

National Tay Sachs & Allied Diseases Recruits Industry Leaders for Advisory Council

Boston, MA—January 30, 2015--[National Tay-Sachs & Allied Diseases](#), the nation's oldest rare disease patient advocacy group, is intensifying efforts to find treatments for neurodegenerative disorders with the formation of a Corporate Advisory Council (CAC). Comprised of industry leaders with direct experience in developing, funding and commercializing therapies for rare diseases, the CAC will help NTSAD advance its research programs, build clinical trial readiness and promote the rare disease organization to potential partners by highlighting its access to patients, to animal models and other resources.

In 2013, the FDA granted NTSAD orphan drug designations for a Tay-Sachs and Sandhoff gene therapy treatments. Research is currently in pre-clinical development stages in partnership with the Tay-Sachs Gene Therapy (TSGT) Consortium, whose goal is the initiation of a gene therapy clinical trial for Tay-Sachs disease and Sandhoff disease.

“NTSAD’s rich resources should be attractive to companies interested in bringing a therapy to market for this group of lysosomal storage diseases,” said CAC Chair, Marion Howard, M.D., Ph.D., founder of Cambridge BioStrategies. “To build closer ties with these companies, we have assembled a group of experts in both drug development and commercialization who have not only brought drugs for rare diseases to market, but also have a passion for this field.”

Other CAC members are:

- Oved Amitay, VP, Head of Commercial, Alnylam Pharmaceuticals
- Ritu Baral, Managing Director, Senior Biotechnology Analyst, Cowen and Company
- Michael Gladstone, Life Sciences Group Associate, Atlas Venture
- Kate Haviland, VP Rare Diseases, Idera Pharmaceuticals
- Joan Keutzer, Ph.D., VP of Scientific Affairs, Genzyme Corporation
- Greg Licholai, M.D., VP and General Manager, McKinsey Solutions company Spotlight
- Bradley Campbell, President and COO, Amicus Therapeutics

“The CAC guidance is proving invaluable to our clinical trial readiness for Phase I clinical trials,” said NTSAD Executive Director, Susan Kahn. “In conjunction with our Scientific Advisory Board and the Tay-Sachs Gene Therapy Consortium, they are also influencing the direction of our basic research grants to expeditiously bring a therapy to market.”

About NTSAD

National Tay-Sachs & Allied Diseases Association (NTSAD) is one of the oldest patient advocacy groups in the country. NTSAD focuses on supporting research and the needs of over 500 families and individuals worldwide in several ways, including raising awareness to prevent disease. Their programs and services includes providing comprehensive support services to affected families through their Peer Support Group. The NTSAD research support program aims to direct, fund and promote promising research to develop treatments and cures. NTSAD also offers educational and awareness programs directly and through collaborations with other rare disease programs and community partners. On a broader front, the association advocates for families and persons of all ages with disabilities on an individual, state and national level.

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