

Mission

To cure Multiple Sulfatase Deficiency

Vision

We aspire for all children with MSD to lead a life without limitations.

Values

Compassion | Inclusion | Community
Collaboration | Responsibility



Strategic Priority #1: Research at Our Core

Goals—

- Help usher the start of AAV9 gene therapy clinical trials (tentative start Fall 2024)
- Complete Prospective Natural History Study by December 2025
- Launch and fully utilize the Patient Registry
- Create a Research Strategy Map by August 2024
- Create more opportunities for dialogue and collaboration between researchers

Strategic Priority #2: Supporting Families with Resources and Community

Goals—

- Develop and maintain a comprehensive online resource hub for our MSD families
- Find better ways to engage our Angel families and develop a transition pipeline for these families after their children have passed
- Create partnerships and resource lists for supports we don't currently offer
- Support international families through translation and interpretation services
- Create engagement opportunities throughout the organization for families
- Promote social connection between families

Strategic Priority #4: Setting a Foundation Through Organizational Capacity

Goals—

- Build an effective Board of Directors for now and in the future
- Cultivate skilled and stable personnel
- Ensure financial resources are available to support mission and strategic priorities
- Utilize volunteers to drive awareness and fundraising goals
- Optimize productivity and efficiency by focusing on effective strategies and leveraging available resources

Strategic Priority #3: Growing Global Awareness of MSD

Goals—

- Make strides on Newborn Screening
- Host a successful 2024 International Scientific and Family Conference
- Gain more visibility in medical communities
- Connect with the hundreds of MSD families still unidentified
- Maximize organizational visibility, and value and impact to stakeholders