

National Ataxia Foundation Announces Launch of NAF Drug Development Collaborative

Pre-competitive pharma industry consortium looks to accelerate development of treatments for Ataxia

Minneapolis, MN (November 5, 2020) –

The National Ataxia Foundation (NAF) announced today the launch of the NAF Drug Development Collaborative. This pre-competitive pharmaceutical industry consortium has a principal goal of accelerating the development of treatments for Ataxia. The group will address the many shared opportunities and challenges in therapy development for this complex neurological disease.

The Collaborative provides a centralized source for access to resources needed to support research and development of Ataxia therapies. Members will benefit from the integration of the patient experience with sound Ataxia scientific and clinical expertise. NAF brings more than 60 years of experience in supporting patients and caregivers and connecting them with research and clinical trial opportunities. NAF has also funded a network of Ataxia clinicians at sites around the US that will be a critical component of the Collaborative's work. Specific objectives of the Collaborative include natural history and biosample data collection, development of biomarkers, validation of rating scales, clinical trial design, patient-reported outcomes and other data necessary for the development and approval of safe and effective therapies.

Initial members of the Collaborative are six leading international pharmaceutical companies all dedicated to developing treatments for Ataxia:

- Biohaven Pharmaceuticals (NYSE: BHVN)
- Ionis Pharmaceuticals (Nasdaq: IONS)
- Seelos Therapeutics (Nasdaq: SEEL)
- Takeda Pharmaceutical Company Limited
- uniQure (Nasdaq: QURE)
- Vico Therapeutics (Private)

Andrew Rosen, Executive Director of NAF, commented, "This is an exciting time for the Ataxia community. The growing number of pharmas interested in Ataxia and the treatments that they are aggressively pursuing are a very hopeful sign for our families that have been waiting for this kind of news. Since our founding, NAF has funded millions of dollars of Ataxia research. The NAF Drug Development Collaborative represents the evolution of this consistent funding, and is an important new initiative for our organization. We thank these first six Collaborative members for their pioneering vision and trust in NAF as we start this work together."

The NAF-funded Clinical Research Consortium for Spinocerebellar Ataxia (CRC-SCA) will play a crucial role in the work of the Collaborative. Vikram Shakkottai, MD, PhD, speaking on behalf of the CRC-SCA co-chairs, Liana Rosenthal, MD, PhD, and Sheng Han Kuo, MD, PhD, said, "We are excited about how this collaboration can make much needed treatment a reality for individuals with Ataxia. The collaboration leverages the CRC-SCA's history of developing clinical measures in the Ataxia population with robust logistics and infrastructure from industry to initiate well designed clinical trials for this population."

NAF invites all pharmaceutical companies and supporting organizations to consider membership in the Collaborative. Inquiries can be directed to Mr. Rosen at andrew@ataxia.org.

About Ataxia: Ataxia is a degenerative disease of the nervous system that affects tens of thousands of people in the US and many more around the world. It can have a hereditary cause (inherited from a parent) or an acquired cause. Many symptoms of Ataxia mimic those of being drunk, such as slurred speech, stumbling, falling, and incoordination. These symptoms are caused by damage to the cerebellum, the part of the brain that is responsible for coordinating movement. People affected by Ataxia may experience problems with using their fingers and hands, arms, legs, walking, speaking or moving their eyes. Ataxia affects people of all ages. Age of symptom-onset can vary widely, from childhood to late-adulthood. Complications from the disease are serious and oftentimes debilitating. Some types of Ataxia can lead to an early death. There are currently no FDA-approved therapies to treat Ataxia.

About NAF: NAF is a nonprofit organization established in 1957 to help persons with Ataxia and their families. The Foundation's primary purpose is to support Ataxia research, provide vital programs and services for Ataxia families, and help in the search for a cure. NAF is the only organization in the United States dedicated to the disease that serves all types of Ataxia. NAF works closely with the world's leading Ataxia researchers, promoting exchanges of ideas and innovation in Ataxia discovery.

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To learn more, contact us or visit NAF's website at www.ataxia.org.