

World FSHD Alliance Strategic Plan

Vision – Treatments and a cure for FSHD

Mission – Accelerate the development of treatments while empowering people living with FSHD

The primary purpose of World FSHD Alliance is to **connect and activate the FSHD community globally** in order to build a worldwide community of activists, empower patients, and advance our shared mission of accelerating the development of therapies. A global program is needed because together, by sharing resources, strategy, and “speaking in one voice,” we can accomplish much more in education, support, and advocacy. Just as importantly, FSHD clinical trials will be international in scope. Coordinating our messages and outreach efforts will result in more timely, cost-effective ways to engage with industry around drug development, clinical trials, regulatory approval, and patients’ access to therapies.

Benefits to the alliance members

- Enhances credibility and influence of organizations as members of a global alliance;
- Builds networks locally and nationally to improve quality of patients’ lives;
- Gives communities greater influence;
- Shares educational resources and best practices;
- Shares knowledge and best practices around fundraising;
- Creates opportunities for previously overlooked populations to participate in research;
- Provides guidance and support for local/national communications and marketing;
- Provides the network to operationalize global campaigns;
- Empowers patient communities to collaborate with industry partners.

Alliance goals

- 1) Accelerate the development of therapies by
 - a. Supporting centers of excellence for FSHD by
 - Promoting high standard of care to patients
 - Helping to build capacity to conduct clinical research and trials
 - b. Supporting resources for clinical trial preparedness, including:
 - Strong patient advocacy network
 - Patient registries following TREAT-NMD standard
 - Advocating for genetic testing for patients and families
 - Advocating for natural history studies
 - c. Networking with international researchers and companies
 - d. Encouraging young researchers to work on FSHD
- 2) Activate and empower patients and families by
 - a. Building strong, sustainable networks for patient education and support. For individual organizations, this would include:
 - Developing leadership teams
 - Building digital member databases
 - Using website and email effectively to educate, engage, and activate

- Organizing meetings
 - Supporting patient registries
- b. Improve patient care
- Getting people into the appropriate health care system
 - Finding and engaging clinicians interested in FSHD
 - Advocating for genetic counseling and testing resources
 - Advocating for physiotherapy/kinesiotherapy & exercise resources
 - Advocating for mental health resources
 - Providing social support and mental health resources

Proposed program aspirations and activities

1. World FSHD Alliance leadership will convene regular meetings (virtual and in-person) to:
 - a. Develop a charter and strategic plan for the Alliance
 - b. Have ongoing, transparent, timely communication about all members' activities and important news regarding research, clinical studies, etc.
 - c. Share knowledge and guidance on developing patient education and engagement
 - d. Share knowledge and guidance on developing research and infrastructure
 - e. Possible activities for implementing the above include
 - Research advocacy webinars
 - Annual summit
 - Regular newsletter
 - Create a website or chat forum
2. Promote training and best practices for patient organization leadership, governance, establishment, marketing, fundraising & development
 - a. Patient advocacy/activation leadership trainings
 - b. Member organizations to set organizational priorities
 - c. Develop leadership teams
3. Community engagement
 - a. Develop relationships with researchers, clinicians, study coordinators
 - b. Make it easier for FSHD patients to access:
 - Research registries
 - Genetic testing labs
 - Neuromuscular clinics
 - Physical therapy and occupational therapy
 - Mental health and psychosocial support
 - Nutritional guidance
 - c. Organize patient meetings and education conferences
 - d. Advocate for registries – build to international standard
 - e. Advocate for natural history studies
 - f. Communications & marketing to members

4. Clinician engagement

- a. Promote education of medical professionals about international care standards in the diagnosis and management of FSHD
- b. Involve physiotherapists, genetic counselors, etc. in screening/diagnosis. A network has evolved for spinal muscular atrophy (SMA). We can examine it as a model for a similar effort for FSHD.

5. Researcher engagement

- a. Connect with and support local research activities.
- b. Assist with patient volunteer recruitment for research.
- c. Engage researchers in patient-driven conferences, education.
- d. Connect researchers to international networks (such as FSHD Clinical Trial Research Network and TREAT-NMD).