Engaging Patients and Patient Advocacy Organizations (PAOs) in the Rare Disease Drug Development Process

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PURPOSE

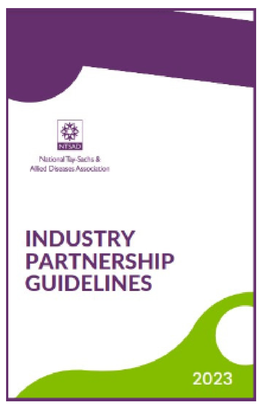
Engaging patients and patient advocacy organizations (PAOs) in the rare disease drug development process has a significant impact. The abstract aims to increase awareness of ways the patient community serves as a valued partner of drug developers and spotlights the contribution of patient voices and PAOs in the research and drug development process.

BACKGROUND

As experts who possess experience with a condition that is ultra-rare, patients and advocacy organizations can inform research strategy, trial design, and regulatory decisions. As a conduit between patients and drug developers, PAOs play a critical role from basic science to human studies, i.e., from bench research through drug development, recruitment, and commercialization. In addition to contributing scientific data related to disease progression, patients and advocates provide an intrinsic understanding of, and insight into, the impact on daily life and unmet needs and can shape pre-clinical research, trial design, therapeutic approval, and post-approval patient services.

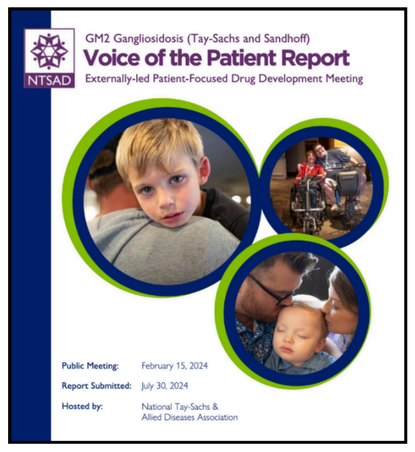
METHODS

The NTSAD community amplifies rare disease awareness for Tay-Sachs, Canavan, GM1 Gangliosidosis, and Sandhoff diseases and actively interacts with the scientific, biotechnology/pharmaceutical, and regulatory communities. In 2022, NTSAD established an Industry Roundtable that meets periodically and produced Industry Partnership Guidelines that outline best practices for engagement between biotechnology /pharmaceutical companies and NTSAD.



Some examples of how NTSAD and members of the NTSAD community have contributed to research and drug development include:

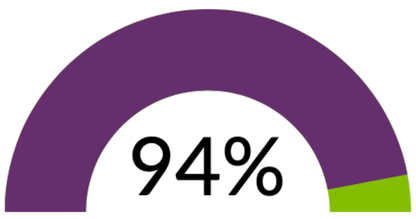
- Annual Family Conferences
- scientific symposia
- Scientific Advisory Council
- scientific consortiums and task forces
- educational programs
- communication about clinical trial and natural history study opportunities
- Research Initiative Program (grants)
- focus groups
- advisory boards
- legislative advocacy efforts
- patient-led meetings with the FDA and key stakeholders
- collaborations and initiatives within the domestic and international rare disease ecosystem
- co-founding of the Global GM1 & GM2 Alliance



GLOBAL GM1 & GM2 ALLIANCE

RESULTS

When patients and PAOs are invited to engage in the drug development process, a mutually beneficial partnership is born. Patient input that informs pre-clinical and trial design early in the process can assist in defining unmet needs, meaningful trial protocols and endpoints, and insight into benefit-risk assessment. *Best Practices for Relationship Building with Patient Advocacy Organizations** survey results indicated that 94% of PAO leaders believe interactions between companies and PAOs should begin as early as possible, and 100% agreed that engagement should begin no later than the pre-clinical and/or Phase 1/2 stage.



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* *"Best Practices for Relationship Building with Patient Advocacy Organizations Workshop" – 2023 Chief Patient Officer Summit; Boston, MA*

CONCLUSION

The goal of enhancing the engagement of patients and PAOs with drug developers is to accelerate paths toward therapeutic approvals. Incorporating valuable insights and "lived" experiences will hopefully translate to meaningful clinical outcomes and identifying efficacious treatments

NTSAD recognizes and thanks members of its Industry Roundtable, members of the regulatory agencies, its Scientific Advisory Council and Task Forces, and, most importantly, affected individuals and family members of the NTSAD rare disease community for their commitment to accelerate research and drug development, as together we strive towards approved therapies for Tay-Sachs, Canavan, GM1 gangliosidosis, and Sandhoff diseases.

Special thanks to the Global GM1 & GM2 Alliance co-founder Daniel Lewi of the CATS Foundation (UK), Jean Campbell of JF Campbell Consultants who co-presented the Best Practices for Relationship Building with Patient Advocacy Organizations workshop, and Patti Engel and Austin Letcher at Engage Health who provided pro bono services for the PAO survey.

NATIONAL TAY-SACHS & ALLIED DISEASES ASSOCIATION *leads the worldwide fight to treat and cure Tay-Sachs, Canavan, GM1, and Sandhoff diseases by driving research, forging collaborations, and fostering community. Supporting families is the center of everything we do.*

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