Summary:

The hereditary ataxias are a group of rare, genetic, degenerative, neurological conditions, affecting both children and adults. At this time there is no cure or any approved treatments to slow or stop progression. 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.

There are over 60 known genetic causes of hereditary ataxia, as well as patients whose genetic basis is still unknown. Hereditary ataxia is diagnosed using a combination of strategies that may include medical history, family history, and a complete neurological evaluation. Genetic testing is available for many types of hereditary ataxia. Medical intervention for hereditary ataxia is limited to addressing specific symptoms, rather than underlying cause, and may include speech and language therapy, occupational therapy, and physical therapy. Hereditary ataxia impacts Americans throughout the country, at every stage and station of life. Military members can be directly affected by hereditary ataxia or 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 National Ataxia Foundation (NAF) and the Friedreich's Ataxia Research Alliance (FARA), both 501(c)3 organizations, work to identify and fund research for treatments and ultimately a cure for hereditary ataxia. In 2020, NAF and FARA funded more than $7.2M 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. Adding hereditary ataxia to the Congressionally Directed Medical Research Programs (CDMRP) would provide the much-needed additional resources to expedite drug development and treat hereditary ataxia.

Proposed Language:
The Committee recommends $5,000,000 to be appropriated to the Department of Defense Congressionally Directed Medical Research Program for the study of treatments and a cure for Hereditary Ataxias.