



Vision of Hope
Meeting the Needs of Today

NNPDF Family Conference

August 12, 2017

DRAFT OF THE VISION FOR THE FUTURE

We envision an enduring patient support community that embodies strength, hope and empathy for those affected by all Niemann-Pick Disease types. We believe deeply in the future of research that promotes clinical treatments with potential to improve patient quality of life and with intent to ultimately cure all Niemann-Pick Disease types.

MISSION

We are a non-profit group dedicated to supporting and empowering patients and families affected by Niemann-Pick disease through education, collaboration and research.

VALUES

We are committed to:

- Supporting and empowering patients and families affected by NPD by meeting them where they are in their NPD journey.
- Upholding a balanced, unbiased approach with families, researchers, clinicians and industry to move treatment forward to provide a variety of therapies and to work together toward a cure. We encourage full transparency among all stakeholders in the Niemann-Pick Disease community.
- Supporting basic science and clinical research through awareness and collaborative funding initiatives.
- Adhering to a written code of conduct that fosters respect for all, regardless of treatment choices or research perspectives, which maintains patient privacy and follows the national HIPPA regulations.
- Encouraging and promoting advocacy and collaborative community partnerships across the NPD community, both in the U.S. and internationally.
- Respecting the diversity of Board members, employees, families, researchers, clinicians and industry, and governing the NNPDF ethically and transparently according to NNPDF Bylaws.

WHO WE ARE

The National Niemann-Pick Disease Foundation is a national non-profit 501(c)(3), rare disease network of families dedicated to providing empowerment resources, support and education for families, caregivers and patients affected by all Niemann-Pick Disease types.

We are families, patients and compassionate friends across the U.S. that share hope, promote research and raise awareness for NPD. We are dedicated to supporting and empowering one another, through education of potential therapies for all Niemann-Pick Disease types. We work tirelessly as volunteers, caregivers, counselors, and fundraisers for patient support, research and clinical treatment.

WHAT DO WE DO

We provide *Support and Advocacy Services* that include:

- Support that empowers families, caregivers and patients from diagnosis, through disease progression, including therapies and grief.
- Information and referral for health care issues.
- First point of contact and community liaison to industry representatives considering or currently investing in therapeutic treatments for NPD.
- Community representation and education regarding drug development and regulatory process.
- Affiliation with the international patient registry which is critical for attracting further industry investments in potential treatments for all Niemann-Pick Disease types.
- A network of family members who can provide support and information to fellow patients/families throughout the journey.

We provide *Educational Resources* that include:

- Accurate and current information about all Niemann-Pick Disease type carrier screening, physicians, and allied health care professionals.
- Accurate, current and unbiased information about experimental trials and expanded access treatment options for all Niemann-Pick Disease types.
- Ongoing, timely information on advocacy, health care, and support resources relevant to the Niemann-Pick communities.
- Clearinghouse for accurate, factual, current information on basic science and clinical research findings which are translated into lay language.

WHY A REINVENTED FOUNDATION WILL NEED SUPPORT

A reinvented foundation will need financial support in order to maintain a patient group capable of meeting the diverse needs of the NPD community. This reinvented foundation, focused on patient support and advocacy, will continue to attract clinical and industry interest in finding effective therapies and potential cures for all disease types. Only through the generous contributions across this diverse community, including from family foundations focused on raising research monies, will we be able to succeed as a community together. A collaboration underlying this financial support is essential for the success of our collective future.