LEVERAGING PARTNERSHIPS TO SUPPORT DIGITAL BIOMARKER DEVELOPMENT

2nd Annual Digital Biomarkers in Clinical Trials Summit

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WHAT WILL BE COVERED TODAY

1. Brief overview of the Michael J. Fox Foundation for Parkinson's Research (MJFF)

2. MJFF's philosophy toward digital biomarkers

3. Case studies highlighting collaborations to support digital biomarker development

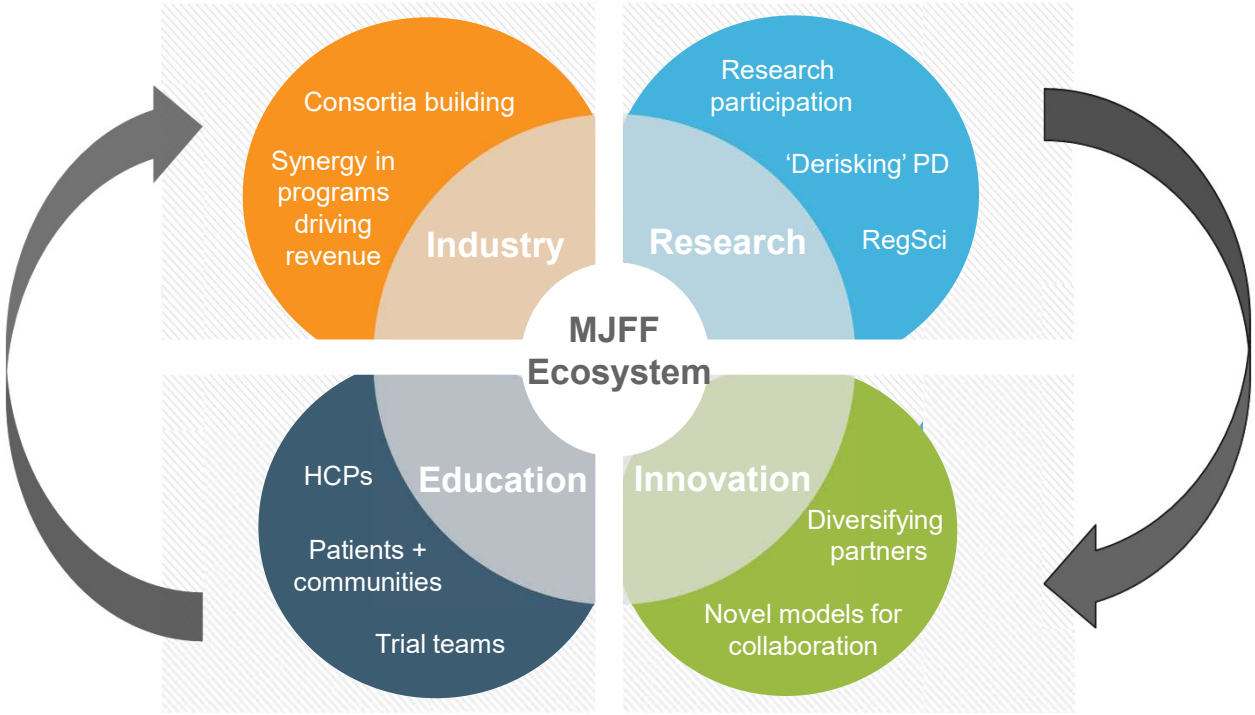
MJFF'S SINGLE URGENT GOAL: ELIMINATE PARKINSON'S DISEASE IN OUR LIFETIME

Today we are the largest nonprofit funder of Parkinson's research worldwide. Everything we do is driven by the unmet need of people living with PD.

- Founded in **2000** by actor Michael J. Fox
- Public charity operating without any endowment
- Over **$800 million** in research programs funded to date
- Over **$90 million** in research programs funded in 2018
- More than **2,600** projects funded to date
- Over **600** active grants in current portfolio
- Active **tool generation** to support PD therapeutic development
- **34%** of funded projects are led by researchers outside the United States
- Funding to **academics, biotechs and pharma**
- Address **policy and advocacy** needs for PD community

"FOR PATIENTS, PARKINSON'S DISEASE IS NOT A TIME-NEUTRAL SITUATION. IT'S A TICKING CLOCK."
– MICHAEL J.

PARTNERSHIPS ARE CORE TO THE MODEL MJFF USES TO ACCELERATE DRUG DEVELOPMENT



MJFF builds partnerships across the pipeline to clinical practice. MJFF's stakeholder approach focuses on engaging an increasingly diverse group of stakeholders to advance our mission.

MJFF APPROACH TOWARD DIGITAL BIOMARKERS

DIGITAL TECH OFFERS MANY ADVANTAGES TO UNDERSTANDING & TREATMENT OF PD

People with PD

- Self-management of disease; minimal risk
- Better understand medication effect on different activities
- Alternative, non-invasive way to engage in research

Clinicians

- Allow for closer, more consistent monitoring of individuals outside the clinic, including medication response and symptom changes

Researchers

- Creates large, innovative datasets for analysis
- Novel, objective endpoints for clinical trials, which could speed time and decrease cost of drug development
- Clinical visit not required for data acquisition

MJFF sees immense value in supporting research that can better align digital tech with the needs of the PD community.

SUPPORTING DIGITAL APPROACHES FOR PD OUTCOME MEASUREMENT

Goal

» Improve Parkinson's therapeutic development through the use of trusted digital endpoints

Objectives

» Drive the development of PD-centric novel digital endpoints

» Determine whether and when digital objective measures are superior to traditional clinical assessments

» Increase understanding of disease heterogeneity through digital measurements

MJFF believes that digital-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**.

To achieve our objectives, we have entered into a number of partnerships focused on **data generation and analyses** to support endpoint development.

CASE STUDIES

CASE STUDY: PARKINSON'S DISEASE DIGITAL BIOMARKER DREAM CHALLENGE

Goal

Catalyze use of remote sensor data to develop methods to diagnose and track Parkinson's disease.

Approach

Open crowd-sourced analytical project divided into two sub-challenges:

1. Use accelerometry and gyroscope data collected through mPower to predict whether the user had PD;
2. Extract features for three PD symptoms to predict clinician-assessed severity of symptoms.

Collaborators

- Challenge enabled by Sage BioNetworks, University of Rochester, Verily, Robert Woods Johnson Foundation and Harvard University.
- Participants included 440 data experts from six continents.

Result

Winners of challenge developed methods that are:

- 38% better than previous models at detecting PD from walk and balance test;
- 58% better at predicting severity of different symptoms than baseline models of sensors.

Impact

Methods increase our confidence in monitoring and tracking disease outside of a clinical setting using objective measurements.

CASE STUDY: DIGITAL DATA CAPTURE IN PPMI

PARKINSON'S PROGRESSION MARKERS INITIATIVE

Goal

Utilizing PPMI – an observational natural history study of Parkinson's patients, individuals at risk to develop PD and controls – correlate digital data capture with traditional markers to improve our understanding of how we should apply digital technology to PD.

Approach

Integrate two forms of digital data capture within PPMI:

1. Roche phone App that includes passive and data collection;
2. Verily watch sensor that passively collects data.

A total of ~1,000 individuals will have digital data collected in addition to providing clinical data and biologic samples and undergoing imaging on a longitudinal basis.

Collaborators

- MJFF
- PPMI leadership and study infrastructure
- Roche
- Verily

Result

Raw and processed digital data collected from study participants will be made available through PPMI's open access database (www.ppmi-info.org). Researchers – both within and external to the study – will be encouraged to analyze that data in conjunction with other collected data.

Impact

Digital disease markers discovered through this collaboration could change the way Parkinson's trials are carried out, by offering a faster way to assess disease change and therapeutic impact.

CASE STUDY: DEVELOP DIGITAL BIOMARKERS FOR PRODROMAL PARKINSON'S

Goal

Incorporate digital biomarkers to help create a 'risk score' that predicts who will develop Parkinson's disease within a two-year time frame.

Approach

Taking a step-wise approach, utilize:

- PRO capture
- Genetic testing
- Smell testing
- Digital biomarkers
- Imaging

to create a risk factor algorithm to identify individuals in the prodromal phase of PD.

Collaborators

- MJFF
- PPMI leadership and study infrastructure
- Roche

Result

Data – PROs, imaging, digital data – will be aggregated to create a risk score to help identify individuals at the prodromal stage who will 'phenoconvert' to Parkinson's disease within a two-year time frame.

Impact

Creation of a 'PD risk algorithm' could facilitate studies that aim to 'prevent' onset of disease in addition to treatment of disease.

THANK YOU!