MJFF’S VISION FOR PARTNERING WITH PATIENTS

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WHAT IS PARKINSON’S?

Parkinson’s disease affects one in 100 people over age 60. Yet, individuals as young as 18 have been diagnosed. In the United States, 60,000 new cases will emerge in this year alone.

1 / 100
AVERAGE AGE 60

60,000 NEW

LESser Known Symptoms of Parkinson’s include depression, apathy, fatigue, loss of energy and dementia.

60 TO 80% OF DOPAMINE-PRODUCING CELLS ARE LOST BEFORE THE MOTOR SYMPTOMS OF PARKINSON’S DISEASE APPEAR.

The exact cause of Parkinson’s disease is unknown.

Although research points to a combination of genetic and environmental factors.

There is no test to diagnosis Parkinson’s disease. Because there is no test for it can take years to diagnose, delaying treatment.

Researchers are investigating potential early symptoms, such as constipation, impaired sense of smell, unusual fatigue and certain sleep disorders.

There is no cure for Parkinson’s disease today. But the Michael J. Fox Foundation is working urgently to change that.

Today’s best Parkinson’s drug was discovered in 1967. Lyndon B. Johnson was president and Neil Armstrong had not yet walked on the moon.

Dyskinesia is often mistaken as a symptom of Parkinson’s disease, but it is a serious side effect of the long-term use of Parkinson’s medications. Many patients report dyskinesia to be as debilitating as Parkinson’s disease.

1M / US
5M / WORLD

Today, an estimated one million people in the United States and more than five million worldwide are living with Parkinson’s disease (PD).
OUR SINGLE, URGENT GOAL: CURE PARKINSON’S DISEASE

MJFF’s Mission

The Michael J. Fox Foundation is dedicated to finding a cure for Parkinson’s disease through an aggressively funded research agenda and to ensuring the development of improved therapies for those living with Parkinson’s today.
TRADITIONAL RESEARCH APPROACHES INFORM CLINICAL DISEASE UNDERSTANDING

- Treatment impact on biological measures
- Cellular mechanisms and pathways
- Understand and predict risk
- Treatment impact on clinical characterization, diagnosis and progression
- Patient selection for clinical trials
MJFF was founded by a person with Parkinson’s disease.

Assessing all potential projects through a patient-focused lens, everything we do is driven by the many unmet medical needs of Parkinson’s patients today.
THE FUTURE OF PERSON-CENTERED THERAPIES

Improved medical products start with patient-centered research

» Patients and care partners are the real experts on Parkinson’s

» MJFF invested in Fox Insight to ignite a virtuous cycle of patient-centered research
  – Unfiltered, direct from patient data drive:
    • Funding decisions
    • Education and public policy efforts
    • Payer relationship and industry collaboration

» De-identified Fox Insight data will be available to qualified investigators to speed breakthroughs for patients
PATIENT-CENTERED MEASURES

Validated PRO instruments anchor cohort characterization and support interpretation of data collected using novel patient-centered tools.

**CASE STUDY 1: PD-PROP**

» PD Patient Reported Outcome of Problems captures uncategorical accounts of PD patients’ bothersome problems
  – “What bothers you the most about your PD?”
  – “In what way does this problem bother you by affecting your daily functioning?”
  – Problem severity (0-1-2-3 categorical scale)

**CASE STUDY 2: MDIC PPI survey**

» Patient engagement for identification of meaningful benefits and risks
» Working with patients as scientific collaborators promotes patient-centricity
» Good research practices can elevate PPI to valid scientific evidence
Our takeaway: the project is a strong model of fully integrating patient groups and patients in research.
OUR VISION FOR PARTNERING WITH PATIENTS

Increasingly, Parkinson’s patients are eager to be active contributors in the development of new treatments – how can we turn this into action?

» Medical products that meet patients’ needs will enable better health outcomes

» The science of patient input is challenging – especially in chronic diseases where preferences change over time and with disease progression

» Integrating patient-centered practices as part of clinical trial design moves us closer to the shared goal of therapies that meet heterogeneous patient needs

We applaud CDRH as a champion of transparent clinical trial design that includes patient preference and deeply value the elevation of the patient voice to a rigorous science.