

SS-31 — Research Summary

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1. What It Is

SS-31 (also known as elamipretide, MTP-131, Bendavia) is a cell-permeable, mitochondria-targeted tetrapeptide (D-Arg-2'6'-dimethylTyr-Lys-Phe-NH₂) with an alternating aromatic-cationic structure that concentrates in the inner mitochondrial membrane and binds reversibly to cardiolipin, a phospholipid essential for electron transport chain organization and mitochondrial energy production.

Major regulatory update: SS-31 (elamipretide) received FDA accelerated approval in September 2025, brand name FORZINITY, to improve muscle strength in adult and pediatric patients (≥30 kg) with Barth syndrome — the first FDA-approved mitochondria-targeted therapeutic and the first approved treatment for Barth syndrome. Developed by Stealth BioTherapeutics.

2. Reported Effects in Research Literature

SS-31/elamipretide has one of the most extensive human clinical trial records of any compound in this set, spanning multiple rare mitochondrial disease programs.

- FDA approval basis (TAZPOWER trial, NCT03098797): A randomized, double-blind, placebo-controlled crossover trial in 12 Barth syndrome patients (≥12 years, >30kg) did not meet its primary endpoints (6-minute walk test, BTHS symptom assessment) in the randomized phase, but knee extensor muscle strength improved during the open-label extension — 10 of 12 patients entered the extension, with 8 reaching week 168 (median muscle-strength change: 63 Newtons). Approval was granted under the accelerated pathway based on this intermediate endpoint, with a confirmatory trial required.
- A separate natural-history comparison study assessed elamipretide's efficacy in Barth syndrome patients against an untreated historical cohort.
- Beyond Barth syndrome, SS-31/elamipretide has undergone Phase II/III trials for primary mitochondrial myopathy (MMPOWER trials, showing mixed results) and heart failure, and received FDA Rare Pediatric Disease designation.
- Preclinical (aged mice): Siegel et al. (2013) found the mitochondria-targeted peptide rapidly improved mitochondrial energetics and skeletal muscle performance in aged mice.
- Preclinical (Barth syndrome mouse model): SS-31 treatment improved cardiac mitochondrial morphology and mitophagy, and improved mitochondrial respiratory chain efficiency, in a TAFAZZIN-knockdown mouse model.
- Preclinical (Friedreich's ataxia): SS-31 was reported to reverse mitochondrial fragmentation (a Drp1-dependent homeostatic response) and upregulate frataxin expression in Friedreich ataxia models.
- Preclinical (DCMA, a related mitochondrial cardiomyopathy): SS-31 reversed mitochondrial fragmentation in skin fibroblasts from pediatric patients with DCMA, a disorder phenotypically related to Barth syndrome.
- Safety: Across multiple Phase II/III trials, SS-31/elamipretide has been generally well tolerated; injection site reactions are the most commonly reported adverse effect.

3. Clinical & Preclinical Study References

Hornby B, et al. "Natural history comparison study to assess the efficacy of elamipretide in patients with Barth syndrome." Orphanet J Rare Dis. 2022;17:336.

Phase 2/3 randomized clinical trial (TAZPOWER) with open-label extension. PMC7935714.

Russo S, De Rasmio D, Signorile A, et al. "Beneficial effects of SS-31 peptide on cardiac mitochondrial dysfunction in Tafazzin knockdown mice." Sci Rep. 2022;12:19847.

Siegel MP, Kruse SE, Percival JM, et al. "Mitochondrial-targeted peptide rapidly improves mitochondrial energetics and skeletal muscle performance in aged mice." Aging Cell. 2013;12(5):763-771. PMID: 23692570

FDA Integrated Review, NDA 215244 (elamipretide/Forzinity), accessdata.fda.gov.

Regulatory/Safety Note: SS-31 (elamipretide) is FDA-approved by prescription as FORZINITY for a single narrow indication — muscle strength in Barth syndrome patients weighing ≥ 30 kg — under the accelerated approval pathway, contingent on a confirmatory trial. It is not approved in neonates or for any other indication. Non-prescription/research-labeled SS-31 is a distinct regulatory category from the approved product.

This compound has not been evaluated by the FDA and is not intended to diagnose, treat, cure, or prevent any disease in humans or animals.

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