Continued inclusion of "Hereditary Ataxia" in the Peer Reviewed Medical Research Program (PRMRP), within the Department of Defense Congressionally Directed Medical Research Programs (CDMRP)

What is the CDMRP?

The CDMRP originated in 1992 via a Congressional appropriation to foster novel approaches to biomedical research in response to the expressed needs of its stakeholders – the American public, the military, and Congress. The CDMRP fills research gaps by funding high impact, high risk and high gain projects that other agencies may not venture to fund.

The PRMRP is one program within the CDMRP and supports research across the full range of science and medicine. All of the CDMRP programs share the common goal of advancing paradigm shifting research, solutions that will lead to cures or improvements in patient care, or breakthrough technologies and resources for clinical benefit. The CDMRP strives to transform healthcare for Service Members and the American public through innovative and impactful research. For more information, please visit https://cdmrp.army.mil/

What is Hereditary Ataxia?

The hereditary ataxias are a group of rare, genetic, degenerative, neurological conditions, affecting both children and adults. <u>There is one approved treatment to slow the progression of Friedreich's Ataxia, one of the 70+ types of hereditary ataxia, and no treatments to stop the progression of any type of ataxia.</u>

Hereditary ataxia is the clinical manifestation indicating the degeneration or dysfunction of the central nervous system which contributes to the coordination, precision and accurate timing of movements. Symptoms progress at different rates and include but are not limited to: lack of coordination of the upper and lower limbs, slurred speech, eye movement abnormalities, difficulty walking, tremors, trouble eating and swallowing, and death. Mobility and quality of life steadily decline requiring increasing assistance with all activities of daily living.

Hereditary ataxia impacts Americans throughout the country, at every stage and station of life. Thousands of military members are directly affected by hereditary ataxia, and many more are indirectly affected when a parent, sibling, spouse, or child is diagnosed. Hereditary ataxia results in a catastrophic effect on patients and their families physically, psychologically, and financially.

The Friedreich's Ataxia Research Alliance (FARA) and the National Ataxia Foundation (NAF), both 501(c)3 organizations, work to identify and fund research for treatments and ultimately a cure for hereditary ataxia. In 2023, FARA and NAF funded more than \$10M in research yet there are still unanswered questions that keep a treatment out of reach to patients. Better understanding of the underlying conditions that cause hereditary ataxia will not only lead to treatment for these conditions but could "unlock" new knowledge or treatments for other neurological conditions that share similar molecular pathways or consequences in other brain regions such as ALS, Huntington's Fragile X syndrome, Parkinson's, to name a few.

Supporting the continued inclusion of "Hereditary Ataxia" in the PRMRP within the CDMRP would provide the much-needed additional resources to expedite drug development and treat hereditary ataxia.

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