



National Niemann-Pick Disease Foundation, Inc.
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NNPDF Industry Policy Statement

The National Niemann-Pick Disease Foundation, NNPDF, represents the concerns and needs of patients and families with Niemann-Pick Disease. An important part of our mission is to recognize, build and maintain relationships with the pharmaceutical/biotechnology companies that are US Food and Drug Administration (FDA) approved or are developing potential treatments for individuals with Niemann-Pick Disease ASMD, (Type A & B), and Niemann-Pick Type C. Our role is to provide accurate and unbiased educational information to families about available and future clinical treatments and to advocate for the needs of our community around these therapies.

NNPDF upholds the following core values in working with companies:

1. Transparency in providing information with families/patients
2. Developing partnerships on topics of mutual interest
3. Respect for independence as a membership governed patient advocacy group
4. Honoring the collective interests of family/patient
5. Ethical practices that ensure a professional boundary between patients and families
6. Adherence to privacy regulations around sharing patient and family information
7. Open and timely communication with responses within 3 -5 business days

The role of the company is to inform the NNPDF about their activities and programs related to Niemann-Pick diseases, and where applicable, the benefits and risks of approved treatment. In addition, companies are expected to provide scientific and educational information to families and where applicable, seek input from the NNPDF into their efforts as it pertains to the needs of the community.

Companies benefit in working with NNPDF in a variety of ways, including:

- Developing a unique perspective about the diseases
- Gaining access to the patient population where appropriate
- Understanding the needs and priorities of NNPDF members
- Acquiring knowledge of disease and family preference to inform clinical trial design
- Advancing patient education
- Receiving feedback on new treatments
- Obtaining advocacy support during the regulatory approval process.

This interaction also provides opportunities for project collaboration including but not limited to general and medical education about the diseases and advocacy for a product/treatment approval and health insurance reimbursement. The NNPDF is actively building a relationship with the FDA in order to represent the voice of Niemann-Pick Disease patients and families, as it pertains to representing the need for approved clinical treatments.

The primary liaison to companies for the NNPDF is the Executive Director. Designated NNPDF Board members may also serve as secondary liaisons between the company and the NNPDF. Communication should be directed to the appointed primary and secondary liaisons at all times to ensure proper coordination with the community. The NNPDF will notify the company of any changes to the liaisons.

NNPDF Information Sharing with the Community

The aim of NNPDF in sharing information is to be a conduit for patients and families regarding up-to-date, unbiased, accurate and fair-balanced information. This includes the latest research and clinical trial information. The community at large is actively advancing research to attract pharmaceutical investment in potential therapies and a cure. It is critical that the information we provide to our members is made available in an organized and easily accessible manner that is open and transparent. Our hope is to realize a future where these therapies will make a positive impact on the lives of many children and adults.

For newly diagnosed families and those contacting NNPDF around trials/expanded access therapies, a phone intake process typically takes place to understand what type of information is desired. Staff shares accurate and fair-balanced information in an unbiased manner and as well offers an opportunity for the family member to speak with a designated Board member. For those families and patients asking for information on social media on our NNPDF Awareness Facebook page, we provide information on a closed message page and ask members to contact the office directly.

Examples of NNPDF Industry Partnerships Involving Funding

Donations/Charitable Contributions: NNPDF will submit requests for targeted or unrestricted operating financial grants to assist in the management of the organization. The donations must be given without conditions, and *no quid pro quo* arrangement will be accepted. Monies received benefit all NNPDF members, not just the subset served by the product of the company.

Company-Sponsored Meetings: The NNPDF will accept company funds for company-sponsored meetings. Funds received by NNPDF will be available for any family in that region attending the meeting, regardless of the disease type. The NNPDF assumes no responsibility for meeting planning. The NNPDF recognizes that the companies are legally limited to services that they can provide for meetings where patients are present, such as speakers, meals and travel to the meetings.

Educational Projects: The NNPDF also accepts company funds for collaborative educational projects. Although these are branded projects between or among the companies and the NNPDF, the NNPDF is responsible for payment of services associated with the projects.

Consultants: NNPDF staff may serve as consultants or working members on committees, or members of a speakers' bureau. Any consulting work, including presentations, for which the staff receives personal compensation, must be scheduled on time taken off from work.

Alternatively, staff can request that compensation be paid directly to the NNPDF, whereby the consulting is considered work time. Compensation includes reimbursement for travel and meal expenses. Staff needs to disclose if they take on consultative relationship with industry.

Guidelines for Sharing Clinical Trial and Expanded Access Information

1. Press releases or notifications received in the NNPDF Central Office are posted on the NNPDF website under the appropriate pharmaceutical company heading
2. At the same time it is received and posted to the website, we will make a summary post on the NNPDF Facebook Page and/or NNPDF Awareness group with a link back to our website where families can find more detailed information. We make a statement that we have received updated information from (insert pharma name) regarding a specific topic, and they can find more detailed information at (insert link to our website).
3. All pharmaceutical company updates are included in the next monthly e-newsletter which comes out the third Tuesday of every month and provides links back to the original posting on the website.
4. All those interested in receiving this important and educational information subscribe to the NNPDF Blog via the website.

Approved by the National Niemann-Pick Disease Board of Directors on January 16, 2018.