



# MJFF'S VISION FOR PARTNERING WITH PATIENTS

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# WHAT IS PARKINSON'S?

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)

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



3 OUT OF 5

AMERICANS WILL SUFFER FROM A NERVOUS-SYSTEM DISEASE SUCH AS PARKINSON'S OR ALZHEIMER'S.

## LESSER KNOWN SYMPTOMS

OF PARKINSON'S INCLUDE DEPRESSION, APATHY, FATIGUE, LOSS OF ENERGY AND DEMENTIA



RESEARCHERS ARE INVESTIGATING POTENTIAL EARLY SYMPTOMS, SUCH AS CONSTIPATION, IMPAIRED SENSE OF SMELL, UNUSUAL FATIGUE AND CERTAIN SLEEP DISORDERS.



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.

60 TO 80%

OF DOPAMINE-PRODUCING CELLS ARE LOST BEFORE THE MOTOR SYMPTOMS OF PARKINSON'S DISEASE APPEAR.



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.



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 IT CAN TAKE YEARS TO DIAGNOSE, DELAYING TREATMENT.

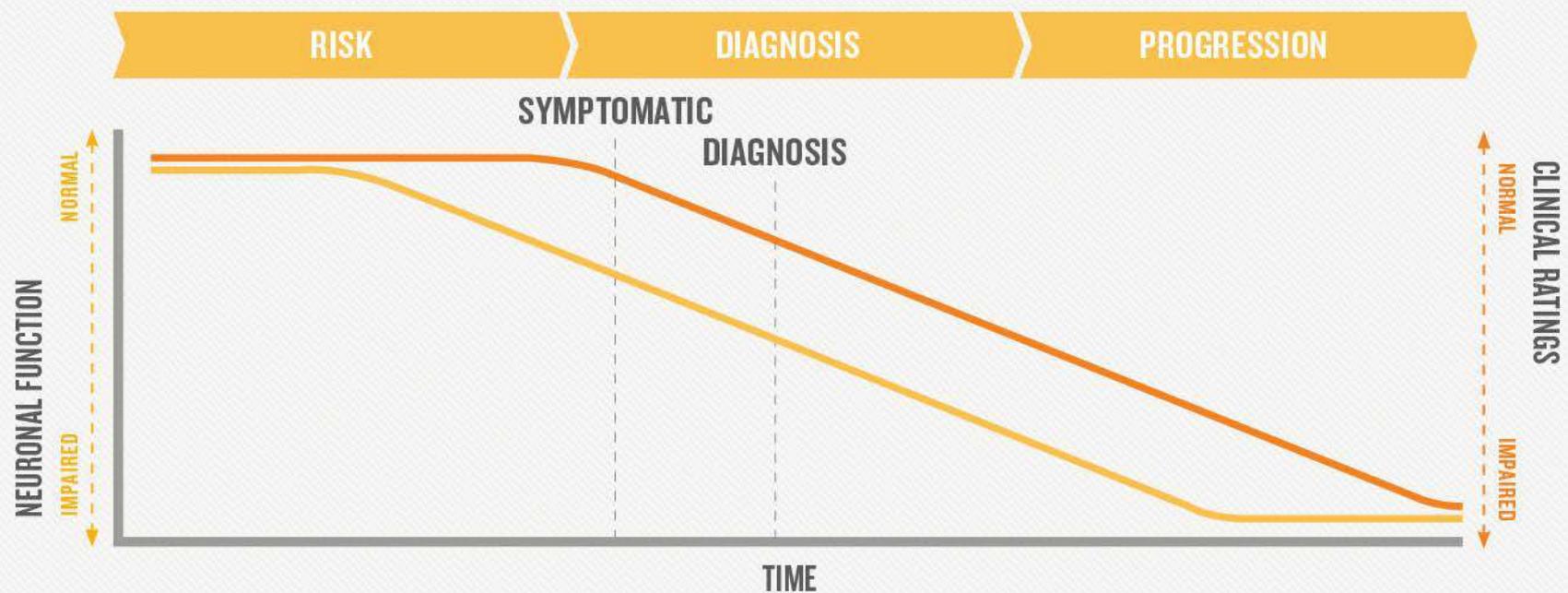
THERE IS NO CURE FOR PARKINSON'S DISEASE TODAY.

BUT THE **MICHAEL J. FOX FOUNDATION** IS WORKING URGENTLY TO CHANGE THAT.

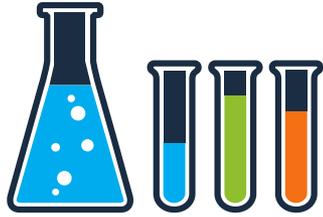
# OUR SINGLE, URGENT GOAL: CURE PARKINSON'S DISEASE

## MJFF's Mission

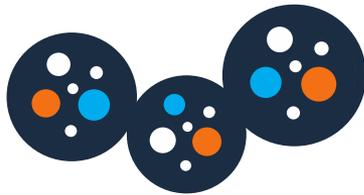
The Michael J. Fox Foundation is dedicated to finding a cure for Parkinson's disease through an aggressively funded research agenda and to ensuring the development of improved therapies for those living with Parkinson's today.



# 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

# PATIENTS' NEEDS DRIVE OUR EFFORTS



**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



The future of Parkinson's research is in powerful hands.

Yours.



# 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

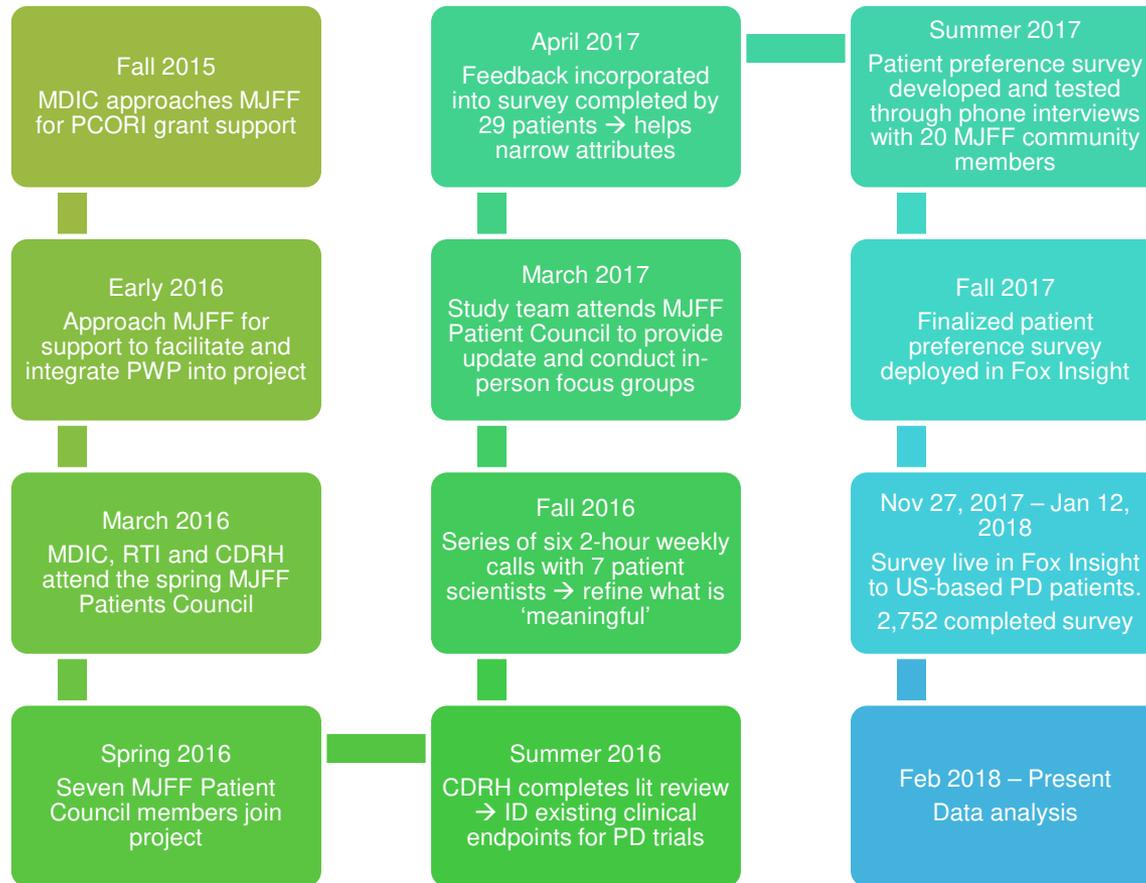
- » PD Patient Reported Outcome of Problems captures uncategorical accounts of PD patients' bothersome problems
  - “What bothers you the most about your PD?”
  - “In what way does this problem bother you by affecting your daily functioning?”
  - Problem severity (0-1-2-3 categorical scale)

## 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



# MDIC PPI SURVEY PROJECT



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.**

