

Dystonia Coalition

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Research Areas



Tool Development

Resource



Biomarker Research

Diagnostic

At a Glance

- Status: **Active Consortium**
- Year Launched: **2009**
- Initiating Organization: **NIH Rare Disease Clinical Research Network**
- Initiator Type: **Government**
- Location: **International**

Abstract

The Dystonia Coalition is a collaboration of medical researchers and patient advocacy groups supported by the Office of Rare Diseases Research in the National Center for Advancing Translational Sciences and the National Institute of Neurological Disorders and Stroke at the National Institutes of Health (NIH).

Mission

The Dystonia Coalition's mission is to advance the pace of clinical and translational research in the dystonias to find better treatments and a cure. Its objectives are to develop a fuller understanding of the many different features of dystonia and how they change over the years, develop validated diagnostic strategies and rating tools for diagnosis and monitoring patients in clinical trials, establish a biorepository where samples of blood and other materials can be stored and distributed for research, catalyze clinical trials for promising new treatments, and promote education and awareness.

The Dystonia Coalition will focus initially on the primary focal dystonias including cervical dystonia, spasmodic dysphonia, blepharospasm and craniofacial dystonia, and limb dystonias. Four main

studies are planned, each involving several academic centers.

Natural History Study of Primary Dystonia. The goal of this study is to form a fuller understanding of how the different forms of dystonia may change over time. Patients with any of the primary focal dystonias will be evaluated carefully at yearly intervals to reveal any changes that may occur over time. A blood sample for the Biorepository for Primary Dystonia will be collected from subjects in this Natural History study at the outset for genetic and other studies.

Biorepository for Primary Dystonia. The goal of this study is to build a large collection of deoxyribonucleic acid (DNA) samples for genetic studies of patients with primary focal dystonia.

Comprehensive Evaluation Tools for Cervical Dystonia. The goal of this study is to refine a clinical rating scale for the many different aspects of cervical dystonia. The new scale will address the movement disorder, pain, psychological correlates, functional disability, and overall quality of life. This scale is intended to be useful for monitoring progression and responses to treatments in clinical trials.

Comprehensive Evaluation Tools for Spasmodic Dysphonia. The goal of this study is to develop and test a novel rating scale that can be used to diagnose the disorder and measure its severity. This scale is intended to help physicians more quickly and accurately diagnose spasmodic dysphonia and to help monitor responses to treatments in clinical trials.

In addition to the four large studies outlined above, the Dystonia Coalition will encourage the development of new studies and investigators relevant to dystonia, promote education and awareness of dystonia, and assist patients in finding state-of-the-art information and expert providers.

Pilot Projects Program. The goal of this program is to foster the most promising clinical and translational studies relevant to primary dystonia. Such studies may include development of clinical rating scales where they do not yet exist, exploration of biomarkers that could be used for diagnosis or as a surrogate for monitoring the disease, pilot clinical trials of promising new treatments, development of unique shared resources for dystonia research, and other projects. Applications will be accepted twice yearly, with anticipated amounts of \$10,000-\$50,000. Priority will be given to clinical studies rather than basic science. Emphasis will be placed on studies that have high impact, provide pilot data essential for larger studies, and encourage collaboration among different centers.

Career Development Award. The primary goal of this program is to facilitate career development for junior investigators interested in clinical and translational research relating to dystonia. A secondary

goal is to encourage more senior investigators from other fields to get more involved in dystonia research. Applications will be accepted up to twice yearly, and each award will be \$50,000.

Promoting Education and Awareness. The coalition's centers are active in promoting education and awareness among colleagues and other medical providers, and in the community. It regularly helps to organize and participate in regional and international meetings.

Assist in Connecting Patients with Expert Providers. Although its primary mission is research, the coalition also wants to assist patients and their families in connecting with the best medical providers. Those in need may contact any one of the coalition's sites directly. Its patient advocacy groups can also facilitate finding skilled medical providers.

Consortium History

October 2009: Consortium announced.

Structure & Governance

This consortium is part of the Rare Diseases Clinical Research Network (RDCRN), an initiative of the Office of Rare Diseases Research, National Center for Advancing Translational Sciences (NCATS). The RDCRN is designed to advance medical research on rare diseases by providing support for clinical studies and facilitating collaboration, study enrollment, and data sharing. Through the RDCRN consortia, physician scientists and their multidisciplinary teams work together with patient advocacy groups to study more than 200 rare diseases at sites across the nation.

Funding and scientific oversight for the RDCRN are provided by NCATS and 10 NIH components: Eunice Kennedy Shriver National Institute of Child Health and Human Development; National Cancer Institute; National Heart, Lung and Blood Institute; National Institute of Allergy and Infectious Diseases; National Institute of Arthritis and Musculoskeletal and Skin Diseases; National Institute of Dental and Craniofacial Research; the National Institute of Diabetes and Digestive and Kidney Diseases; National Institute of Mental Health; National Institute of Neurological Disorders and Stroke; and Office of the Director. In addition, patient advocacy groups provide funds for many of the projects.

Patent Engagement

Integral to the coalition's mission is its patient advocacy groups, including Action for Dystonia, Diagnosis, Education and Research, The American Dystonia Society, The Bachmann-Strauss Dystonia and Parkinson's Foundation, Beat Dystonia, The Benign Essential Blepharospasm Research Foundation, DySTonia, Dystonia Ireland, The Dystonia Medical Research Foundation, The Dystonia Medical Research Foundation-Canada, The Dystonia Society, Dystonie-Québec, The European Dystonia Foundation, The National Spasmodic Dysphonia Association, The National Spasmodic Torticollis Association, Tyler's Hope, and We Move. Each of its patient advocacy groups is encouraged to be involved in the yearly meeting of the Dystonia Coalition, participate in the Pilot Projects Program or Career Development Program, and refer patients to its centers for participation in studies or expert treatment, and help keep patients and providers well informed.

The primary goal of the coalition is to conduct research relating to dystonia. The academic centers in the coalition all have a special interest in dystonia research, as well as expertise in its diagnosis and treatment of all forms of dystonia. It therefore expect many patients to go to these centers for both research opportunities and expert clinical care.

Links/Social Media Feed

Homepage

<http://www.rarediseasesnetwork.org/dystonia/>

Points of Contact

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