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

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

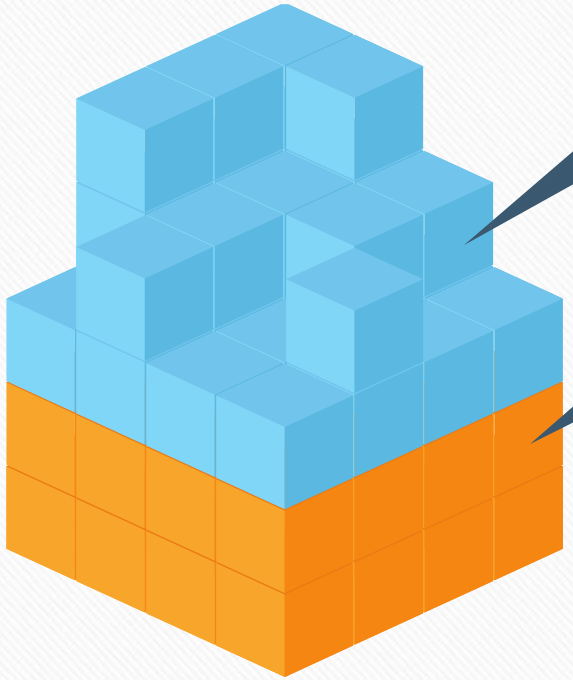
» More than **$650 million** in research funded to date

» **$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

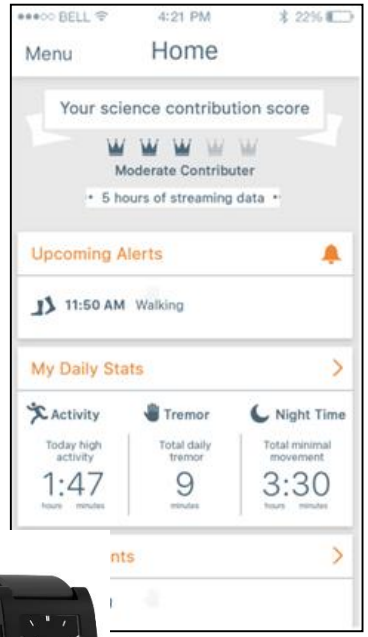
- Pre-clinical Research Tools
- Biomarkers
- Clinical Scales
- Regulatory Hurdles
- Patient Participation in Clinical Trials

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

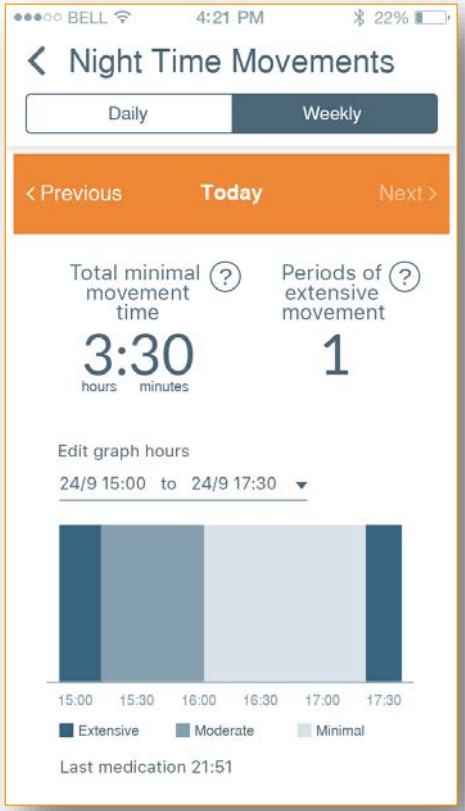
Performance outcomes (PerfO): Gait quality, hand rotation speed

Patient Reported Outcomes (PRO): Medication reporting and adherence, symptoms, questions, app feedback

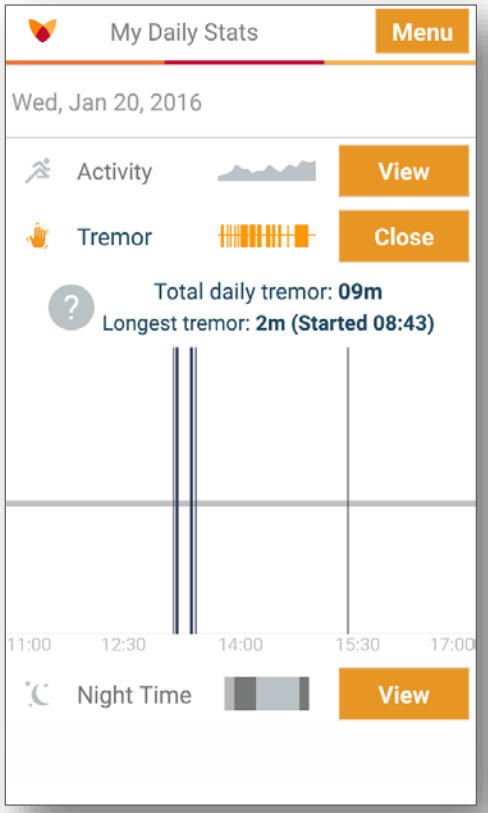
REAL-TIME ANALYTICS APPLIED TO CONTINUOUSLY COLLECTED DATA

Activity Level

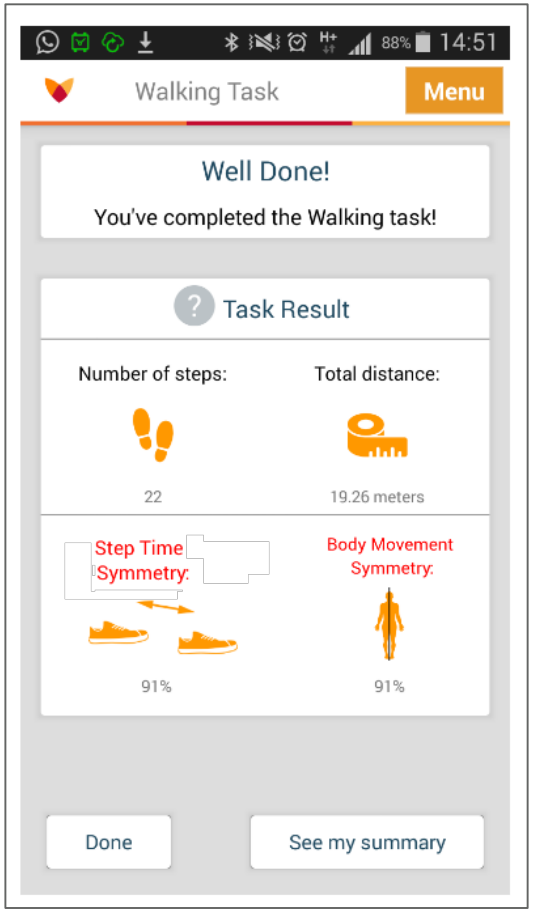
Nighttime Movement

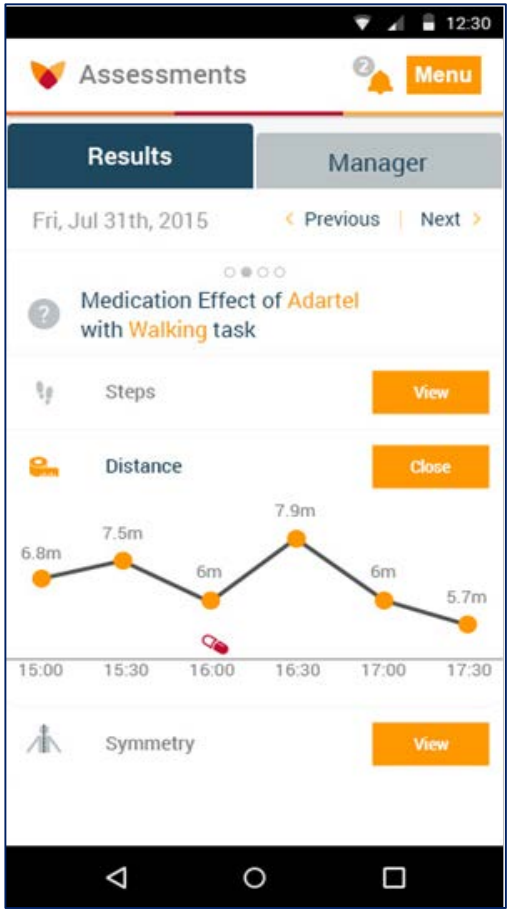


Tremor

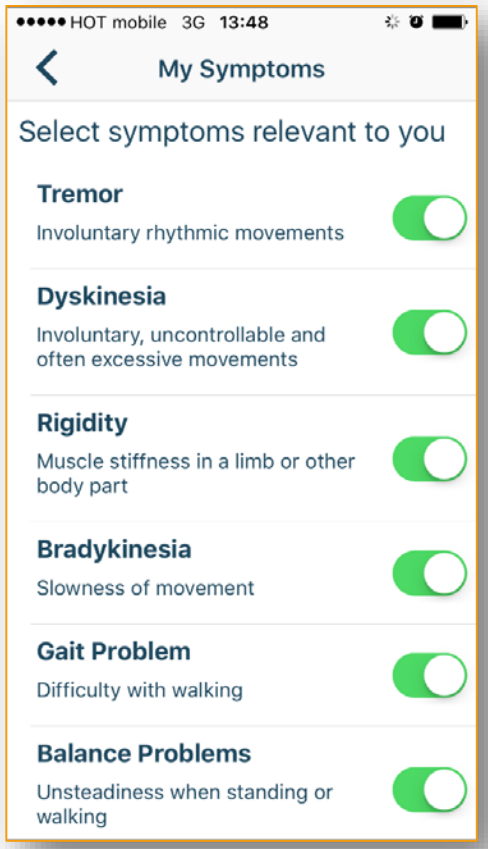


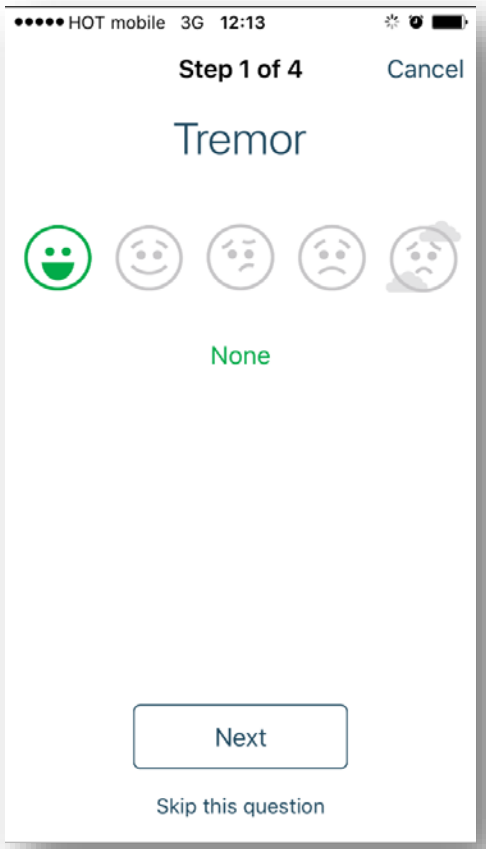
PERFORMANCE OUTCOMES

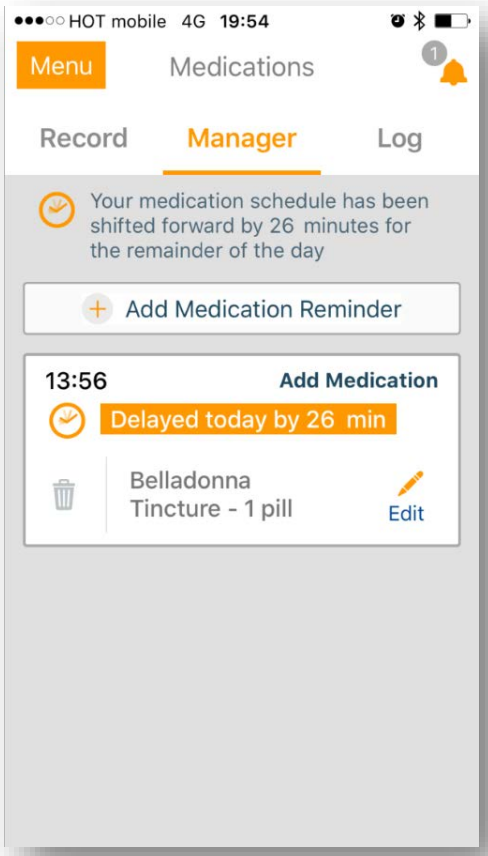




PATIENT REPORTED OUTCOMES







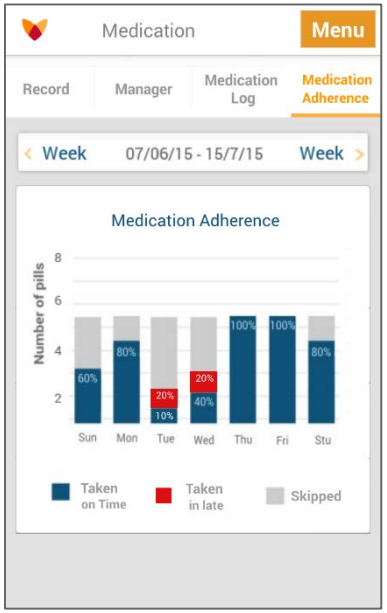
BETA TESTING & APP DEVELOPMENT

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…



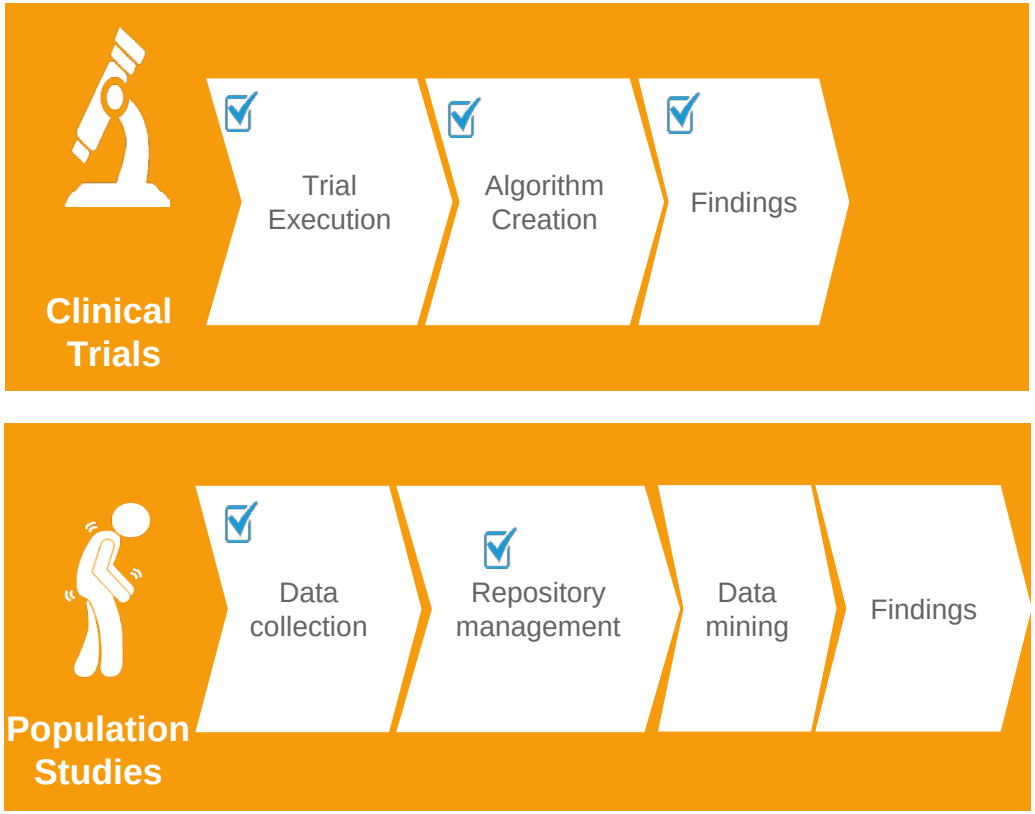
Improve medication adherence with reporting

Enhance activity level with daily activity goals

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

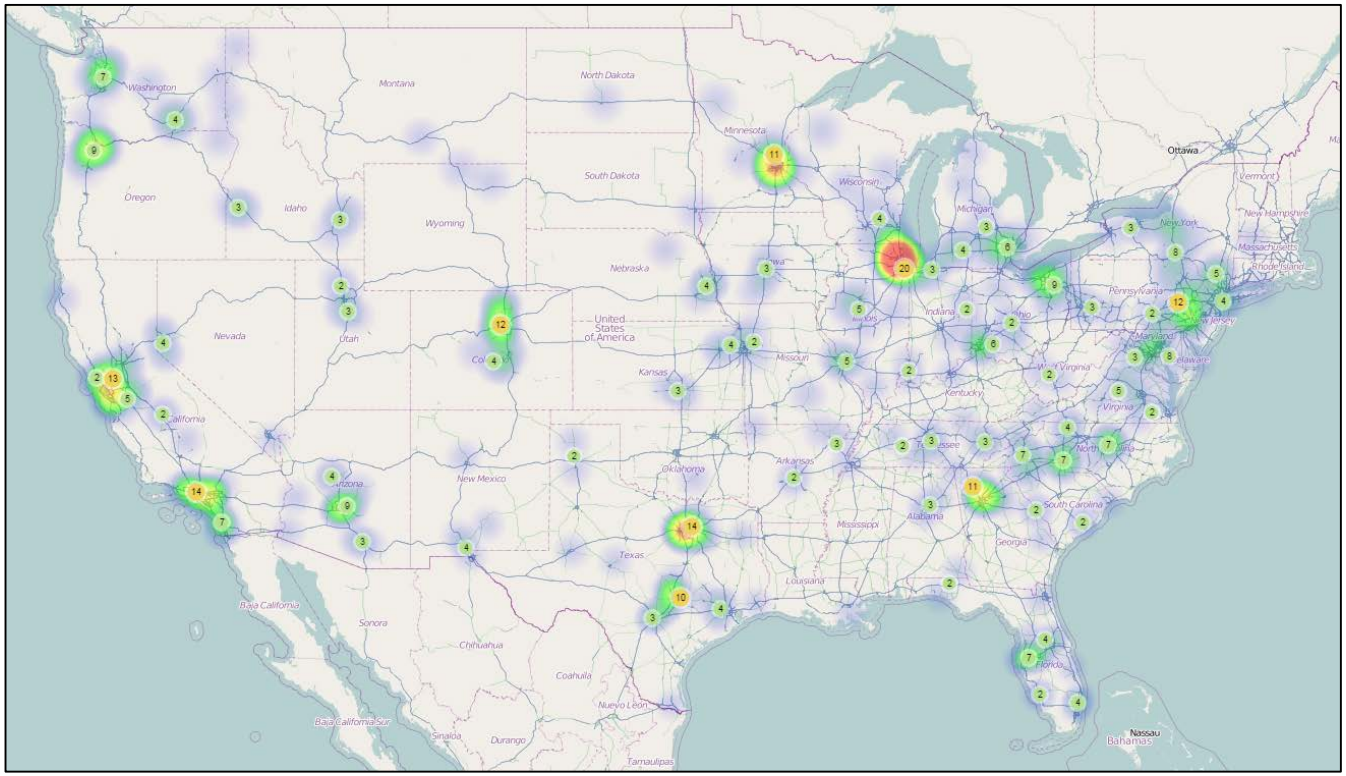


EXAMPLE 1: LONGITUDINAL OBSERVATIONAL COHORTS

	Fox Insight Wear	Parkinson@home
n	700 (~ 3,000)	350 (~1,000)
Phase 1	Feasibility, System optimization	
Phase 2	Correlate sensor data to data collected through virtual, online trial Fox Insight	Correlate sensors data to at-home collected clinical data

- **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

OBJECTIVE

- Feasibility of using remote capture wearable device data from PD patients in clinical practice

STUDY POPULATION

- 50 people with PD who are current patients of study co-investigators
- Subjects followed for 6 months: 5 in-person visits

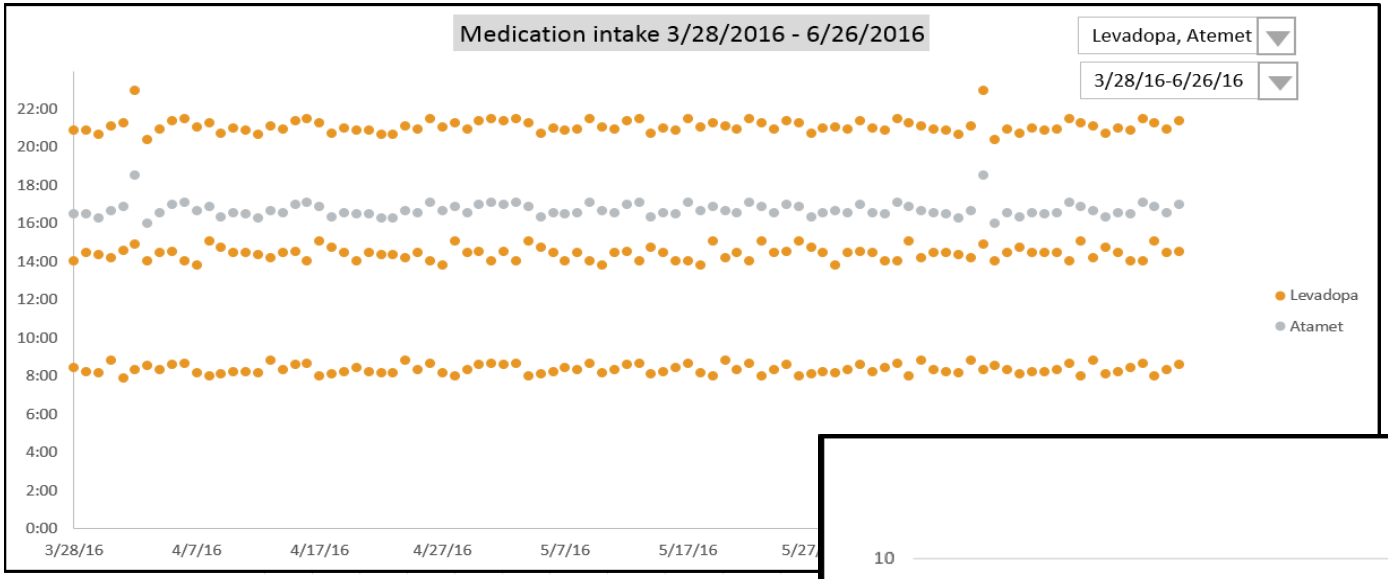
ASSESSMENTS/ DATA COLLECTION

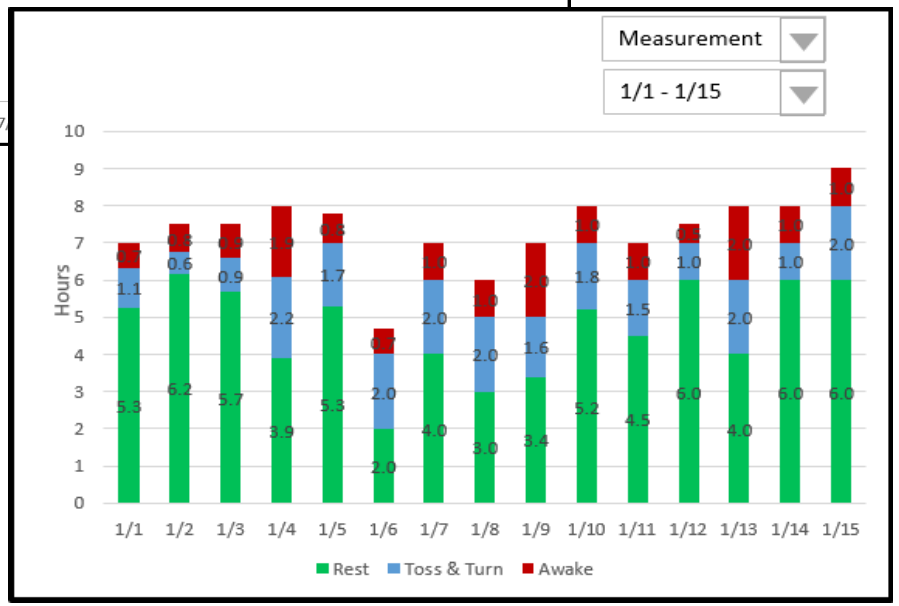
- Motor assessments in OFF/ON states
- Medical history and surveys
- Hauser diary
- In-office and at-home remote monitoring: 24/7 activity level and PD symptoms monitoring

OUTCOMES

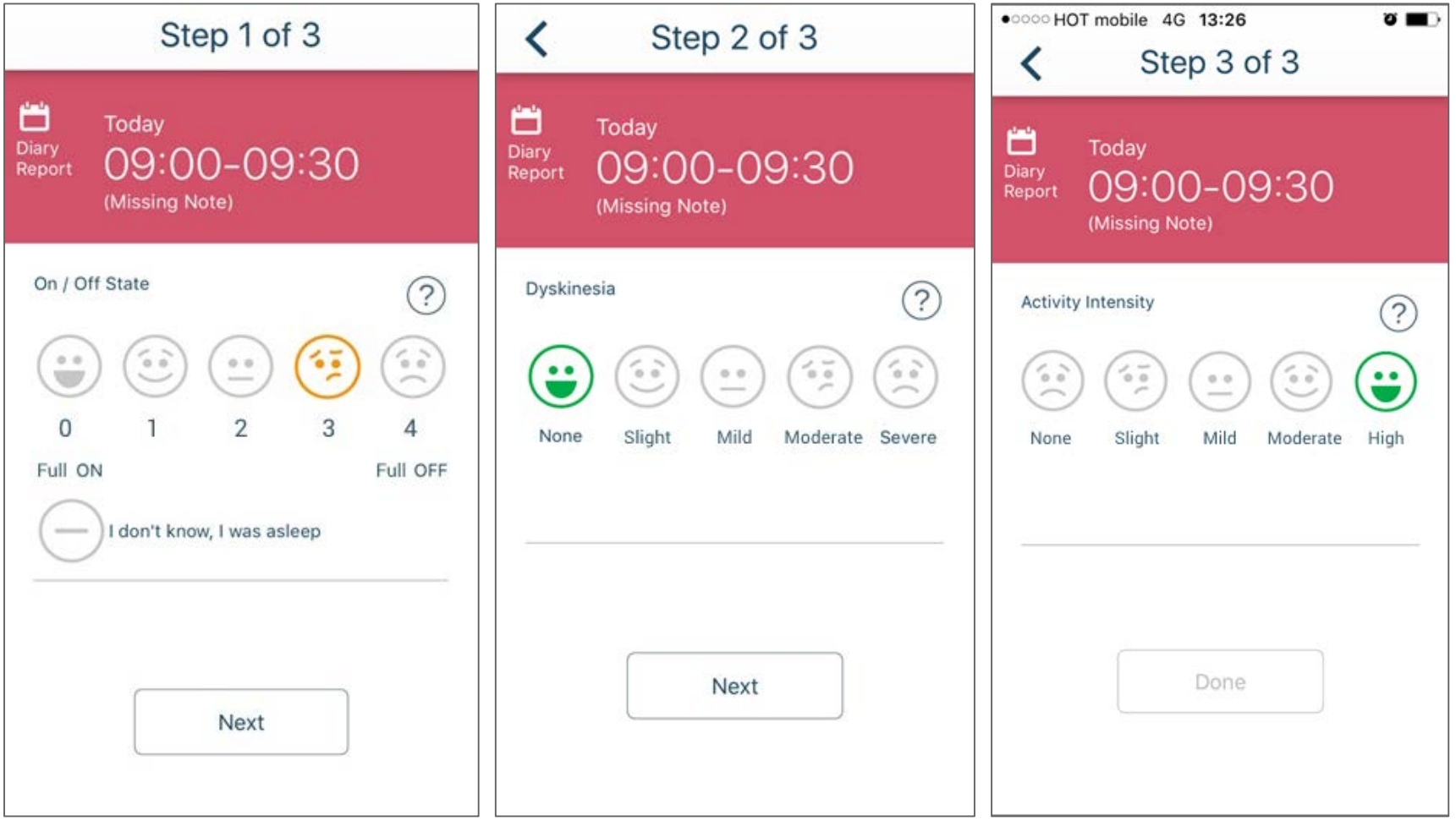
- Develop data dashboard for the clinicians
- Develop electronic ON/OFF diary
- Correlation of FI app's reported measures with patient's reported experiences

CLINICIAN DASHBOARD MOCK-UPS





ON/OFF DIARY


Step 1 of 3
Diary Report
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
Diary Report
Today
09:00-09:30
(Missing Note)
Dyskinesia
None
Slight
Mild
Moderate
Severe
Next
HOT mobile 4G 13:26
Step 3 of 3
Diary Report
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

DATA CHALLENGE

Q1 2017

- The **Levodopa Response Trial** was designed to assess the feasibility of using wearable sensor data to estimate clinically relevant measures of the severity of PD symptoms
  - 30 participants
  - Streamed accelerometer data from two watches on both wrists in lab and home environment; clinical assessments
- **The Michael J. Fox Foundation will share these data and host a challenge to stimulate analysis to determine if sensor data can be used as a biomarker or objective clinical outcome measure**
- Exact parameters and prizes for challenge in development

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

IMPROVED BIOMARKERS AND CLINICAL OUTCOME MEASURES

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.

- Imaging Studies
- Clinical/Physiological Studies
- Biochemical assays
- Data Science

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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