THE MICHAEL J. FOX FOUNDATION’S MOBILE TECHNOLOGY STRATEGY

2016 CAMD Annual Regulatory Science Workshop
OUTLINE

- Rationale
- Fox Insight Wear App
- Clinical Studies
- Data Availability and Sharing
- Overview of The Michael J. Fox Foundation Mobile Tech Advisory Council
RATIONALE
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
» More than $650 million in research funded to date
» $87.8 million in research grants funded in 2015
» 2,100 research projects funded to date – industry and academic grantees
» 35% of funded projects are outside of the United States
MOBILE TECH IN THE CONTEXT OF OUR RESEARCH PORTFOLIO

Drug Development
- Disease Modification
- Improving Symptoms

Field-Wide Challenges
- Pre-clinical Research Tools
- Biomarkers
- Clinical Scales
- Regulatory Hurdles
- Patient Participation in Clinical Trials

Mobile and sensor-based technologies have the potential to support field-wide solutions for research and patient engagement
FOX INSIGHT WEAR APP DEVELOPMENT
LEARNING THROUGH ACTION: FOX INSIGHT WEAR

The mobile application paired with a Smart Watch allow users to provide as much – or as little – information as they want.

Current collection strategies:

**Passive data acquisition:** accelerometry

**Performance outcomes (PerfO):** Gait quality, hand rotation speed

**Patient Reported Outcomes (PRO):** Medication reporting and adherence, symptoms, questions, app feedback
REAL-TIME ANALYTICS APPLIED TO CONTINUOUSLY COLLECTED DATA

Activity Level

Nighttime Movement

Tremor

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PERFORMANCE OUTCOMES
PATIENT REPORTED OUTCOMES

My Symptoms

Select symptoms relevant to you

Tremor
Involuntary rhythmic movements

Dyskinesia
Involuntary, uncontrollable and often excessive movements

Rigidity
Muscle stiffness in a limb or other body part

Bradykinesia
Slowness of movement

Gait Problem
Difficulty with walking

Balance Problems
Unsteadiness when standing or walking

Step 1 of 4

Tremor

Record

Manager

Log

Add Medication Reminder

13:56

Delayed today by 26 min

Add Medication

Belladonna
Tincture - 1 pill

Edit
BETA TESTING & APP DEVELOPMENT

Beta Testing
» Before the launch of new versions of the app, a group of Beta Testers test the app
» Their feedback helps Intel identify bugs and improvement suggestions

App Development
» With feedback from users, Beta Testers, researchers, and data experts, MJFF and Intel work together to prioritize new app features for both patient and researcher benefit
» Examples of new features include…

Gathering feedback from patients and developing features that are important to them is essential for long-term engagement and retention.
THE APP IS BEING USED TO COLLECT DATA IN SIX RESEARCH STUDIES

✓ Collect sensor data in controlled and non-controlled environments
✓ Develop objective measures of PD symptom severity
✓ Feasibility of long-term use
✓ Further develop technology
**EXAMPLE 1: LONGITUDINAL OBSERVATIONAL COHORTS**

<table>
<thead>
<tr>
<th></th>
<th>Fox Insight Wear</th>
<th>Parkinson@home</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>n</strong></td>
<td>700 (~ 3,000)</td>
<td>350 (~1,000)</td>
</tr>
<tr>
<td><strong>Phase 1</strong></td>
<td>Feasibility, System optimization</td>
<td></td>
</tr>
<tr>
<td><strong>Phase 2</strong></td>
<td>Correlate sensor data to data collected through virtual, online trial Fox Insight</td>
<td>Correlate sensors data to at-home collected clinical data</td>
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**System optimization:**
- Patient engagement
- Data assessment
- iOS and Android compatible
- Additional smartwatches
- Ecological validation
MOBILE TECHNOLOGIES CAN SHIFT THE PATIENT ENGAGEMENT PARADIGM

Mobile supports massive geographic distribution at an unmatched scale
## Example 2: Clinician-Input Study

<table>
<thead>
<tr>
<th>Objective</th>
<th>Study Population</th>
<th>Assessments/Data Collection</th>
<th>Outcomes</th>
</tr>
</thead>
<tbody>
<tr>
<td>- Feasibility of using remote capture wearable device data from PD patients in clinical practice</td>
<td>- 50 people with PD who are current patients of study co-investigators</td>
<td>- Motor assessments in OFF/ON states</td>
<td>- Develop data dashboard for the clinicians</td>
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<td></td>
<td>- Subjects followed for 6 months: 5 in-person visits</td>
<td>- Medical history and surveys</td>
<td>- Develop electronic ON/OFF diary</td>
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<td></td>
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<td>- Hauser diary</td>
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<td></td>
<td></td>
<td>- In-office and at-home remote monitoring: 24/7 activity level and PD symptoms monitoring</td>
<td>- Correlation of FI app’s reported measures with patient’s reported experiences</td>
</tr>
</tbody>
</table>
CLINICIAN DASHBOARD MOCK-UPS


Levodopa, Atemet
3/28/16-6/26/16

Measurement
1/1 - 1/15

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ON/OFF DIARY

Step 1 of 3

Today
09:00-09:30
(Missing Note)

On / Off State

? 0 1 2 3 4

Full ON  Full OFF

I don’t know, I was asleep

Next

Step 2 of 3

Today
09:00-09:30
(Missing Note)

Dyskinesia

? None  Slight  Mild  Moderate  Severe

Next

Step 3 of 3

Today
09:00-09:30
(Missing Note)

Activity Intensity

? None  Slight  Mild  Moderate  High

Done
EXAMPLE 3: COLLECTING SECONDARY OUTCOMES IN PHASE 3 CLINICAL TRIAL

» Parent trial: Cynapsus; sublingual apomorphine – fast acting treatment to turn patients “ON” and provide rapid relief for “OFF” episodes

» Substudy: n= 40

Objective measures: Accelerometry; activity level, tremor, gait detection

PROs: symptoms, med intake and adherence

2 DAYS
DATA AVAILABILITY AND ACCESS
MOBILE AND SENSOR-DERIVED DATA

» Watch and phone sensors
  – Accelerometer
  – Additional sensor data coming soon

» Aggregated measures
  – Activity Level
  – Tremor Level
  – Movements Level
  – Gait detection
  – Walking assessment measures
  – Activity level threshold
  – Nighttime analysis
  – Nighttime measurements

» Non-analytical data
  – Medication reports
  – Medication schedule
  – Symptoms reports
  – ON/OFF pop-up
  – ON/OFF diary
A sample of the Levodopa Response Trial data is now available on the MJFF website.
MJFF HAS AN INITIATIVE TO PROVIDE RESEARCHERS WITH CURATED, HARMONIZED DATA

» Parkinson’s research will be more efficient, timely, and cost-effective for the research community with curated & harmonized data

  » Data format: Data available from clinical trials are often raw and uncurated, making it difficult to compare the same variables across datasets.

    » Solution:

      » All datasets are curated into the same format, allowing for integration into standardized variables

      » Provide access to the curated datasets - rather than raw datasets - once qualified researchers sign appropriate data use agreements

» Analyzing common variables across data sets will enable novel data discovery

  » Potential for meta-data analyses

  » More efficient hypothesis generation and testing

  » Increased sample size leads to increased power

  » Ability to look across disease areas
The Improved Biomarkers and Clinical Outcomes program supports research that will develop improved biomarker tools and clinical outcome measures to assist in clinical trial design, execution and interpretation of results.
MOBILE TECH ADVISORY COUNCIL

Who: Representatives from pharma leveraging mobile technology to support therapeutic drug development

Why: Help shape MJFF allocations for mobile and wearable technology around therapeutic PD drug development

Objectives:
- Understand the rate limiting steps of using mobile technology in PD drug development
- Identify strategies to overcome barriers
- Share outcomes and recommendations with technology companies working in healthcare space
- Identify funding priorities and specific projects for MJFF and Council members to support and collaborate on
THANK YOU

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