

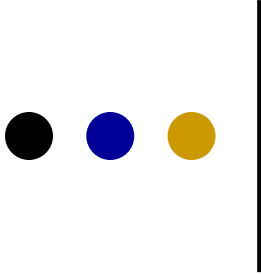


# **Patient Advocacy Groups**

## ***Updates and Future Plans***

**Janet L. Hieshetter**  
***DMRF Administrative***  
***Unit Representative***

**Kimberly Kuman**  
***Patient Advocacy***  
***Group Liaison***



# Current DC Patient Advocacy Groups (PAGs)

- Benign Essential Blepharospasm Research Foundation\*
- Cure Dystonia Now
- Dystonia Europe
- Dystonia Ireland
- Dystonia Medical Research Foundation\*
- Dystonia Medical Research Foundation Canada
- Dystonia Society
- National Spasmodic Dysphonia Association\*
- National Spasmodic Torticollis Association\*
- Tyler's Hope

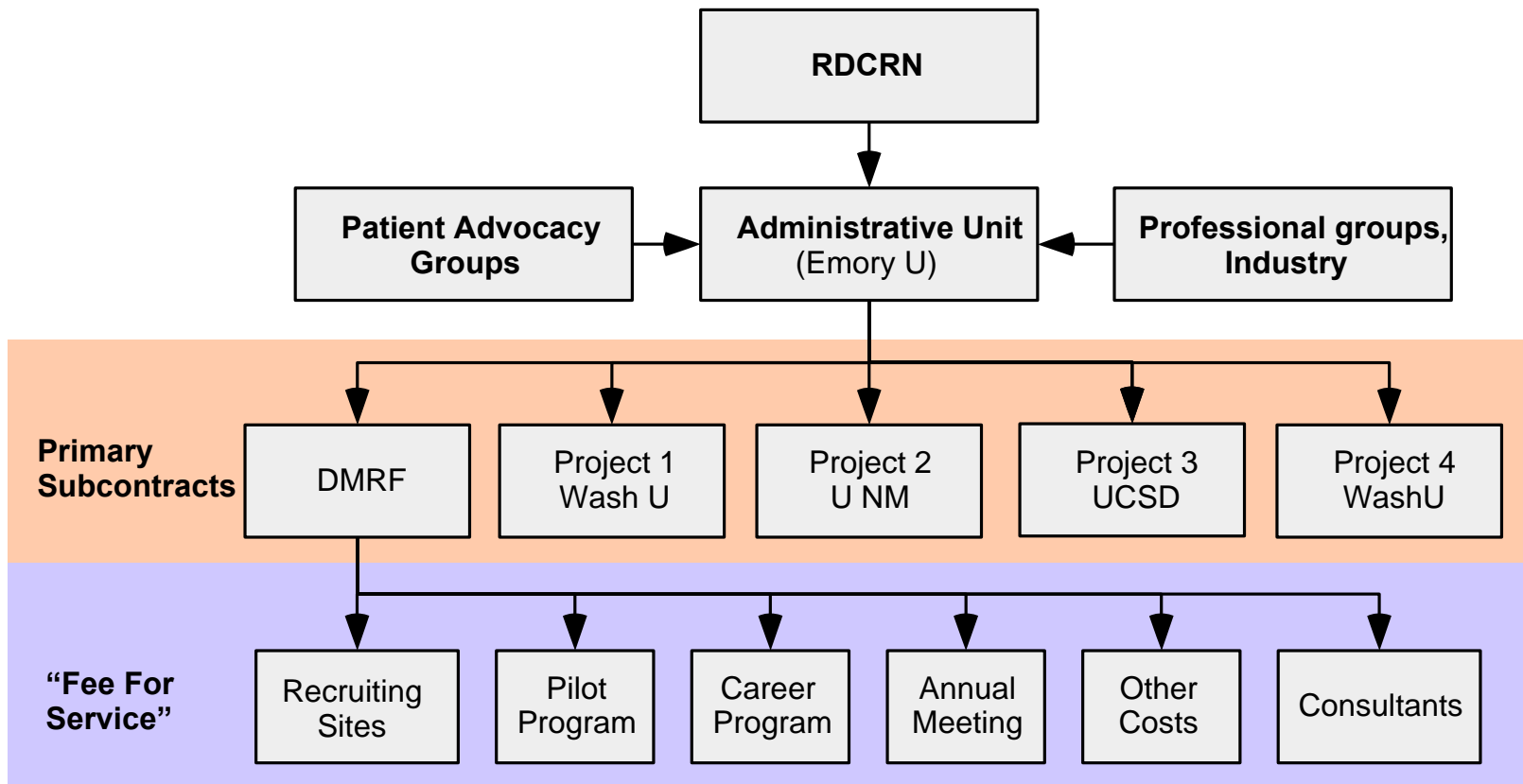
*\*Original PAG Groups with initial application*



# Administrative Function of the DMRF

- Unique model with a PAG in this role
- Manage 40+ centers
- Paying grants and fees = stretches grant funds
- Organize & manage meetings/webinars

# Administrative Function of the DMRF





# Additional Responsibilities of the DMRF

- Participate in review of PPP/CDA grants
- Manage community funds
- Represent the DC on the CPAG Steering Committee



# Key Areas of Collaboration

- Organizational and administrative activities
- Opportunities for co-funding projects
- Innovative approaches to patient registries



# PAG Project and Grant Support

## Main Clinical Projects

## Pilot Project Awards

*Purpose is to serve as seed funding to foster promising ideas into more competitive research project*

## Career Development Awards

*Purpose is to help junior faculty start and develop careers, or senior faculty to reorient careers, in dystonia research*

## Travel Scholarships for Junior Investigators

*Purpose is to support junior faculty to attend annual Dystonia Coalition meetings*



## Internet-based registry

- Owned and funded by PAGs
- Voluntary and compliant with all privacy laws
- Data fields compatible with DMCC Registry

## Access and use governed by

- PAG Committee  
Current: BEBRF, CDN DMRF, DMRFC, NSDA
- Scientific Committee





### Help us learn more about dystonia!

By completing your profile, your information will be utilized to move forward research and clinical trials that could lead to future treatments and cures for dystonia!

#### Newsflash

##### Understanding Dystonia

Dystonia is a neurological movement disorder that causes muscles to contract and spasm involuntarily.

##### Research studies through the Dystonia Coalition

The Dystonia Coalition is a cooperative project between medical researchers and patient advocacy groups. Its mission is to advance the pace of clinical and translational research in the dystonias to find better treatments and a cure.

#### Register Now!

Welcome to the Global Dystonia Registry! The goal of the Global Dystonia Registry is to support future dystonia studies, including clinical and research trials, through the collection of data on persons affected by dystonia. Although the focal dystonias have many different manifestations, most experts believe they share a common pathogenesis or mechanism that causes the disorder. The common causes may be a similar gene defect, similar lifetime experiences, or both. Collecting information from different patient populations may help us identify the common features that they may share. This Registry compliments the current scope of research for the Dystonia Coalition, a National Institutes of Health supported clinical research effort.

Your responses will help us better understand the dystonic experience and help guide future directions in research. Please know your responses will remain confidential. Your participation is completely voluntary and the parameters of each study will be defined for you should you decide to be involved. We hope you will register today!

#### Sponsoring Patient Organizations

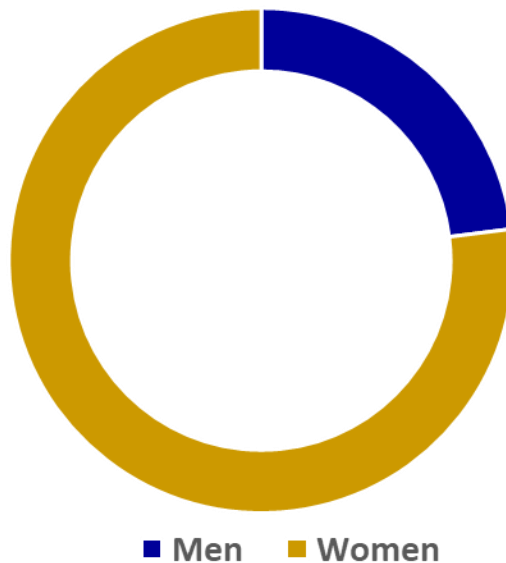




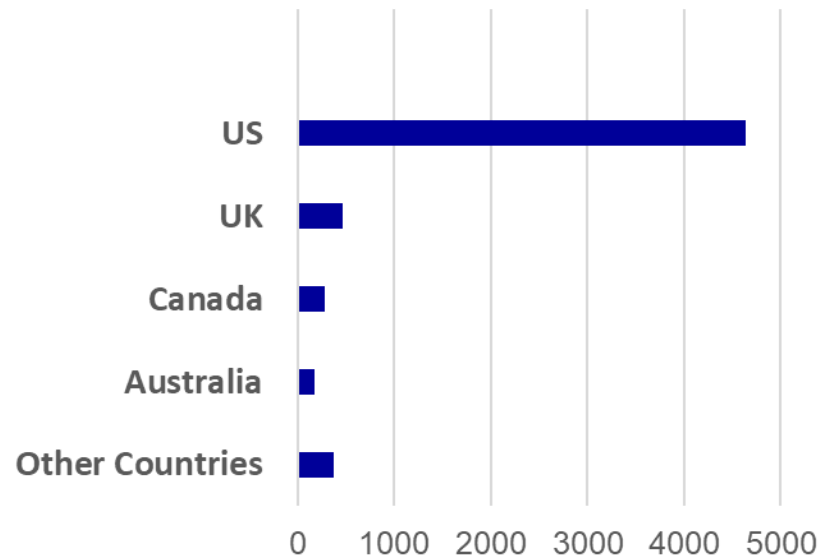
# The Global Dystonia Registry

- Launched December 2011 with currently close to 6,000 people registered
- 90 countries represented with highest percentages in US, UK, Canada, and Australia

Gender



By Country





# Continued PAG Collaboration

- Distribution of Recruitment Information on various DC Projects to Patients
- E-mail and Conference Call Updates
- Attendance at Annual Meetings
- Participation in Executive Committee Calls
- Advertisement of Grant Programs and DC Meetings to research contacts
- Represented on the Dystonia Coalition Website  
<https://www.rarediseasesnetwork.org/cms/dystonia>
- Scientific Representation from PAGs on Grant Review Committees

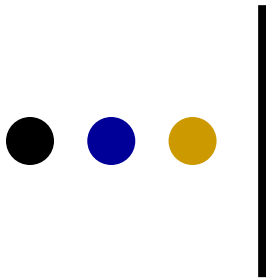


# Patient Centered Outcomes Project

**PI: Dr. Sarah Piro-Richardson**

PAG Collaboration on Focus Groups

- Cervical Dystonia: Monica Benson and Janet Hieshetter
- Blepharospasm: Nilda Rendino
- Laryngeal: Kimberly Kuman



# **DYSTONIA COALITION**

Benign Essential Blepharospasm  
Research Foundation



**DYSTONIA  
EUROPE**



**DYSTONIA  
MEDICAL  
RESEARCH  
FOUNDATION**

*serving all dystonia-affected persons*

**DYSTONIA  
MEDICAL  
RESEARCH  
FOUNDATION  
CANADA**  **FONDATION DE  
RECHERCHE  
MÉDICALE SUR LA  
DYSTONIE  
CANADA**

*serving all dystonia-affected people  
d'asservant toutes personnes atteintes de dystonie*


The  
**dystonia**  
society

 **NSDA**  
NATIONAL SPASMODIC DYSPHONIA ASSOCIATION

 **NSTA**  
NATIONAL SPASMODIC TORTICOLLIS ASSOCIATION

 **Tyler's Hope**  
for a Dystonia Cure

 **Office of  
Rare Diseases  
Research**  
National Center for Advancing  
Translational Sciences – NIH

 **RARE  
CLINICAL  
RESEARCH  
NETWORK**  
Initiative of the National Center for Advancing  
Translational Sciences (NCATS)

 **OPAG**  
Coalition of Patient  
Advocacy Groups

 **NATIONAL INSTITUTE OF  
NEUROLOGICAL  
DISORDERS AND STROKE**