

BSF Strategic Plan 2026–2028

Executive Summary

VISION

A world in which everyone impacted by Barth syndrome can thrive and live the life they choose.

MISSION

To collectively and tenaciously work toward saving and improving the lives of those impacted by Barth syndrome.

Over the next three years, we'll pursue this mission by focusing on five things.

01 Protecting the Heart of the Community

Our community is growing globally and spans more generations, life stages, and disease presentations than ever before. Our job is to meet families and affected individuals with information they can trust and connections that cut isolation.

- Support every step of our community's individual and collective experiences with Barth syndrome
- Design a future family services model that can evolve as the community grows and changes
- Convene scientists, healthcare providers, and families for a multidimensional international Barth syndrome conference in 2026 and 2028
- Strengthen collaborations between BSF and international affiliates to support global community
- Continue to incorporate the perspectives of families in guiding our work
- Rebuild the BSF website as an engaging front door for every audience we serve

02 Driving Toward Treatments and a Cure

The first drug approval for Barth syndrome shows what's possible even for an ultra-rare disease when patient communities, regulators, and industry work together. We'll foster new opportunities and coordinate closely with partners on the development and accessibility of therapies, rallying our community when targeted action can make a real difference.

- Support follow-on work from FORZINITY™ 2025 approval
 - Provide patient education for Stealth BioTherapeutics' phase IV trial
 - Coordinate with international affiliates on trial activities and geographic expansion of approvals and access
 - Push for label expansion to younger patients as the clinical picture supports it
 - Help U.S. families navigate reimbursement and access barriers

- Continue developing additional treatments by advancing multiple potential therapies of various stages and types that address different targets
- Emphasize later stage opportunities to expedite treatment options
- Support ongoing gene therapy work and explore a venture philanthropy model

03 Investing in Research

A strong, advancing pipeline of multiple programs, ranging from work that deepens our understanding of Barth syndrome to seeding therapy development, is our best long-term bet to ensure people with Barth syndrome can live the life they choose. We'll fill gaps deliberately, create and sustain tools researchers depend on, and be the obvious partner for anyone working on Barth syndrome.

- Proactively work to fill knowledge and research gaps, including gastrointestinal health and biomarker and clinical endpoint work
- Continue funding the seed grant program, Barth syndrome's main source of proof-of-concept funding
- Derisk critical early-stage projects to strategically bridge them to new milestones, increasing funding pathways of potential treatments for Barth syndrome
- Strengthen community resources necessary for a robust research program, including the patient registry, biorepository, and broader research infrastructure

04 Advocating for the Community

Relationships with regulators, legislators, and patient advocacy coalitions have produced real results. We'll keep up those relationships and bring along the next generation of advocates.

- Keep current with key elected officials, federal agencies, and media contacts
- Develop the next generation of advocates
- Contribute to ultra-rare disease policy work, as appropriate
- Use targeted help to support families with reimbursement and access

05 Never, Ever Giving Up

Tenacity has carried BSF from a handful of families looking for answers to a global community with real influence. But tenacity alone won't get us through what comes next. It takes real investment in the fundamentals that let a small team do extraordinary things for decades to come.

- Build an effective fundraising program grounded in reality
- Invest in technology, including generative AI, not to replace the human touch rather to extend what our small team can do
- Strengthen our volunteer leadership, including a thoughtful board succession plan
- Grow our volunteer program