THE MICHAEL J. FOX FOUNDATION'S DIGITAL HEALTH STRATEGY

Lauren Bataille, MS
Senior Associate Director, Research Partnerships

MJFF BACKGROUND

MJFF IS THE WORLD'S LARGEST NONPROFIT FUNDER OF PD RESEARCH

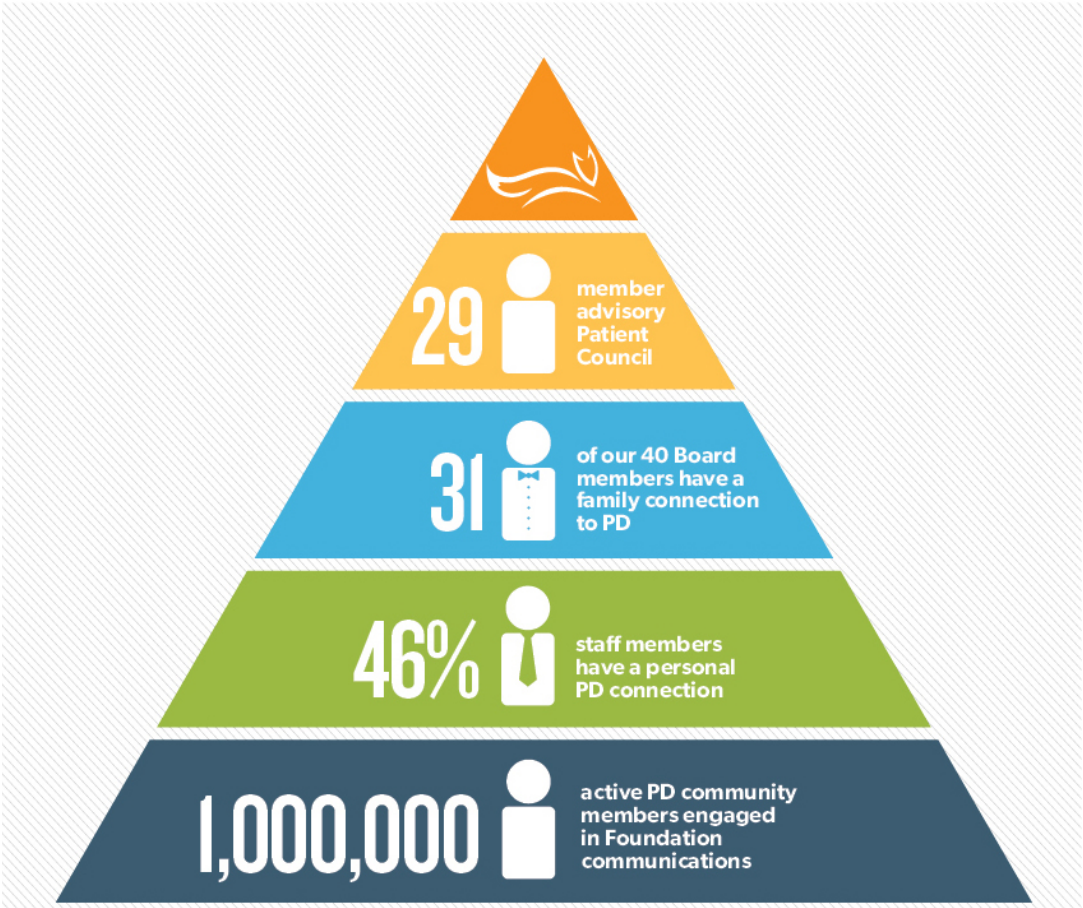
Our Mission

To accelerate the development of improved therapies, and ultimately a cure, for people living with Parkinson's disease today.

Vital Stats

- Founded in **2000** by actor Michael J. Fox
- Public charity
- Nearly **70,000** donors in 2015 (individuals, corporations, nonprofits)
- No chapters: team of **100** based in NYC
- **3,300** grassroots fundraisers reaching **150,000** supporters worldwide in 2015
- More than **$600 million** in research programs funded to date
- **$87.8 million** in research programs funded in 2015
- Nearly **2,100** projects funded to date
- **550** active grants in current portfolio
- **33%** of funded projects are led by researchers outside the United States
- Fund academics, biotechs and pharma

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.

MOBILE AND SENSOR TECHNOLOGY IN PD RESEARCH

PORTFOLIO OVERVIEW

GOAL: Improve Parkinson's therapeutic development through the use of trusted digital endpoints.

OBJECTIVES:

1. Drive the development of PD-centric novel digital endpoints
2. Determine whether digital objective measures are non-inferior or superior to traditional clinical assessments
3. Facilitate development of a digital PD biomarker

Mobile-generated data can enhance traditional endpoints or create new endpoints for Parkinson's disease that are MORE objective, have HIGHER data resolution, and can collect data CONTINUOUSLY

SUPPORTING THE USE OF MOBILE & SENSOR-BASED TECH IN PARKINSON'S RESEARCH

Partnerships with key opinion leaders to use mobile and sensor-based technology in Parkinson's research

- **Baseline studies**: sensor-derived data vs. gold standard clinical assessments
- **Population studies**: feasibility of long-term use, user-behavior
- **Clinician Input**: impact of remote-capture sensor and PRO data on clinical care
- **Ecological validation**: analytical tools development and validation dataset for application in real-world environment
- Implementing sensor sub-studies in MJFF cohorts (e.g. PPMI, Fox Insight)
- Open RFAs
- Partnering as thought leaders and/or funders to support external research efforts

All study materials and datasets from MJFF sponsored studies to be made publicly available for field-wide use

FACILITATING COMMUNITIES FOR OPEN ACCESS DATA SHARING & PRE-COMPETITIVE PARTNERSHIPS

- **Digital Analytical Tools Development**: initiatives to develop open-access analytical tools for the Parkinson's research community
  - Make datasets publicly available
  - DREAM Digital Biomarker's Data Challenge
- **CTTI Novel Endpoints Project:** issue recommendations to clarify pathway for developing novel endpoints for use in clinical trials
- **Mobile Tech Advisory Council:** consortium representing 12+ pharma companies to accelerate use of mobile technology in regulated trials
  - *2017 data standards focus*

PROPOSED ROADMAP FOR DIGITAL ANALYTICAL TOOLS PRIORITIZATION

	Short Term	Medium Term	Long Term
Priority Areas	**Replication of gold standard motor assessments**	**Replication of gold standard non-motor assessments and novel measures**	**Novel Measures**

High Priority Symptoms in Index or Indices	- Motor Symptom
	  - Bradykinesia
	  - Dyskinesia
	  - Tremor
	  - Gait/Ambulation
		- ON/OFF
		- Non-Motor Symptoms
		  - Sleep
		  - Cognition
		- Activity
		- Posture
			- QoL (mood, depression/anxiety, socialization)
			- Priorities for de novo cohorts (integration of sensor measures for HR variability, autonomic function)

Validation Strategy	Validated against existing gold standards	Mixed	Need for novel validation strategies

Multi-pronged approach to execution of tools development

Parkinson's Disease Digital Biomarker DREAM Challenge

ENABLED BY

HARVARD UNIVERSITY · THE MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH · Robert Wood Johnson Foundation · Sage · verily · UNIVERSITY of ROCHESTER

Launching Summer 2017

L-Dopa Challenge

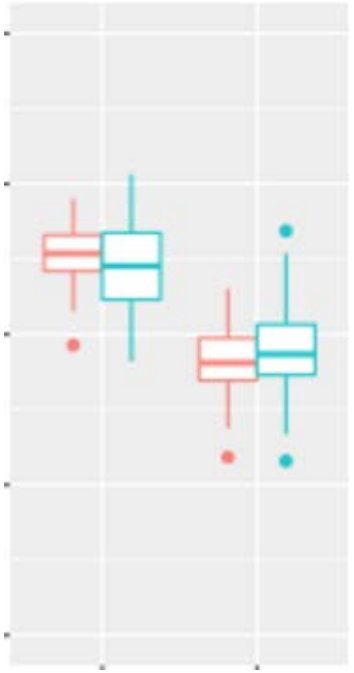
THE MICHAEL J. FOX FOUNDATION FOR PARKINSON'S RESEARCH

Gait

Balance

???

Feature Engineering



www.synapse.org/DigitalBiomarkerChallenge

CTTI MOBILE CLINICAL TRIALS (MCT) PROGRAM

» **PURPOSE:**

Develop evidence-based recommendations that affect the widespread adoption and use of mobile technology in clinical trials

» **ANTICIPATED IMPACT:**

Increase the number of clinical trials appropriately leveraging mobile technology

4 PROJECTS

**Scope: FDA-regulated clinical trials after the time of initial research volunteer consent*

THE MCT NOVEL ENDPOINTS PROJECT

- **Project objective**: Describe best practices for developing novel endpoints, generated using mobile technology, for use in clinical trials.
- We defined novel endpoints as either
  1) new endpoints that have not previously been possible to assess, or
  2) existing endpoints that can be measured in new and possibly better ways
- Interdisciplinary project team – pharma, tech, regulatory, patient organizations
- Recommendations and tools will be officially launched during a webinar on June 26
  - Sign up at https://www.ctti-clinicaltrials.org/briefing-room/webinars

Recommendations, novel endpoint development benefit framework, selection tool to support decision between viable endpoints for development, guide to interacting with FDA regarding novel endpoint development, four use cases to provide tangible examples of novel endpoint development (Parkinson's, heart failure, diabetes, Duchenne's muscular dystrophy)

MOBILE TECH ADVISORY COUNCIL

- **Mission**: The Mobile Tech Advisory Council is a pre-competitive consortium representing pharma/biotech with the mission of accelerating the development and use of novel endpoints in Parkinson's disease clinical research to benefit people living with Parkinson's disease.
- **Goals**
  - Understand the rate limiting steps of using mobile technology in PD drug development
  - Identify strategies to overcome barriers
    - Identify funding priorities and specific projects for MJFF and Council members to support and collaborate on
  - Achieve buy-in and adoption from key stakeholders

MOBILE TECH ADVISORY COUNCIL 2017 PRIORITIES

- Case study and best practices sharing
- Engagement with patient community
- Mobile-generated data standards development

MTAC ROADMAP

- **Long-term goals:** Validated analytical tool(s) for use in PD trials that improve quality of clinical research
  - **Data standards:** develop a set of standards that provides contextual information for individual data points collected from a mobile device
  - **Data collection:** Establish a repository of datasets that can be used to (1) develop research tools for use in Parkinson's research, and (2) identify new insights about PD (e.g. symptoms, disease subtyping and progression
  - **Data analytics and tools development:** MJFF to incentivize data sharing and algorithm sharing

	2017	2018	2019
Standards development	**Metadata standards development**	**PD CDISC 2.0 including pilot mobile standards**	Standards utilized
Dataset repository development (data collection)	**Prioritize dataset collection** (e.g. MJFF-sponsored trials, industry, academic)	Commitment from industry to share data	Industry shared data
Analytical tools development and testing (experimenting)	**Data challenges, grant support for analysis of available datasets**	1) Data challenges, grant support for analysis of available datasets 2) Evaluation of available research tools	Sharing outcomes
Analytical tools used in PD clinical development (proof of concept)	Exploratory	Exploratory	Exploratory/secondary
Patient engagement →			
Engagement with partners/groups working on similar initiatives →			

MOBILE-GENERATED DATA STANDARDS DEVELOPMENT

Metadata ontology development

Bottom-up approach

- OVERVIEW: specifications outlining what information needs to accompany mobile-generated data in order for it to be meaningful
- MTAC initiative
- RATIONALE
  - Automated analysis of datasets
  - Interoperability and reproducibility of mobile-generated data (enable meaningful data sharing)
  - Facilitate development of trusted digital endpoints
- *Summer 2017 workshop*

Parkinson's CDISC 2.0 development

Top-down approach

- OVERVIEW:
  - Update current standards to reflect data captured across regulated PD studies
    - Include pilot mobile PD standards
- CPP/CDISC initiative
- RATIONALE
  - Identify key motor endpoints for Parkinson's disease research
  - Develop standardized specifications for regulatory submissions

QUESTIONS

LEARNING THROUGH ACTION: UNDERSTANDING THE STATE OF THE FIELD