



National Tay-Sachs &
Allied Diseases Association

INDUSTRY PARTNERSHIP GUIDELINES

2023



The NTSAD Industry Partnership Guidelines were established to optimize the engagement between NTSAD and the industry partners with whom they interact.



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About NTSAD

MISSION

NTSAD leads the worldwide fight to treat and cure Tay-Sachs, Canavan, GM1, and Sandhoff diseases by driving research, forging collaboration, and fostering community. Supporting families is the center of everything we do.

VISION

NTSAD envisions a world in which Tay-Sachs, Canavan, GM1, and Sandhoff diseases are no longer fatal or debilitating. To uphold our vision, NTSAD will:

- Provide compassionate support, advocate for affected individuals and their families, and promote early diagnosis and prevention
- Empower, educate, and connect affected individuals and families
- Be a key resource for individuals, families, clinicians, researchers, and industry members
- Direct, promote, and invest in research to accelerate the development of treatments, therapies, and cures
- Serve as a leader within the rare disease community



GOALS AND OBJECTIVES

- Establish and execute “best practices” for building collaborative partnerships between NTSAD and the companies with whom they interact.
- Create and maintain relationships rooted in mutual respect that benefit the patient community and experiences.
- Possess a clear understanding of the terms of engagement.
- Maintain direct dialogue and transparency.
- Re-assess and re-evaluate partnerships as needed or when circumstances change.



PARTNERSHIP

Key Contributors of the Partnership

- NTSAD's CEO and Director of Family Services
- Company's rare disease patient advocacy leader*
- Company's rare disease patient advocacy key staff members

** When an international company has multiple contacts, the U.S. patient advocacy leader will serve as the main point of contact, unless otherwise defined.*

Engagement Parameters

- Commitment to mutual collaboration
- Regular and frequent interaction, with a preference to connect at least once per month
- Meetings at scientific/advocacy conferences
- Transparency and accountability
- Participation in NTSAD's periodic Industry Roundtable Meetings
- Respect for each other and the process
- Confidentiality Agreement, if applicable





COMMUNITY ENGAGEMENT

Patient families and caregivers provide valuable input during drug development and clinical trial design processes.

Engagement with NTSAD and Affected Individuals, Families, and Caregivers

- NTSAD can be a valued resource to industry partners; early engagement is encouraged.
- NTSAD represents the voice of affected individuals and families, connecting patient families with companies.
- NTSAD serves as a communication liaison between patient families and industry partners.
- NTSAD serves as an advocate for affected individuals and families.
- Companies share engagement opportunities with NTSAD, as appropriate.
- Companies, individually and collaboratively as a group, can engage with NTSAD and its patient community in myriad ways (i.e., sharing of expertise, insight, financial support, educational resources).
- Companies that wish to interact with affected individuals or families present to NTSAD a clear description of engagement parameters and purpose.



COMMUNITY ENGAGEMENT

Confidentiality and Patient Privacy

- Company interactions with NTSAD respect patient privacy; patient/family health data is de-identified/ anonymized.
- NTSAD willingly signs and adheres to Confidentiality Agreements when requested by industry partners.

Research, Drug Development, and Clinical Trials

- Companies share periodic updates with NTSAD on the status of research, drug development, and clinical trial data.
- Company representatives serve as subject matter experts and key opinion leaders.
- NTSAD shares company communications with the patient community, as appropriate.
- NTSAD invites companies to present updates at its Annual Family Conference Research Day.
- NTSAD educates families and individuals about drug development and clinical trial processes, and provides coaching and support, when needed and as appropriate.

INVESTING IN NTSAD'S PATIENT ADVOCACY



NTSAD is a 501(c) non-profit organization that is sustained through philanthropic support from industry partners, foundations, and individual donors.

Financial Contributions

NTSAD drives research, forges collaboration, and fosters community. Funding the programs and services NTSAD provides to individuals and families affected by Tay-Sachs, Canavan, GM1, and Sandhoff disease.

NTSAD receives support from companies via:

- program or educational grants,
- conference and event sponsorships,
- general unrestricted donations.



INVESTING IN NTSAD'S PATIENT ADVOCACY



In-Kind Support

NTSAD also receives in-kind (non-monetary) support from companies via:

- Sharing expertise and insight,
- Collaboration with other industry partners,
- Offering resources,
- Educational program development

***NTSAD is grateful for the support
we receive from industry partners.***





APPENDIX

Examples of How NTSAD Engages with Industry Partners

- Share the voice of the affected individuals and families
- Connect companies with patients (to share their stories)
- Advocate – clinical trial design, newborn screening, industry and FDA accountability
- Share information/communication/updates with NTSAD community (i.e., meetings, educational programs, community letters)
- Serve on patient advocacy advisory committee/board, standing council
- Natural history studies – educate and connect
- Clinical trial design/protocol feedback
- Patient family focus group coordination/recruitment
- Attend company events/meetings to represent the family experience, when invited
- Invite company representative to speak at conferences, educational webinars, etc.
- Collaborate with companies on producing educational materials/resources
- Support research initiatives

APPENDIX

Examples of How Industry Partners Engage with NTSAD

- Provide status updates on pipeline, research, clinical trials/data, FDA meetings, economic impact on company's program, if applicable
- Invite NTSAD to serve on a patient advocacy advisory committee/board or standing council
- Invite NTSAD to speak to company researchers/lab staff or at a Rare Disease Day event
- Join the NTSAD community in raising awareness (i.e., social media posts, Day of Hope)
- Collaborate with NTSAD on educational materials or media
- Serve as a resource or subject matter expert
- Offer meeting space
- Provide financial support for: NTSAD events (Annual Family Conference, *Imagine & Believe*); Family Services programming and support; custom focus groups, Externally-Led Patient-Focused Drug Development meetings, International GM1 and GM2 Alliance (IGGA), etc.

APPENDIX

Other Engagement Opportunities to Explore with NTSAD

- Attend FDA/regulatory meetings with company, when appropriate.
- Invite NTSAD or affected individuals, families, and caregivers to investor meetings to share their stories.
- Participate in NTSAD's annual Industry Roundtable Meeting.





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**National Tay-Sachs &
Allied Diseases Association**

Supporting families is the center of everything we do