Patient Advocacy Groups

Updates and Future Plans

Kimberly Kuman
Patient Advocacy Group Liaison (NSDA)

Janet L. Hieshetter
DMRF Administrative Unit Representative
Current DC Patient Advocacy Groups (PAGs)

- Beat Dystonia
- 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
Key Areas of Collaboration

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

Scientific Representation from PAGs on Grant Review Committees
Patient-Centered Outcomes Project

PI: Dr. Sarah Piro Richardson

PAG Collaboration on Focus Groups
- Cervical Dystonia: Janet Hieshetter
- Blepharospasm: Heidi Hcoggeshal and Charlene Hudgins
- Laryngeal Dystonia: Kimberly Kuman

All focus groups have been completed.

Next steps is the online survey to the broader PAG community
Internet-based Patient-Reported Registry

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

Access and Use Governance

- PAG Committee | Current: Beat Dystonia, BEBRF, CDN, DMRF, DMRFC, NSDA
- Scientific Committee
- Application for access of data
www.globaldystoniaregistry.org
Launched December 2011 with currently over 6,100 people registered

90 countries represented with highest percentages in US, UK, Canada, and Australia
Administrative Function of the DMRF

- **Primary Subcontracts**
  - DMRF
  - Project 1: Wash U
  - Project 2: U NM
  - Project 3: UCSD
  - Project 4: WashU

- **"Fee For Service"**
  - Recruiting Sites
  - Pilot Program
  - Career Program
  - Annual Meeting
  - Other Costs
  - Consultants

- **RDCRN**
- **Patient Advocacy Groups**
- **Administrative Unit (Emory U)**
- **Professional groups, Industry**
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
Additional Responsibilities of the DMRF

- Participate in review of PPP/CDA grants
- Manage community funds
- Represent the DC on the CPAG Steering Committee
- Support for PAG Webinar | June 8, 2021