



**2016**

## **Operating Priorities**

From the beginning the goals of the organization were simple, and endure to this day: To advance research for more effective treatments and ultimately a cure, to promote awareness and education, and to support the well-being of dystonia-affected individuals and their families.

The DMRF will focus on the following priorities in 2016 in support of the Foundation's mission.

## **RESEARCH**

To achieve its ultimate mission of finding a cure for all forms of dystonia, the DMRF will support scientific investigations through its Research Programs. In doing so, DMRF will: attract new investigators in the field, support innovative research that will lead to better understanding of the mechanisms of the dystonias and identification and characterization of therapeutic targets; facilitate the development of more effective agents and devices for the dystonias in the next decade.

**RES Objective 1: Identify, stimulate, support and monitor cutting-edge research aimed at expanding the knowledge of the genetic, molecular, physiologic and pathologic basis of dystonia.**

Action Steps:

1. MSAC and recognized outside experts invited to annual meeting to review recent research progress and provide recommendations to Science Committee/Board of Directors for pursuing the most critical areas of research and new directions;

2. Develop and release an RFA for the Myoclonus Dystonia Research Program based on input from the M-D meeting, review responses and identify proposals for funding. Once approved, monitor these approved grants;
3. Assess the first year of the Mahlon R. DeLong Young Investigator Award (MDYIA), revise criteria as recommended, develop and release an RFA for the next MDYIA;
4. Develop criteria for how the DMRF will review requests for support of innovative research ideas that fall outside the scope of any 2016 RFAs;
5. Identify the area of research the DMRF will explore in 2016 and then develop and release an RFA. Utilizing the Letter of Intent system, review the letters, determine those to be invited to submit a full application, review and once approved, monitor these grants;
6. Develop criteria for pre-doctoral research training award and release a call for applications by July 1, 2016;
7. Continue to work with the NINDS staff to identify approaches and strategies to promote and support dystonia research;
8. Provide funding support for the Dystonia Coalition's Career Development Award (CDA), with DMRF Chief Scientific Officer to serve as a reviewer for the CDA Program;
9. DMRF Chief Scientific Officer to represent DMRFC whenever scientific assessment, advice or reviews are needed; and
10. Per Interagency Agreement, provide quarterly research updates to DMRFC.

**RES Objective 2: Establish contracts with academic researchers or pharma/biotech companies to conduct specific translational research or development projects with the immediate goal to discover and validate druggable targets.**

Action Steps:

1. Continuously assess opportunities for new research contracts with translational or clinical potential that will be presented to the Science Committee for consideration;
2. Monitor progress of ongoing contracts with regard to agreed milestones; review reports, provide feedback, hold meetings, if necessary; and
3. Determine the best way to publish the data from Project FireSky.

**RES Objective 3: Support collaboration, resources and mechanisms to share information to promote and advance worldwide dystonia research.**

Action Steps:

1. Promote to the research community funding and collaboration programs and initiatives at DMRF, Dystonia Coalition, NIH and other dystonia research funding sources;
2. Continue implementing brain banking plan for increased donor registrations and investigational use of tissue samples and continue to work with the Dystonia Brain Collective to engage other dystonia patient organizations to support the operation of the private dystonia collection at the Harvard Brain Tissue Resource Center;
3. Provide administrative support for the Global Dystonia Registry;
4. Promote the enrollment in patient registries as a resource for the research community;
5. Maintain communication and explore collaborative opportunities with European dystonia organizations and research groups;
6. Provide current updates and report on research news on the DMRF website and in other DMRF publications;
7. Develop a web platform that will promote the availability of research resources such as brain tissue, cell lines, etc.;
8. Explore potential dates and locations for the next international meeting (IDS6).

**RES Object 4: Interact with the pharmaceutical/biotech companies and other potential partners in the research and development of dystonia therapies.**

Action Steps:

1. Continue to identify, engage and interact with pharmaceutical and medical devices companies that have potential or expressed interest in dystonia therapeutics development, present dystonia as an opportunity;
2. Attend the American Academy of Neurology and Society for Neuroscience Annual Meeting, the Movement Disorders Society Annual Meeting and other meetings to introduce dystonia and DMRF to potential commercial partners;
3. Work in partnership with the Dystonia Study Group to support their work as needed;
4. Collaborate with industry partners to advance the development of their drug candidates for dystonia, including but limited to assisting with the trial design, identification of KOLs

for consultation and possible participation, and assist with the regulatory path and detailed scientific rationale for the indication, as needed.

**RES Objective 5: Serve as the administrative center for the Dystonia Coalition, a clinical research network (collaboration of medical researchers and patient advocacy groups) sponsored by the NINDS and the National Center for Advancing Translational Sciences (NCATS) to advance the pace of clinical and translational research in the dystonias to find**

Action Steps:

1. Work with Emory University to finalize the subcontract agreement between Emory and the DMRF for the Foundation's role as administrative center for the Dystonia Coalition;
2. Develop and monitor subcontract agreements with sites participating in Dystonia Coalition Main Clinical Projects;
3. Provide payments to sites contributing to the Dystonia Coalition in proportion to their recruitment efforts for various studies being conducted;
4. Provide logistical and planning support for the Dystonia Coalition's annual meeting, including payment of all approved expenses;
5. Provide payments to international consultants involved with the Coalition;
6. Disburse award payments to recipients of the Coalition's Career Development and Pilot Project Awards Programs;
7. Manage the funds received from Emory to pay out project related expenses outlined above;
8. DMRF Chief Scientific Officer to serve as a member of the Career Development and Pilot Project Award Review Committees;
9. Manage the private funds contributed by other dystonia patient organizations to support key Coalition projects/activities;
10. Provide access to DMRF's conference call system to hold conference calls as needed;
11. Participate in monthly Executive Committee calls and other administrative calls to ensure smooth operations of all Coalition activities;
12. DMRF Executive Director to serve as RDCRN Coalition of Patient Advocacy Groups co-chair.

**RES Objective 6: Bring key thought leaders together from various areas of expertise, in and outside of the dystonia community, to promote dystonia research, address current scientific/therapeutic issues, facilitate information sharing, network building or identification of critical research areas and future directions.**

Action Steps:

1. Executive Director to serve on NINDS Council and attend all meetings;
2. Hold think tank like meeting of MSAC to address key areas of dystonia research and suggest 'hot' topics for an RFA;
3. Organize a Myoclonus-Dystonia Meeting for February 2016 to identify critical gaps that an RFA will address;
4. Attend/provide support for other dystonia-related meetings, when appropriate;
5. Organize a Clinical Fellowship dinner meeting in conjunction with AAN Annual meeting in Vancouver, BC in April 2016;
6. Assess readiness for 6<sup>th</sup> International Dystonia Symposium.

**RES Objective 7: Improve the clinical treatment for those affected by dystonia through the implementation of a clinical fellowship program to facilitate the training of clinicians and young investigators in dystonia research.**

Action Steps:

1. Secure financial support for the DMRF Clinical Fellowship Program;
2. Work with the Clinical Fellowship Review Committee to oversee the program's objectives, application procedures and then evaluate 2015 program – when the RFA should be released, deadlines, etc.;
3. Promote the program within the movement disorders clinical community;
4. Make site visits to fellows as travels/schedules allow.

## AWARENESS & EDUCATION

The DMRF supports awareness and education programs and activities to decrease the number of misdiagnosed persons, improve the quality of life of affected persons and support the Foundation's priorities. In 2016, the DMRF will build on the momentum of past efforts to increase awareness and understanding of dystonia and of the Foundation

with the general public, the healthcare community and affected persons and their families.

**A&E Objective 1: Keep the community apprised of relevant, accurate dystonia and Foundation news through the production of three issues of The Dystonia Dialogue, scientific updates through the Promise & Progress and the 2015 Annual Report.**

Action Steps:

1. Working with the editorial board, identify key topics for each issue of the Dialogue;
2. Develop, implement and comply with a 2016 Dialogue production schedule that complements the DMRF's development efforts with issues distributed February 29, June 27 and October 31;
3. Place on-line versions of stories and publications following each printing of the Dialogue;
4. Working with program staff to develop copy for the 2015 Annual Report to be available by May 2, 2016;
5. Working with the development team, develop a plan to promote network events, materials and provide support for post-event awareness;
6. Working with science team, develop a lay summary of DMRF research efforts for the Promise & Progress report to be distributed by March 28, 2016.

**A&E Objective 2: Utilizing electronic platforms to promote accurate educational resources and DMRF priorities.**

Action Steps:

1. Update the website on an as needed basis to keep content current and easily accessible;
2. Post webinar recordings to extend educational outreach;
3. Explore possible partners to increase outreach to the dystonia community to increase access to accurate dystonia information;
4. Evaluate all efforts and report regularly to the Board of Directors.

**A&E Objective 3: Make educational programs accessible by organizing community forums, web-based educational forums and organize a national educational symposium.**

Action Steps:

1. Identify at least 4 markets to develop and implement plans for community forums and evaluate each session;
2. Identify persons to help develop and implement the national family symposium in 2016;
3. Identify topics for 2-3 webinars (topics to be determined), promote availability of sessions, sessions to be held in March, September and November;
4. Work with support groups to assist in their educational programming. Attend local support group or other educational meetings when schedules permit;

**A & E Objective 4: Promote dystonia awareness by engaging the DMRF community and the news media.**

Action Steps:

1. Working with the Awareness & Education Committee, explore how the internet and social networks can be better utilized to raise dystonia awareness;
2. Assess the need for a policy to guide management of these networks – nationally and each DMRF support group operated site.
3. Maintain a pipeline for regular production and distribution of coordinated communications on DMRF events and news, including press releases, website announcements, texts, and use of social media;
4. Conduct “Dystonia Moves Me” campaign to take place in September 2016 during Dystonia Awareness Month. Announcement to support network and community in March 2016.

**A & E Objective 5: Maintain and promote scientifically and organizationally accurate resource materials that meet the needs of the community.**

Action Steps:

1. Review of resource materials, print and electronic (video/audio);
2. Develop and implement a schedule for revising the identified materials;
3. Identify three educational resources to translate into Spanish and develop a plan for promoting these and responding to inquiries by August 1, 2016;
4. Coordinate the dissemination of dystonia news through e-news; social networking sites, website, announcements and press releases (when relevant).

# SUPPORT

The DMRF will improve the quality of life for dystonia-affected persons and their families through support programs and services.

**S Objective 1: Provide support resources for all affected persons/families.**

Action Steps:

1. Through the Support Committee, assess and monitor support needs and the programs designed to address these needs;
2. Conduct survey to all support Leaders to help identify needed resources and programs.
3. Implement and assess an on-line module for the newly diagnosed by May 1<sup>st</sup>, 2016;
4. As appropriate, share the program successes of other support groups with all leaders;
5. Explore the new technologies to make efficient and extend the reach of support resources; and
6. Ensure all electronic and written materials are medically accurate and presented in an appropriate manner/level for understanding.

**S Objective 2: Ensure that those affected by dystonia have accurate information to make good treatment decisions and to know that they are not alone through availability of local resources such as support groups, regional contacts, physician referral and the DMRF Information &Referral line, etc.**

Action Steps:

1. Work with existing support leaders and on-line moderators in their efforts to assist individuals/families in obtaining accurate dystonia information and coping/sharing opportunities;
2. Identify new support group leaders and provide the resources required for their success in becoming a local resource for those affected by dystonia and their families;
3. Review and revise job descriptions for area and regional contacts and confirm appointments to these positions by May 1, 2016;
4. By July 1<sup>st</sup> recruit for the newly approved position, Manager of Community Engagement, to support current leaders, develop and implement a plan for outreach to key areas to develop groups, support educational forums, and support local volunteers in their community activities;

5. Work with all network leaders to ensure they have what is needed to provide support and education to their respective communities;
6. Encourage leaders to have their local movement disorder neurologists join the DMRF physician referral list or if currently participating, to verify information and identify other movement disorder neurologists to join referral list;
7. Enlist patients to act as DMRF representatives to bring DMRF materials to high priority movement disorder clinics for their waiting rooms so patients have access to accurate, balanced educational materials and are aware of the DMRF as a resource for support and information;
8. Respond to inquiries/requests for information;
9. Support the work of regional and area contacts;
10. Promote new listings while confirming the contact information of existing listings on physician referral list.

**S Objective 3: Support educational and networking opportunities for affected persons/families.**

Action Steps:

1. Develop and implement a plan to develop support groups in key metropolitan areas;
2. In conjunction with our awareness & education efforts, host webinars – topics to be determined that will provide important information to identified audiences;
3. Maintain and monitor subject bulletin boards to ensure accurate information is being disseminated to ensure effectiveness of the resource;
4. Support and monitor social networking and encourage participation in these electronic networks.

## ADVOCACY

The DMRF will work to improve the lives of those affected by dystonia through an increased awareness of elected officials of dystonia and the policy needs of the dystonia community.

Ad Objective 1: Develop and implement a legislative agenda that meets the needs of the dystonia community.

Action Steps:

1. Develop 2016 legislative agenda in consultation with Health & Medicine Counsel of Washington (HMCW) and the other Dystonia Advocacy Network (DAN) member organizations, by February 10, 2016 and monitor progress throughout the year;
2. Develop and implement plans for Advocacy Day, April 12-13, in Washington, DC;
3. Recruit advocates to participate in Advocacy Day from key legislative districts as identified by HMCW and distributed to DAN member organizations by February 12, 2016;
4. Announce the Douglas Kramer Young Advocate award applications by February 12, 2016 and evaluate applications and determine awardee by March 18;
5. Maintain a legislatively up-to-date DAN website that also allows for advocate recruitment;
6. Develop and implement a communication plan for dystonia advocates;
7. Assess the need for State legislative programs to address patient access concerns as needed in 2016;
8. Partner with other organizations, as identified by issues, support mutual legislative efforts.

Ad Objective 2: Maintain listing of dystonia on the Congressionally Directed Medical Research Program managed by the Department of Defense.

Action Steps:

1. Identify Congressional champions for DoD funding and a strategy to engage them;
2. Include this issue on the DAN legislative agenda;
3. Identify veterans who are willing to work on this issue and assist efforts;
4. Working with HMCW team develop a program for visits in key Senate offices during the August recess by advocates on this topic;
5. Implement plans for a letter writing campaign on DoD dystonia research funding;
6. Identify a person(s) willing to serve as consumer reviewers for the DOD application review panel and complete application(s).

Ad Objective 3: Ensure access to all FDA approved treatments for dystonia.

#### Action Steps:

1. Identify key States and develop a plan to educate the major carriers and Medicare/Medicaid in those States regarding dystonia and its treatments, including but not limited to working with State Insurance Commissioners, Medicare Regional Medical Directors and others who might be helpful with this issue;
2. Look for opportunities to partner with the clinical and pharmaceutical communities on implementing the plan;
3. Investigate the benefits for dystonia treatment offered through the State exchanges and develop a plan to address those that may present challenges for treatment;
4. Provide information to the dystonia patient community on the pharma sponsored patient assistance programs.

## ORGANIZATIONAL CAPACITY

The DMRF will work to ensure organizational integrity and efficiencies and organizational effectiveness.

**Org Objective 1: Ensure the Foundation's operations are compliant with all applicable Federal, State and local laws and approved DMRF policies.**

#### Action Steps:

1. Working with Audit Committee and outside financial consultant, ensure compliance with I.R.S. and Foundation fiscal and personnel policies and procedures;
2. Conduct 2015 audit and prepare for preliminary 2016 audit utilizing outside auditing services and conduct agreed upon procedures on Dystonia Coalition accounts;
3. Appoint a Tax Review Task Force to review 2015 taxes prior to filing;
4. Provide appropriate information to charitable rating organizations and monitor ratings;
5. Research, develop and implement a schedule for compliance with State and local charitable filing requirements;
6. Require completion of 2016 Board of Directors Code of Conduct Declaration Forms;
7. Conduct annual performance reviews on all DMRF staff;
8. Assess need for and then retain, as needed, insurance for DMRF sponsored events;
9. Utilizing a bidding process, work to ensure all expenses are contained.

**Org Objective 2: Strengthen and expand the Foundation's Board of Directors.**

Action Steps:

1. Working with the Nominating Committee identify individuals who will enhance the Foundation's capacity through service on the board or other organizational structures;
2. Elect members of the Audit Committee at annual meeting of Board of Directors;
3. Provide an orientation to new Board Members;
4. Identify individuals for service on Foundation's committees;
5. Ensure all Directors are apprised of Foundation activities, achievements and risks through regular communications from the Executive Director, and others as needed.

**Org Objective 3: Ensure efficient Foundation operations.**

Action Steps:

1. Develop and submit operating budget for approval by Board of Directors, monitor operating budget to expenses monthly and issue monthly financial statements with a report on budget variances;
2. Ensure all Directors are apprised of Foundation financial status and risks through regular communications from the Executive Director, and reports from the Treasurer at each Board of Directors meetings;
3. Explore all cost savings opportunities through the bidding process and comparative purchasing for office equipment, supplies and services;
4. Develop and manage by individual staff working plans.
5. Manage the subtenant relationship;
6. Work with the broker to explore future office space options.