



**STATEMENT OF  
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**ON BEHALF OF  
THE DYSTONIA ADVOCACY NETWORK**

**INNOVATION HEARING – OUTSIDE WITNESS HEARING**

**SUBMITTED TO  
THE SENATE COMMITTEE ON APPROPRIATIONS**

**APRIL 24, 2014**

Thank you for your examination of the importance of federal investments in research. The Dystonia Advocacy Network (DAN) strongly supports increased funding for the National Institutes of Health (NIH), the Department of Defense (DOD) Peer-Reviewed Medical Research Program, and the Veterans Medical and Prosthetic Research Program. These agencies fund promising research on dystonia that hold great hope for bettering the lives of dystonia patients, many of whom are unable to work because of their condition. The community is concerned that eroding budgets may limit the ability of competitive dystonia researchers to renew grants, slowing the progress of much needed innovation.

Dystonia is a neurological movement disorder characterized by involuntary muscle spasms that cause the body to twist, repetitively jerk, and sustain postural deformities. Focal dystonia affects specific parts of the body, while generalized dystonia affects multiple parts of the body at the same time. Some forms of dystonia are genetic but dystonia can also be caused by injury or illness. Although dystonia is a chronic and progressive disease, it does not impact cognition, intelligence, or shorten a person's life span. Conservative estimates indicate that between 300,000 and 500,000 individuals suffer from some form of dystonia in North America alone. Dystonia does not discriminate, affecting all demographic groups. There is no known cure for dystonia and treatment options remain limited. . Patients frequently rely on invasive therapies like botulinum toxin injections or deep brain stimulation (DBS) to help manage their symptoms.

## **National Institutes of Health**

NIH is a critical source of funding for the dystonia community and other communities like it. Currently, dystonia research supported by NIH is conducted through the National Institute of Neurological Disorders and Stroke (NINDS), the National Institute on Deafness and Other Communication Disorders (NIDCD), the National Eye Institute (NEI), and the Office of Rare Diseases Research (ORDR) within the National Center for Advancing Translational Sciences (NCATS).

The majority of dystonia research at NIH is conducted through NINDS. NINDS has utilized a number of funding mechanisms in recent years to study the causes and mechanisms of dystonia. These grants cover a wide range of research including the genetics and genomics of dystonia, the development of animal models of primary and secondary dystonia, molecular and cellular studies in inherited forms of dystonia, epidemiology studies, and brain imaging.

Within NCATS, ORDR coordinates the Rare Disease Clinical Research Network (RDCRN) which provides support for studies on the natural history, epidemiology, diagnosis, and treatment of rare diseases. RDCRN includes the *Dystonia Coalition*, a partnership between researchers, patients, and patient advocacy groups to advance the pace of clinical research on cervical dystonia, blepharospasm, spasmodic dysphonia, craniofacial dystonia, and limb dystonia. The *Dystonia Coalition* has made tremendous progress in preparing the patient community for clinical trials as well as funding promising studies that hold great hope for advancing our understanding and capacity to treat primary focal dystonias.

NIDCD and NEI also support research on dystonia. NIDCD has funded many studies on brainstem systems and their role in spasmodic dysphonia, or laryngeal dystonia. Spasmodic dysphonia is a form of focal dystonia which involves involuntary spasms of the vocal cords causing interruptions of speech and affecting voice quality. NEI focuses some of its resources on the study of blepharospasm. Blepharospasm is an abnormal, involuntary blinking of the eyelids which can render a patient legally blind due to a patient's inability to open their eyelids.

## **Defense Peer-Reviewed Medical Research Program**

Dystonia can be hereditary or caused by trauma such as a car crash or an IED blast exposure as experienced by military personnel. The DOD Peer-Reviewed Medical Research Program is the most essential program studying dystonia in military and veteran populations. This program is critical to developing a better understanding of the mechanisms connecting trauma and dystonia. Dystonia researchers have competed successfully within the peer-reviewed system every year they have been eligible, which underscores the relevant and important nature of their work.

## **Veterans Medical and Prosthetic Research Program**

The DAN is increasingly becoming aware of the impact of dystonia on veterans. In many cases, the onset of dystonia is associated with traumatic brain injury or periphery trauma. Dystonia has also been associated with post-traumatic stress disorder. As our veterans age, some begin to notice dystonic symptoms which over time develop into dystonias. Currently, the Department of Veterans Affairs is supporting one project on dystonia. The DAN encourages

Congress and the VA to expand research on the above-mentioned mechanisms that lead to dystonia after trauma.

### **The Dystonia Advocacy Network**

The Dystonia Medical Research Foundation (DMRF) submits these comments on behalf of the Dystonia Advocacy Network (DAN), a collaborative network of five patient organizations: the Benign Essential Blepharospasm Research Foundation, the Dystonia Medical Research Foundation, the National Spasmodic Dysphonia Association, the National Spasmodic Torticollis Association, and ST/Dystonia, Inc. DAN advocates for all persons affected by dystonia and supports a legislative agenda that meets the needs of the dystonia community.

DMRF was founded over 33 years ago. Since its inception, the goals of DMRF have remained to advance research for more effective treatments of dystonia and ultimately find a cure; to promote awareness and education; and support the needs and well being of affected individuals and their families.

Thank you for the opportunity to present the views of the dystonia community. If the DAN can provide additional information, please contact the DAN Washington Representative, Sarah Buchanan, at (202) 544-7499 or [sbuchanan@hmcw.org](mailto:sbuchanan@hmcw.org).