Patient Advocacy Groups

Updates and Future Plans

Janet L. Hieshetter
DMRF Administrative Unit Representative

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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

- RDCRN
- Administrative Unit (Emory U)
- Patient Advocacy Groups
- Professional groups, Industry

Primary Subcontracts:
- DMRF
- Project 1 (Wash U)
- Project 2 (UNM)
- Project 3 (UCSD)
- Project 4 (WashU)

“Fee For Service”:
- Recruiting Sites
- Pilot Program
- Career Program
- Annual Meeting
- Other Costs
- Consultants
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
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
• Launched December 2011 with currently close to 6,000 people registered
• 90 countries represented with highest percentages in US, UK, Canada, and Australia
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