



The Future of Parkinson's Public Policy: Top Priorities and Concerns of the Patient Community March 2016

In Spring 2016, the Parkinson's Action Network (PAN) and The Michael J. Fox Foundation for Parkinson's Research (MJFF) publicly announced PAN's plan to cease operations as an independent 501(c)3, with MJFF integrating key PAN staff as part of a new public policy team.

To help inform planning and prioritization for the new joint effort in policy, the organizations launched a dialogue with patients/supporters and industry partners to ensure that programmatic activity reflects the concerns of these vital stakeholders. This white paper reflects the top policy priorities articulated by these constituent groups for consideration and integration into the Foundation's programs.

Key themes raised by the community included research and access to new therapies; programs and resources affecting health care delivery; and the need to ensure that the Parkinson's community speaks on policy and regulatory issues with a unified voice.

Feedback reported in this white paper was captured through a webinar for grassroots advocates held on February 25, 2016, a panel discussion held on February 29, 2016, at the annual PAN Forum, and an online survey distributed to PAN constituents. Additional input was provided by The Michael J. Fox Foundation Board of Directors and members of its Patient Council.

This community dialogue will continue throughout the transition process and beyond to ensure that MJFF's public policy effort evolves in step with the Parkinson's community it serves.

Therapeutic Development: Need for Access to New and Available Treatments

MJFF and PAN share a history of supporting research to understand more about Parkinson's disease (PD) and to develop improved therapies. The Foundation is the largest nonprofit funder of PD research worldwide and works to mobilize patients toward greater research engagement. PAN has lobbied for increased allocation of taxpayer funds to support Parkinson's research through the National Institutes of Health and the Department of Defense. This dedication to furthering knowledge and supporting efforts to eradicate PD remains a cornerstone of the new joint effort in public policy.

Treatments emerging from the lengthy drug development pipeline still must navigate the complex regulatory approval process. In the United States, new therapies must be approved by the Food and Drug Administration (FDA) for physicians to prescribe them and for patients to be reimbursed by payers such as insurance companies and Centers for Medicare & Medicaid Services. PAN coordinated an FDA Patient-Focused Drug Development meeting on Parkinson's disease in 2015, among other actions, and MJFF has met with the FDA and has organized meetings between patients and payers to educate on the experience of Parkinson's and the potential impact of new therapies in development.

MJFF will build on the organizations' past work in this area to build a holistic approach to the development, testing and regulatory approval of new therapies. Expert coordination is required as drugs with the potential to slow or stop PD progression and new therapies to address untreated symptoms and drug side effects (PD psychosis and dyskinesia, for example) move closer to or through clinical testing. An integrated policy effort will allow MJFF to work seamlessly with patients, researchers, study sponsors, regulators and payers to determine and undertake best actions to speed progress. For example, the Foundation may work with investigators to design trials with FDA-accepted clinical scales; consult with biotech/pharmaceutical companies to chaperone emerging treatments through complex regulatory pathways; and help amplify the patient voice to give payers a more comprehensive understanding of unmet medical needs in Parkinson's.

The Foundation also is attuned to patients' concerns around evolving policy on drug pricing. Parkinson's drug development must balance the R&D costs incurred by drug makers (billions of dollars can go into the development of a single medication) with the need to ensure that patients are not "priced out" of new therapies as they come to market. MJFF is well positioned to work with companies and payers to set a standard for pricing and reimbursement that takes as its highest goal fair access to emerging therapies.

Parkinson's community members are enthusiastic about the potentially synergistic effects of a united research and policy effort. While calling for continued research support, they recognize how a central player whose work and expertise span the full drug development, regulatory and reimbursement process could lend a birds-eye view to help advance new treatments through testing and approval and ensure that patients have access to the treatments they need.

Health Care Delivery: Need for Access to Resources and Programs

The community clearly expressed its hopes for a public policy effort that encompasses, but is not limited to, research and therapeutic development. Access to health care and support services exerts a significant impact on patients' management of symptoms and quality of life.

Legislative history has shown that we cannot take our foot off the pedal. For example, since 2006, Congress has voted for exemptions on Medicare Therapy Caps, which limit coverage of extended physical, occupational and speech therapy for beneficiaries. However, exemptions are temporary and must be repeatedly renewed; the latest expires in March 2017.

The Advancing Research for Neurological Diseases Act to create a national data collection system for brain diseases, including Parkinson's disease, has passed the House of Representatives but needs approval by the Senate. Such a registry would help plan and implement research, care, programs and services to improve the lives of people with Parkinson's and other neurological diseases.

"Our grassroots leaders have experience advocating for all of these issues, and we look forward to working with MJFF on these matters," said Israel Robledo, PAN Texas state director, a member of the PAN Board of Directors and an MJFF clinical research participation ambassador.

PAN State Directors Look Forward to Continued/Expanded Involvement

A key element of the integration of PAN and MJFF will be the transition of PAN's grassroots leaders program to MJFF's new Grassroots Advisory Board. As the leaders program has been a vital component

of PAN's accomplishments, members (known as State Directors and Assistant State Directors at PAN) are focused on what new actions they can take and how they can best contribute to an evolving public policy strategy.

In addition to pointing out the value of established advocates for maintaining continuity through the transition, constituents shared what they saw as the most important aspects of their grassroots work. Many cited education on both ends – to their community members on the potential impact of advocacy and to legislators and to key opinion leaders on the experience of Parkinson's disease.

Steve Dewitte, an MJFF Patient Council member and PAN Connecticut Assistant State Director, wrote in an email: "I can relay examples of legislators moved by support group meetings and relationships with people with Parkinson's. Such advocacy can be done 365 days of the year: at home, at the state fair, at the coffee shop, not just in DC."

In addition to the Grassroots Advisory Board, MJFF is forming a Public Policy Council to provide expert guidance on policy strategies.

A new Unified Parkinson's Advocacy Council will become the platform for representatives from state, regional and national PD organizations to provide input on priorities and policy matters — a key consideration for many community members who called attention to the importance of a unified voice for Parkinson's advocacy.

Industry Recognizes Impact for Patients and on Drug Development

MJFF and PAN leaders also shared news of the planned PAN-MJFF integration with industry partners (biotech and pharma representatives). PAN's work promoting federal funding for Parkinson's research and FDA approval of new therapies supported a framework for drug discovery and bringing new therapies to market. MJFF works with industry across the spectrum of Parkinson's drug development: funding early-stage work and initial clinical trials, convening public-private and precompetitive consortia to address field-wide challenges, and creating tools from assays to clinical scales to assist in study design.

Biotech and pharma representatives recognized the potential impact of the unification on the drug development process. Bringing breakthrough treatments to market now requires focusing on policy issues in concert with R&D.

Two decades ago, the state of Parkinson's research as a whole lay primarily in basic research — the field held only a rudimentary understanding of the etiology of PD, little biomarker development was taking place, researchers were working with the (now-eclipsed) premise that dopamine replacement alone could meet Parkinson's patients' medical needs, and few promising drug candidates were advancing toward clinical testing.

Today, more drug development programs are reaching late-stage clinical testing and more companies are individually developing regulatory and reimbursement strategies where a collaborative approach, orchestrated by a centralized and unbiased convener such as MJFF, could be more effective.

Industry contacts also recognized the power of unifying the PAN and MJFF patient communities. This consolidation holds potential to reduce duplicative efforts and amplify the patient voice on issues of

greatest importance to people with Parkinson's. They hypothesized that the integration could introduce the impact of policy advocacy to greater numbers of Parkinson's patients and elicit broader community involvement.

MJFF Policy Priorities Will Grow from Patient and Industry Needs

Central to the philosophies of both MJFF and PAN is a partnership with patients and supporters to identify priorities, shape programs and publicize participation opportunities. The PAN grassroots community has been deeply engaged in outreach related to public policy. As the Foundation designs a holistic policy initiative, it will look to grassroots advocates to build on the initial discussion reported in this paper and to continue shaping the future of Parkinson's research and care.

Similarly, MJFF will work more comprehensively with companies to identify policy-related needs and strategize to streamline processes and speed progress.

As one industry contact put it: "MJFF and PAN are stronger together than they were separately." The Foundation believes that the synergistic effect extends to work with members and groups across the Parkinson's community.

To learn more about the joint future of Parkinson's public policy, visit www.michaeljfox.org/policy.