extensor muscle strength was a secondary endpoint, which also did not meet significance in the randomized trial (part 1), but an increase in this outcome was observed with longer term use during the extension phase (part 2) and considered by the FDA to be reasonably predictive of clinical benefit. Forzinity also met several other endpoints during the OLE, as well as in a natural history cohort comparison study.
Barth syndrome is a rare X-linked disorder of mitochondrial cardiolipin (CL) metabolism. It is a multisystem disorder characterized by cardiomyopathy, neutropenia, muscle weakness, fatigue, and other features. It is caused by mutations in the *TAZ* gene (also known as *TAFFAZIN*), which is involved in production of CL, which plays a role in mitochondrial function.
Forzinity is the first drug approved for Barth syndrome. It acts mechanistically by improving mitochondrial morphology and function.

Forzinity (elamipretide) will be considered for coverage when the following criteria are met:

Barth Syndrome

For **initial** authorization:

1. Member is a male, at least 12 years of age, and weighs at least 30 kg; AND
2. Medication must be prescribed by or in consultation with a cardiologist, neurologist, or geneticist; AND
3. Member has a documented diagnosis of Barth syndrome confirmed by genetic testing that shows a pathogenic variant in the *TAZ* gene; AND
4. Member is ambulatory with documentation of an impaired baseline 6-minute walk test (6MWT); AND
5. Member meets the following renal function requirement:
   a) Adult: GFR at least 30 mL/min and not on dialysis
   b) Pediatric: No renal impairment; AND
6. Member has NOT had a heart transplant and is not currently on a transplant waitlist.
7. **Dosage allowed/Quantity limit:** 40 mg subcutaneously once daily. QL: 4 vials per 28 days.

If all the above requirements are met, the medication will be approved for 6 months.

For **reauthorization**:

1. Chart notes must document positive clinical response to treatment with improvement of at least one of the following: 6MWT, total fatigue score on the Barth Syndrome Symptom Assessment (BTHS-SA), knee extensor muscle strength as measured by handheld dynamometry (HHD), five times sit-to-stand test (5XSST), or left ventricular stroke volume on an echocardiogram (ECHO).

If all the above requirements are met, the medication will be approved for an additional 6 months.

CareSource considers Forzinity (elamipretide) not medically necessary for the treatment of conditions that are not listed in this document. For any other indication, please refer to the Off-Label policy.

DATE	ACTION/DESCRIPTION
10/30/2025	New policy created for Forzinity.

References:

1. Forzinity [prescribing information]. Stealth BioTherapeutics Inc.; 2025.
2. 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
3. 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;26(7):101138. doi:10.1016/j.gim.2024.101138
4. Hornby B, Thompson WR, Almuqbil M, et al. Natural history comparison study to assess the efficacy of elamipretide in patients with Barth syndrome. *Orphanet J Rare Dis*. 2022;17(1):336. Published 2022 Sep 2. doi:10.1186/s13023-022-02469-5

Effective date: 05/01/2026
Revised date: 10/30/2025