



DYSTONIA  
MEDICAL  
RESEARCH  
FOUNDATION

erving all dystonia-affected persons

# Operating Priorities

## 2017

For over forty years the Dystonia Medical Research Foundation has worked to put itself out of business. The mission of the organization from the beginning has been to **advance research** for more effective treatments and ultimately a cure; and because we recognize that the science can never move fast enough for those who are waiting, the DMRF is dedicated to the promotion of dystonia **awareness** and to providing up-to-date and accurate **educational resources**; and last but certainly not least, to **support** the well-being of dystonia-affected individuals and their families.

## RESEARCH

**Decades of supporting dystonia research has shown that advancing our understanding of dystonia requires a robust, multi-layered scientific program. It involves consulting with the scientific leaders to identify research gaps, stimulating dystonia research globally, creating an environment that fosters collaboration among investigators and constant scientific monitoring of not only the investments the DMRF has made in scientific projects but dystonia and other neurological research worldwide. DMRF's Chief Scientific Officer, a full-time scientist, will work with DMRF's Scientific Director, the DMRF Medical & Scientific Advisory Council (MSAC) and other global key opinion leaders to support innovative research leading to insights on the mechanisms of the dystonias, identify and characterize therapeutic targets, facilitates the development of more effective agents, and attracts young and experienced investigators.**

**R 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. Invite MSAC and recognized outside experts to the DMRF 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. Assess the best approach, Request for Applications (RFA) or contract, for the Myoclonus Dystonia Research Program. Develop and implement timeline to

support identified goals. Once approved, monitor these approved research projects;

3. Develop timeline and release a Request for Applications (RFA) for Musician's Dystonia. Utilize letter of intent mechanism to identify suitable projects to invite for full application. Once approved, monitor approved research projects;
4. To support and recognize the work of young investigators, develop and release a Request for Applications for research fellowships utilizing the Letter of Intent (LOI) mechanism, review the letters and identify those to be invited to submit a full application. Following review determine the strongest projects to support and should one be exemplary – designate it as the Mahlon R. DeLong Young Investigator Award.
5. After receiving input from the MSAC and Members of the Science Committee, identify the area of research the DMRF will explore in 2018 and then develop an RFA and the timeline for implementation for review by the Science Committee at the Mid-year Board of Directors' Meeting. Plan to utilize the LOI mechanism, review the letters, determine those to be invited to submit a full application, review and once approved, monitor these grants;
6. Continue to work with the National Institute of Neurological Diseases and Stroke (NINDS) staff to identify approaches and strategies to promote and support dystonia research, including but not limited to the development of a program for a 2018 consensus conference meeting to assess the state of dystonia research; and
7. DMRF Chief Scientific Officer (CSO) to represent DMRFC whenever scientific assessment, advice or reviews are needed.

**R Objective 2: The CSO shall establish contracts with academic researchers or pharma/biotech companies to conduct specific mechanistic or translational research and share appropriate outcomes to advance the field.**

*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.

**R Objective 3: Stimulate and support collaboration and the sharing of resources and mechanisms globally to advance dystonia research.**

*Action Steps:*

1. Promote to the research community funding opportunities from DMRF, the Dystonia Coalition, the National Institutes of Health (NIH), the Department of Defense Peer Review Medical Research Program (PRMRP) 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 enrollment in patient registries as a resource for the research community;
5. Maintain communication and explore collaborative opportunities with other dystonia organizations and research groups;
6. Provide current updates and report on research news on the DMRF website and in other DMRF publications; and
7. Develop a web platform that will promote the availability of research resources such as brain tissue, cell lines, etc.

**R Object 4: Engage and interact with pharmaceutical/biotech companies and other potential partners in 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 that have expressed interest in dystonia therapeutics development, and present dystonia as an opportunity;
2. Develop a program and convene a meeting of key clinical research experts to explore use of approved drugs for dystonia;
3. 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;

4. Work in partnership with the Dystonia Study Group to support their work as needed; and
5. Collaborate with industry partners to advance the development of their drug candidates for dystonia, including but not limited to assisting with the trial design, identification of key opinion leaders for consultation and possible participation, and assist with the regulatory path and detailed scientific rationale for the indication, as needed.

**R Objective 5: Support the international collaborative effort to reduce barriers to the development of new treatments by serving 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.**

*Action Steps:*

1. Work with Emory University to provide payments to the Coalition participating clinical centers as approved by Dystonia Coalition Principal Investigator (PI);
2. Develop and monitor subcontract agreements with sites participating in Dystonia Coalition Main Clinical Projects;
3. Provide payments to international consultants involved with the Coalition;
4. Disburse award payments to recipients of the Coalition's Career Development and Pilot Project Awards Programs;
5. Manage the funds received from Emory to pay out project related expenses outlined above;
6. Manage the private funds contributed by other dystonia patient organizations to support key Coalition projects/activities;
7. Participate in monthly Executive Committee calls and other administrative calls to ensure smooth operations of all Coalition activities;
8. DMRF Executive Director to serve as RDCRN Coalition of Patient Advocacy Groups co-chair; and
9. Provide access to DMRF's conference call and webinar platforms as needed.

**R Objective 6: Organize and participate in scientific meetings with key thought leaders, in and outside of the dystonia community, to foster and encourage collaboration and promote dystonia research, address current scientific/therapeutic issues, facilitate information sharing, network building, and identify critical areas of research and future directions.**

*Action Steps:*

1. Hold think tank-like meeting of MSAC in February to address key areas of dystonia research and suggest 'hot' topics for an RFA or meetings;
1. Support and participate in the, "*Dystonia: the link between hypo- and hyperkinetic movement disorders*" taking place in May in Rome, Italy;
2. Organize small, topic-focused meetings on identified aspects of dystonia research to identify areas to explore further. Meetings to be scheduled throughout 2017;
3. Attend/provide support for other dystonia-related meetings, when appropriate;
4. Organize a Clinical Fellowship dinner meeting in conjunction with AAN Annual meeting in Boston, MA in April 2017;
5. Executive Director to serve on NINDS Council and attend all meetings; and
6. Develop program committee for 6<sup>th</sup> International Dystonia Symposium. Explore potential dates and locations for the next international meeting (IDS6).

**R 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 timeline;
3. Review and identify qualified candidates;
4. Promote the program within the movement disorders clinical community; and
5. Make site visits to fellows as travels/schedules allow.

## **AWARENESS & EDUCATION**

**Raising awareness of dystonia is critical if we are to reach those who may be affected but not yet diagnosed, to reach those who are affected and feel isolated, to help the public understand what dystonia is and how it affects lives, and to engage researchers and clinicians to the field. The importance of having access to accurate information and educational resources cannot be overstated for people who are making treatment decisions, learning how to successfully manage their disorder and for family/friends to understand how they can help. Additionally, accurate educational materials help healthcare providers inform their patients.**

**A&E Objective 1: Keep the community apprised of relevant, accurate news on dystonia and DMRF activities through communications including three issues of The Dystonia Dialogue, scientific updates through Promise & Progress newsletter, and 2016 Annual Report plus news updates, as needed.**

*Action Steps:*

1. Working with the Editorial Board, identify key topics for each issue (Spring, Summer and Winter) of the Dialogue;
2. Develop, implement and comply with a 2017 Dialogue production schedule that complements the DMRF's development efforts with issues distributed March, June and October;
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 2016 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 April 2017; and
7. Working with the DMRF Chief Scientific Officer, develop lay summaries of significant publications on dystonia research.

**A&E Objective 2: Utilizing electronic platforms, promote accurate information on dystonia, DMRF priorities/activities and the availability of educational resources.**

*Action Steps:*

1. Update the website on an as needed basis to keep content current and easily accessible;
2. Working with Sharecare.com, complete the dystonia portal and monitor use;
3. Implement and assess an on-line module for Cervical Dystonia by May 1<sup>st</sup>, 2017;
4. Explore possible partners to increase outreach to the dystonia community and increase access to accurate dystonia information;
5. Identify key areas of interest and schedule 2 webinars (Spring and Fall) to provide information on these topics for the DMRF community and post webinars on the DMRF site following the program; and
6. Evaluate all efforts and report regularly to the Board of Directors.

**A&E Objective 3: Make educational programs accessible in key markets by organizing community education forums.**

*Action Steps:*

1. Identify at least 4 markets to develop and implement plans for community forums and evaluate each session; and
2. Work with support groups to assist in their educational programming. Attend local support group or other educational meetings as 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. Develop and distribute a monthly e-mail news (e-news) to highlight news, current activities, and any calls to action;
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; and
4. Conduct “Dystonia Moves Me” campaign to take place in September 2017 during Dystonia Awareness Month. Announcement to support network and community in April 2017.

**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 (online/video/audio);
2. Develop and implement a schedule for revising the identified materials;
3. Complete the translation of select publications into Spanish, distribute to the network, and promote availability of these resources by March 1, 2017;
4. Coordinate the dissemination of dystonia news through e-news; social networking sites, website, announcements and press releases (when relevant).

# SUPPORT

**The DMRF is committed to improving quality of life for dystonia-affected persons and their families through support programs and services. The DMRF is also committed to connecting people to one another to reduce feelings of isolation and facilitate the development of personal support networks.**

## **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. As appropriate, share the program successes/challenges of other support groups with all leaders;
3. To help leaders stay abreast of dystonia news, communicate all DMRF and dystonia news to leaders through regular communications;
4. Identify resources to help support leaders, including but not limited to webinars specifically for leaders, information, updates to leader manuals, etc.;
5. Explore new technologies to make efficient and extend the reach of support resources;
6. Assess the need for a policy and guidelines for leaders to use in managing group websites and social media outlets; and
7. 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 access to accurate information to make informed 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 obtain accurate dystonia information and access 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 March 1, 2017;
4. Work with all support leaders to ensure they have what is needed to provide support and education to their respective communities;
5. Encourage leaders to invite local movement disorder neurologists to join the DMRF physician referral list or if currently participating, to verify information and work with DMRF Clinical Fellows and identify other movement disorder neurologists to join referral list;
6. 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;
7. Respond to daily inquiries/requests for information within 48 hours of receipt;
8. Support the work of regional and area contacts; and
9. Promote new listings while confirming the contact information of existing listings on physician referral list.

**S Objective 3: Create opportunities for affected persons/families to connect with and educate one another.**

Action Steps:

1. Develop a plan to develop support groups in key metropolitan areas by April 1 and include a timeline for implementation;
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 online forums to ensure accurate information is being disseminated to ensure effectiveness of the resource; and
4. Support and monitor social networking and encourage participation in these electronic networks.

## ADVOCACY

**Through an increased awareness of dystonia among governmental officials at the federal, state and local levels and awareness of how they can support needed policy changes and funding for dystonia research, the DMRF will work to improve the lives of those affected by dystonia.**

### **Ad Objective 1: Develop and implement a legislative agenda to address the needs of the dystonia community.**

#### Action Steps:

1. By February 1st develop and have approved the 2017 legislative agenda. This should be done in consultation with Health & Medicine Counsel of Washington (HMCW) and the other Dystonia Advocacy Network (DAN) member organizations. The agenda should be monitored throughout the year;
2. Develop and implement plans for Advocacy Day, March 21 -22, 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 1, 2017;
4. Announce the Douglas Kramer Young Advocate award applications by February 1, 2017 and evaluate applications and determine awardee by March 2nd;
5. Develop and implement a communication plan for dystonia advocates to be used to engage their prompt response when Congressional action/inaction calls for it;
6. Working with HMCW team develop a program for visits in key Congressional District offices as needed to support the legislative agenda;
7. Partner with other organizations, as identified by issues, support mutual legislative efforts; and
8. Assess the need for State legislative programs to address patient access concerns as needed in 2016;

### **Ad Objective 2: Support opportunity for additional dystonia research funding by having dystonia included on the list of eligible conditions of the Department of Defense Peer-Reviewed Medical Research Program.**

#### Action Steps:

1. Working with identified Congressional champions for DoD funding to include dystonia on the FY2017 (Congress is currently working with a continuing resolution until April 30, 2017) and FY2018 lists;

2. Identify future champions and develop a strategy to engage them;
3. Include this issue on the DAN legislative agenda;
4. Identify veterans who are willing to work on this issue and assist efforts;
5. Implement plans for a letter writing campaign on DOD dystonia research funding;  
and
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. Work with HMCW to develop a plan for 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

Receiving and maintaining the public's trust is critical to the DMRF being able to achieve its mission. The DMRF takes seriously the responsibility of directing donor support effectively and efficiently. The DMRF will work to ensure organizational integrity and maximize the support provided the Foundation.

## Org Objective 1: Ensure the Foundation's financial and organizational integrity.

### *Action Steps:*

1. Working with the DMRF Audit Committee and outside financial consultant, ensure compliance with I.R.S., State and Foundation fiscal and personnel policies and procedures;
2. Conduct 2016 audit utilizing the outline of services in the 2016 engagement letter form external auditor;
3. Present engagement letter for review and approval to the and prepare for preliminary 2017 audit utilizing outside auditing services and conduct agreed upon procedures on Dystonia Coalition accounts;
4. Appoint a Tax Review Task Force to review 2016 taxes prior to filing and have a full copy of the tax filing available for all Members of the Board;
5. Work with newly appointed Finance Committee and outside financial consultants to develop an investment strategy; monitor investments and report to the Board;
6. Develop and submit operating budget for approval by Board of Directors at the annual meeting, monitor operating budget to expenses monthly and issue monthly financial statements with a report on budget variances;
7. 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;
8. Provide appropriate information to charitable rating organizations and monitor ratings;
9. Research, develop and implement a schedule for compliance with State and local charitable filing requirements;
10. Require completion of 2017 Board of Directors Code of Conduct Declaration Forms;
11. Conduct annual performance reviews on all DMRF staff per review schedules;
12. Assess need for and then retain, as needed, insurance for DMRF sponsored events;

13. Utilizing a bidding process, work to ensure all expenses are contained.

**Org Objective 2: Strengthen and expand the Foundation's leadership ensuring the DMRF's ability to achieve its mission.**

*Action Steps:*

1. Working with the Nominating Committee identify individuals who will enhance the Foundation's capacity through service on the Board of Directors or the Community Leadership Council or other organizational structures;
2. Orient leaders as needed to ensure they are well prepared to serve;
3. Elect members of the 2017 Audit Committee at annual meeting of Board of Directors on February 18th;
4. Identify individuals for service on Foundation's committees; and
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. Explore all cost savings opportunities through the bidding process and comparative purchasing for office equipment, supplies and services;
2. Develop and manage by individual staff working plans; and
3. Reduce rent responsibility by leasing space within the DMRF offices and manage the subtenant relationship.