Keeping Up with Consumers:
How can mHealth apps and wearables help evaluate medical products?

mHealth as a Source of Real-World Data (RWD) – Working Group

Duke-Margolis Center for Health Policy

June 26, 2017, 2 – 4pm *EST*

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Housekeeping

- We will be recording this presentation, including any comments or questions from the participants.

- The slides from the presentation as well as the audio recording will be publically posted on the Duke-Margolis Center for Health Policy website: https://healthpolicy.duke.edu/

- To make a comment or to ask a question during the comment periods of this presentation, please type into the Q&A box or use the "raise hand" feature*.
  - Longer comments can be emailed to margolismhealth@duke.edu by July 12, 2017
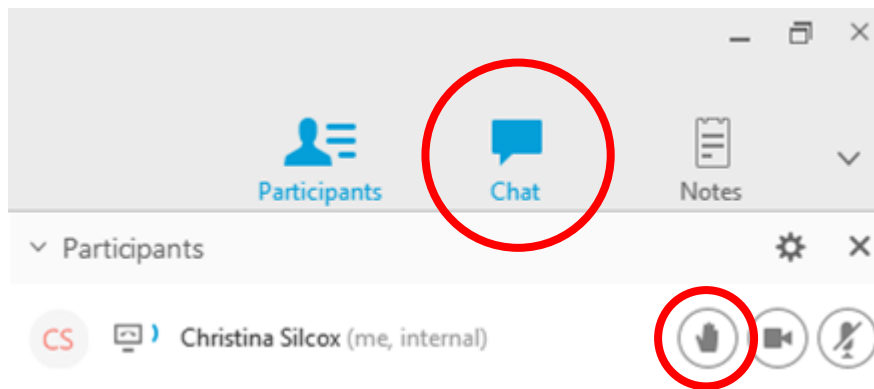
*You can only use the "raise hand" feature if you are using audio through the computer or put in an individual attendee ID when calling in. When we call on you to make your comment, we will unmute you.

Disclosure

Funding for this working group was made possible by the Food and Drug Administration through grant 7U01FD004969. Views expressed in the written materials and by speakers and moderators do not necessarily reflect the official policies of the Department of Health and Human Services nor does any mention of trade names, commercial practices, or organization imply endorsement by the United States Government.

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Webinar Agenda

- Project Scope
- Addressing Needs to Build Engagement
  - What causes patients to regularly and sustainably engage with mHealth apps and wearables?
  - Why would mHealth companies add functionality necessary for evidence generation?
  - How can researchers and mHealth companies work together to promote methods for frequent, real-life, and/or novel measurements?
  - Public Feedback
- Proposed mHealth Data Types
  - Person-Reported Data
  - Task-Based Measures Data
  - Passive Sensing Data
  - Public Feedback
- Next Steps and Closing Comments
  - Public comment period details

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mHealth – Challenges and Opportunities

- Inflection point in the availability and usability of mHealth technologies
  - Empowers patients to participate in their own health
  - Proliferation and advancement of consumer wellness and health technology
  - Development of analytical tools enabling the transformation of data into knowledge
  - NEST can support the use of mHealth as real-world data for the evaluation of medical devices
- Enormous growth in opportunity, but also in the complexity and cost of both healthcare and clinical research.
- Patients are becoming an integral part of how medical products are researched, developed, marketed, and used
- mHealth is a disruptive technology including:
  - Feeding into the next generation of evidence development platforms
  - Regulatory and research policies need to keep up with promising technologies and methods

Project Scope

- mHealth apps and wearables can collect data from patients about their experiences and symptoms, as well as objective data about activities
  - Medical studies often collect similar data (PROs, PerfOs)
  - Opportunity for novel outcome measures important to patients
- Real-world evidence generation and NEST
  - Already being used for consumer and clinical use
- Requirements for research use
  - Accuracy measurements
    - Validation studies in patient group of interest
  - Sustained longitudinal collection
  - Predictability for how the data will be verified, transmitted, and linked

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Outside of the Scope

- Evaluating mHealth apps and wearables as medical devices
  - Does using a mHealth technology in research studies make it a "medical device"?
    - App/wearable is a medical device when it is intended for use in the diagnosis of disease or other conditions, or in the cure, mitigation, treatment or prevention of disease
    - FTC tool to find out when FDA, Federal Trade Commission (FTC) or Office of Civil Rights (OCR) laws apply - https://www.ftc.gov/tips-advice/business-center/guidance/mobile-health-apps-interactive-tool

Connected/Quantified Life in 2024

"Christie" is a 39-year-old female with a history of high blood pressure. She completes her annual physical exam each year. Like most people in 2024, Christie monitors her weight, steps, energy expenditure, sleep, blood pressure, stress, and heart rate through wearable trackers, some of which have been provided through her insurer or employer. She also keeps food- and mood-tracking journals through an app on her phone. Ingestible sensors track her medication adherence.

Christie authenticates her apps and devices so that her PGHD could be fully integrated into centralized software that also records EHR and pharmacy information (such as flu shots received at her local pharmacy and cold medicine prescribed at the community health clinic) without needing individual action from Christie. Within a centralized app, Christie controls who can see and use her information by authorizing and revoking access.

With her permission, algorithms linking data from these multiple sources continuously analyze Christie's health status and alert Christie and/or her doctor's practice if there is a significant concern.

Accenture Draft White Paper for a PGHD Policy Framework
https://www.healthit.gov/sites/default/files/Draft_White_Paper_PGHD_Policy_Framework.pdf

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Leveraging Real-World Evidence
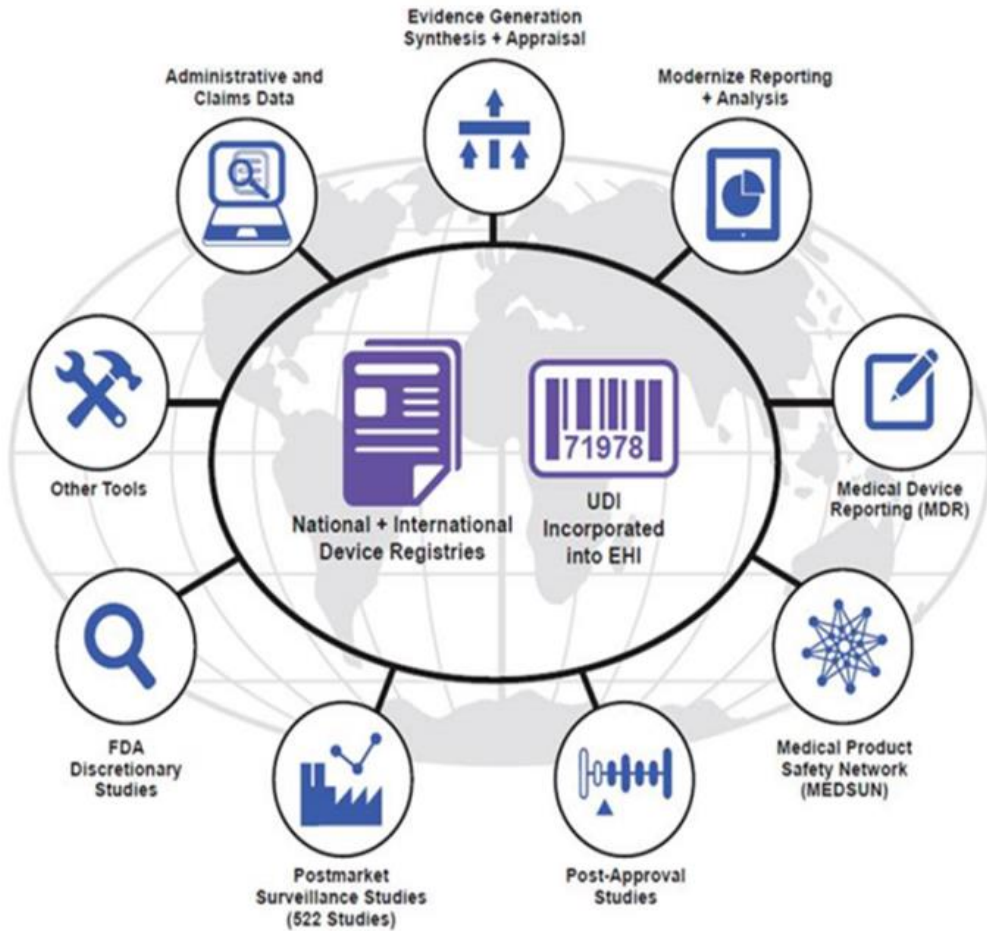
- Types of real-world data (RWD)
  - EHR
  - Claims
  - Registries
  - mHealth

- Efficient linking and analysis of RWD
  - Standardized informed consent guidelines, data use agreements, and data governance practices
  - Common data models and data quality standards
  - Well-characterized methodologies

How do we get from 2017 to 2024?

- What types of mHealth data are useful now?

- What reasonable changes could technology companies incorporate to make mHealth data more useful for evidence generation in the near-term?
  - What challenges are impeding these changes?

- How do we engage people to securely share data for evidence generation?
  - What would meaningful informed consent look like?
  - How can the use of these tools be sustained to ensure the data is useful?

- How can researchers and mHealth companies work together to promote methods for frequent, real-life, and/or novel measurements?
  - What information about the validity and reliability of mHealth technology must be characterized for the data to be used for evidence generation?
  - What makes an off-the-shelf mHealth solution more attractive than a proprietary solution designed for a specific study?
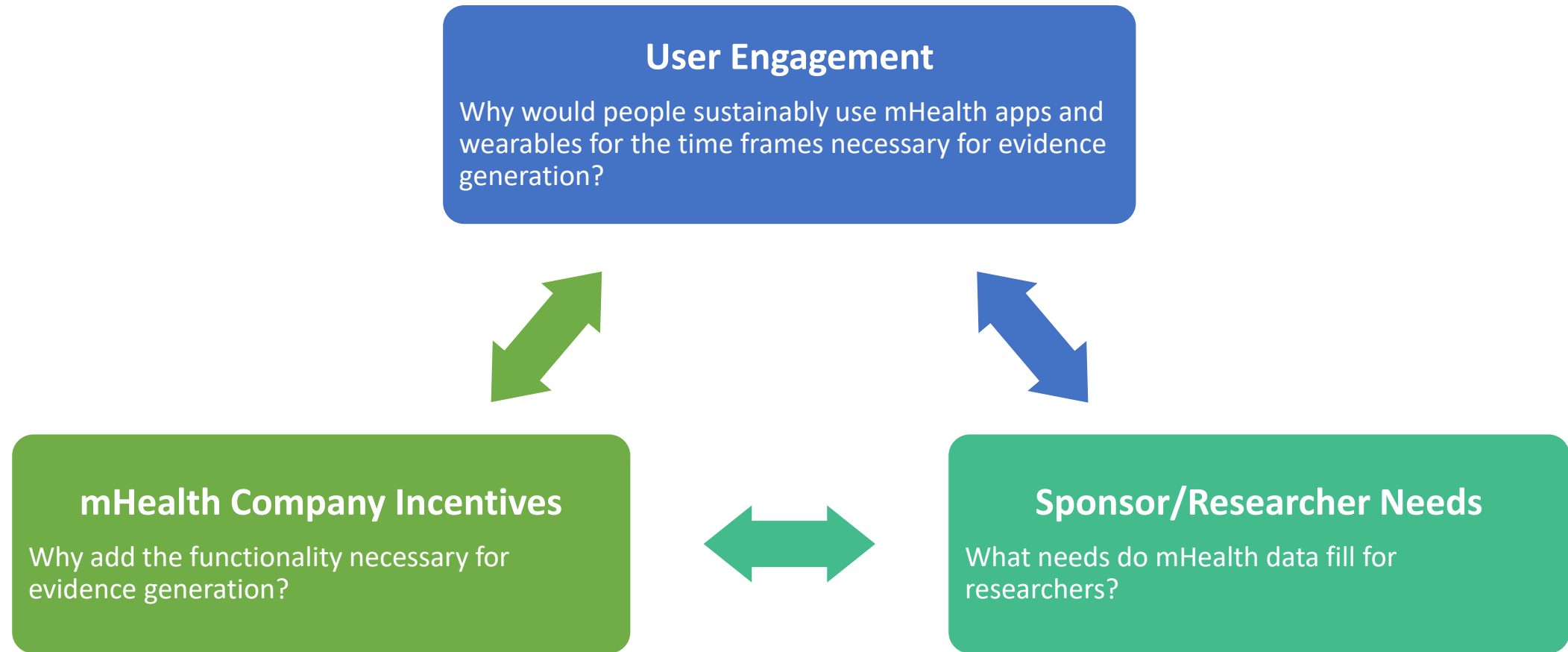
What will the Action Plan address?

- Contribution of mHealth data for novel real-world evidence generation
  - Role of NEST in encouraging the inclusion and use of mHealth in evidence development for medical devices

- Ways to think about different types of person-facing mHealth technologies, software, and data

- Recommendations for advancing person-facing mHealth adoption and usage
  - User engagement
  - Researcher/sponsor needs
  - mHealth company incentives

- Overarching challenges in digital health data: Current work and resources
  - Best practices for patient-consumer informed consent
  - Data linkages and interoperability
  - Accommodating diversity of wearable technology and application
  - Fit-for-purpose: validation and reliability of data

- Recommended next steps to advance consumer/clinical mHealth technologies as a viable source of reliable data for evidence generation

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- Addressing Needs to Build Engagement
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  - Public Feedback
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Balancing Stakeholder Benefits

User Engagement

Why would people sustainably use mHealth apps and wearables for the time frames necessary for evidence generation?

mHealth Company Incentives

Why add the functionality necessary for evidence generation?

Sponsor/Researcher Needs

What needs do mHealth data fill for researchers?

User Engagement

User engagement is necessary for individuals, and/or their care givers, to start and maintain use of mHealth technology

- What characteristics of mHealth apps and wearables encourage sustained usage?
- How can we ease the transition from being a user of mHealth into being a research participant?

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User Engagement – What should the action plan highlight?

1. Approaches to cultivate sustainable usage
   - Easy to use
   - Actionable information to support ongoing input in their own health status and care
     - Personal wellness management
     - Integration with clinical information in ways that assist clinicians with shared decision-making
   - Person-centred design
   - Security and privacy
   - Emphasis of value to society (i.e., altruism)
2. Features of successful mHealth apps and wearables
3. Potential implications for evidence generation from features meant to increase user value (e.g., biasing)
4. Recommendations to bridge transition from "user" to "research participant"

Real World User Engagement

• From patientslikeme.com

"Without our voices, things would remain status quo."

- Cris, member living with ALS

Learn More

*From* https://mhealth.jmir.org/2017/2/e14/

- *I very much like participating. I feel as if I am helping to reach an overall outcome*

- *I lost interest/motivation and stopped recording for a while...*

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THREAD™

Engagement Predictor Tool

Motivation Factors

Financial incentives

Clinical benefit

Altruistically fulfilling

Enjoyable (education or entertainment)

Passive data collection
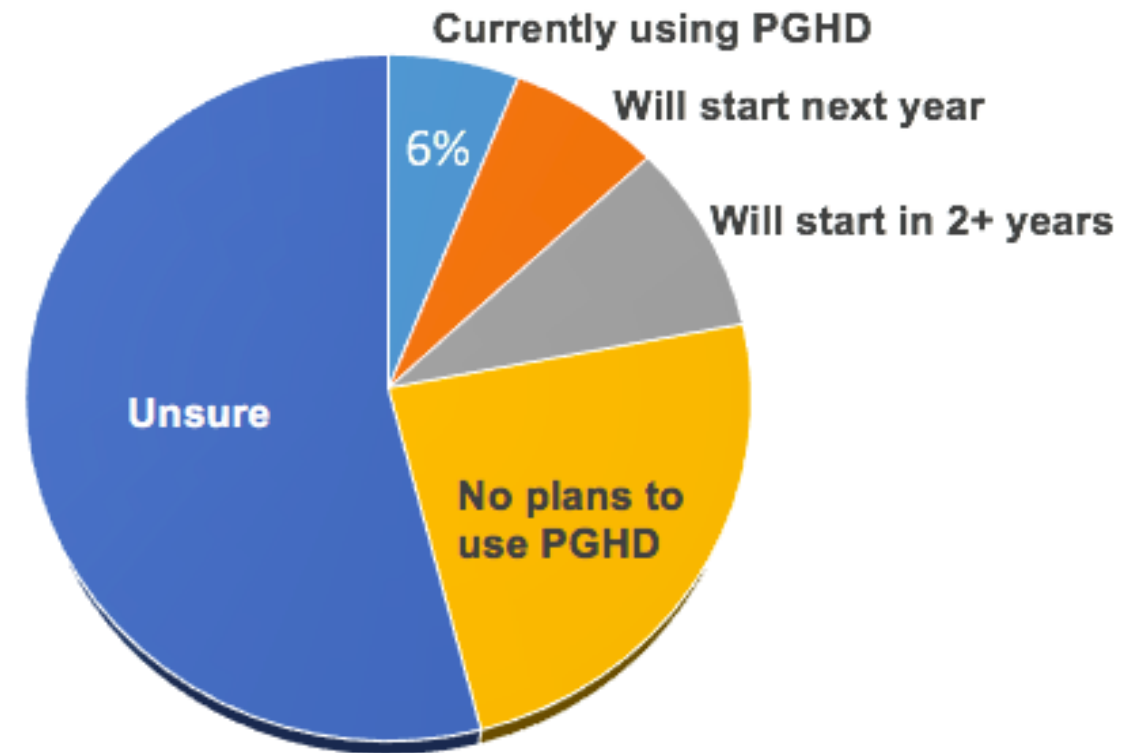
Added value to daily living
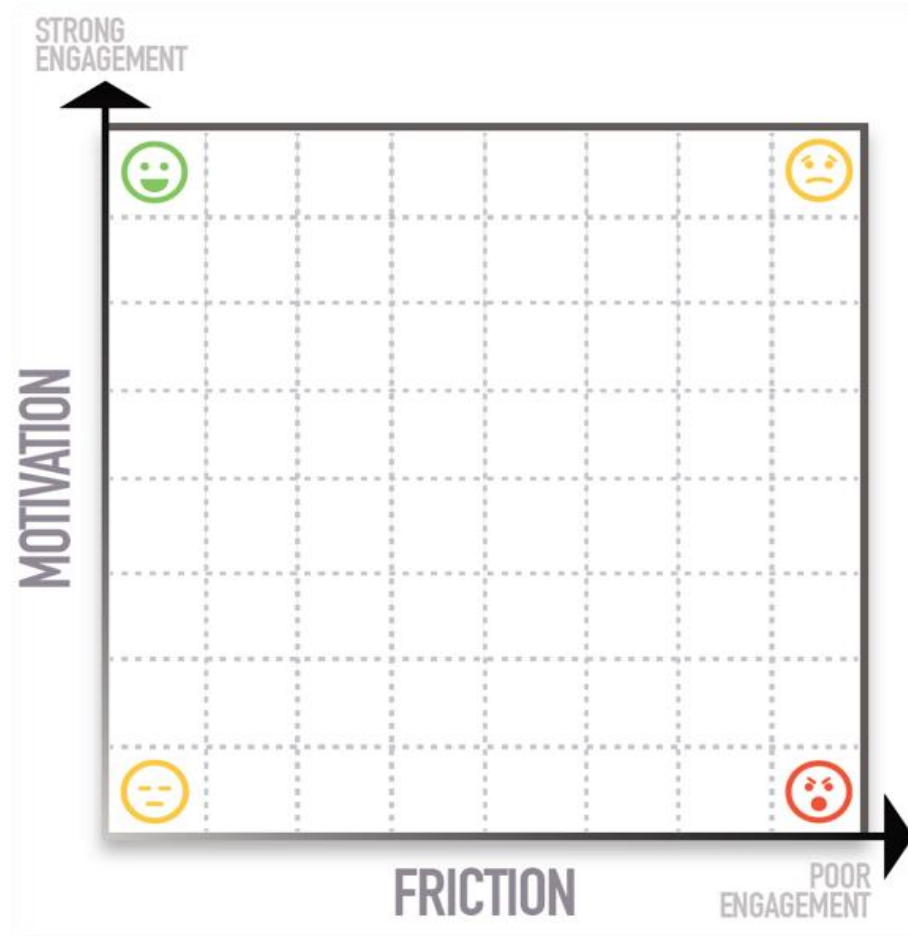
Friction Factors

Interaction frequency

Duration of interaction

Convenience of interaction

Cognitive load requirements

Active data collection

Reminders / notifications

STRONG ENGAGEMENT

MOTIVATION

FRICTION

POOR ENGAGEMENT

Clinical Use of Patient Generated mHealth Data

Do clinicians use patient generated mHealth data (PGHD) in clinical decision-making?

NO
- Only 6% currently use PGHD in clinical decision-making.
  - Majority use the information as part of patient engagement in health status and activity goals
  - Few received data directly from a device

- 81% do not use wearable data from patients for clinical decision-making.

Currently using PGHD
6%
Will start next year
Will start in 2+ years
Unsure
No plans to use PGHD

Leventhal R. *MGMA Poll: Providers Not Yet Using Data from Patients' Wearables*. Healthcare informatics. June 20, 2017. Retrieved from https://www.healthcare informatics.com/news item/patient engagement/mgma poll providers not yet using data patients wearables

And How Are People Using PGHD?

Survey by Klick Health of 1012 US adults

2017 KLICK HEALTH CONSUMER SURVEY
HEALTHCARE INNOVATION

70% say technology will help them personally manage their health

	AVG. TOTAL	MALE	FEMALE	AGE 18-34	AGE 35-54	AGE 55+
Help me stay healthy	24%	26%	21%	29%	23%	19%
Help me better manage my condition	15%	12%	17%	10%	15%	18%
Help me locate the best treatment options	10%	10%	10%	8%	9%	13%
Help me provide better care to a loved one	3%	3%	2%	4%	3%	1%
Help me prevent sickness or disease	14%	12%	15%	14%	15%	13%
Help me communicate with others about my health condition	4%	3%	5%	6%	4%	2%
Help me locate the best healthcare professionals	3%	4%	3%	4%	3%	3%
Help my physician or caregiver(s) better empathize with my condition	2%	3%	1%	2%	2%	3%
Help my physician provide earlier diagnosis	14%	13%	15%	11%	17%	14%
Other	1%	1%	2%	0%	1%	3%
It won't have a positive impact on my health	10%	12%	9%	12%	9%	11%

Q. Where do you think technology will have the most positive impact on your health in the future?

41% use mHealth technology NOW to manage their health

How do we Bridge the PGHD Gap?

- 70% of people want to use PGHD to manage their health

- Only 6% of clinicians use PGHD in clinical decision making

- PGHD gap
  - Solve for the right user needs
  - Incentivize mHealth companies to develop the right tools
  - Address sponsor/clinician barriers to adopting PGHD

mHealth Company Incentives

What *incentives* are necessary for *mHealth companies* to intentionally design their products to have the capability to collect and share data to support secondary evidence generation?

- The types of mHealth technologies of interest (including mobile apps, wearables, sensors, etc.) record data directly from patients and can characterize the accuracy and reliability of that data

mHealth Company Incentives – What should the action plan highlight?

1. Potential market opportunities for mHealth technologies to support evidence generation
   - Emerging markets and payment models (e.g., employer wellness programs, insurance programs)
   - Characteristics of payer and delivery systems that promote effective partnerships

2. Current challenges for mHealth Technology
   - Lack of interoperability and data standards
   - Regulatory concerns (privacy, data security, SaMD)

3. Lessons learned from mHealth technologies developed for different markets/uses (e.g., specifically for clinical trials)
   - Identifying approaches that add value to consumers as well as collect appropriate data

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Does Size Affect Incentives?

Large multinational technology corporations

- Trusted known company

- Optics and public relations (e.g., branding)

- Using their size and broader market presence to:
  - Balancing extended development timelines (e.g., access to existing capital)
  - Manage risk
  - Leverage skills and expertise

Start-ups and small companies

- More likely to embrace open source and open innovation in non-competitive areas

- Access to incubator and/or accelerator programs to support startups

- Digital health remains a growing vertical for capital infusion

- Connectivity with other apps and/or platforms enhance usability and offer new value to end-users

Shared opportunities

- Markets interested in disrupting healthcare with digital and consumer-facing solutions
- Fostering altruism contributing to better health and care in society by creating better services
- Partnering with life sciences/healthcare companies allows innovators to leverage their experience in regulatory, data privacy, etc.

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Sponsor/Researcher Needs

- *Sponsor/Researcher needs* must be defined in order to understand how the relative data needs of organizations conducting and/or sponsoring research on medical products actively incorporating mHealth data can differ depending on purpose, as well as what novel outcome measures based on mHealth would be useful to them.
  - "Sponsors" include any organization that is funding evidence generation (e.g., medical device and drug manufacturers, clinical societies, patient organizations, hospitals, employers and payers).
- Potential Use Cases
  1. Regulatory decision making
  2. Quality measurement
  3. Value-based payment models
  4. Novel Outcome Measures
  5. Evidence generation for shared decision-making models

Sponsor/Researcher Needs – What should the action plan highlight?

1. Existing mHealth technologies capable of collecting data appropriate for evidence generation
2. Opportunities for improved collection and sharing of data
   - Convenience and accessibility of mobile phones makes them ideal for data collection in the field
   - Improved and more representative recruitment by reducing time and travel costs associated with the study
3. Lessons learned from other mHealth areas (e.g., clinical support, medical device regulation, clinical trials implementation)
   - Potential to bias data through user engagement features (e.g., progress tracking could encourage some people and discourage others)
   - Large amounts of data require increased level of expertise and labor needed to analyze data (e.g., high drop-out rates, missing data)
4. Relative needs on data quality, specificity, validation methods, and longitudinality for different purposes for mHealth data

What Affects "Fit-for-Purpose"?

The utility of mHealth data for secondary research is highly dependent on the study objective, design, and other data types

- Use of the data
  - Outcome measure
    - Primary
    - Secondary
    - Ancillary
    - Exploratory
  - Exclusion/Inclusion criteria
    - Recruitment
  - Characterizing subgroups
    - Analysis
    - Risk-adjustment

- Type of Study
  - Regulatory
    - Premarket clearance/approval of medical products
    - Postmarket studies
    - Postmarket surveillance
    - Quality Assurance/Quality Control
  - Quality Improvement/Clinical Guidelines
  - Predictive analytics for high-risk identification
  - Comparative effectiveness
  - Clinical practice research studies

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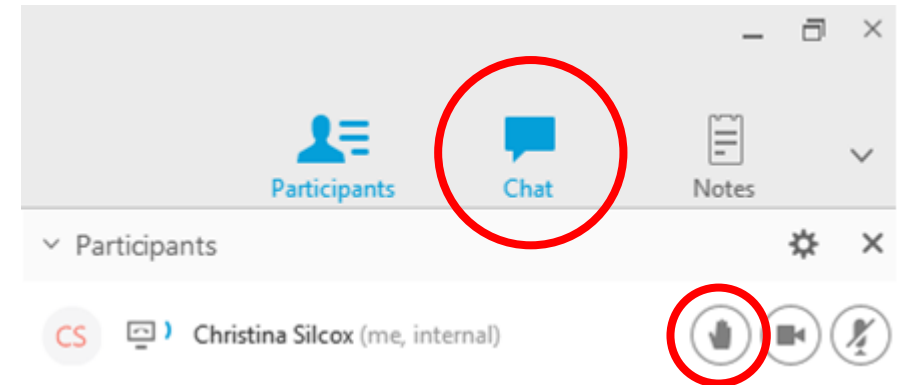
Demystifying "Validation"

- What information is most helpful?

- Where is accuracy needed?

- Who is responsible for validation?
  - Accuracy in healthy/target population
  - Validation in specific disease population
  - Surrogate measure validation (often a medical claim)

- Validation is dependent on use (fit-for-purpose)

Validation of a mHealth Surrogate Marker

- A secondary analysis of Multiple Sclerosis (MS) patients
  - 786 persons with Multiple Sclerosis (MS)
    - 157 healthy controls.
  - Participants
    - wore an accelerometer or pedometer over a 7 day period during waking hours
    - provided demographic information
    - People with MS also reported:
      - MSWS-12 (Multiple Sclerosis Walking Scale-12)
      - PDDS (Patient Determined Disease Steps) scales
      - Other clinical and health information

- Conclusion
  - Change in motion sensor output of 800 steps/day represents a lower-bound estimate of clinically meaningful change in free-living walking behavior in interventions of MS

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Public Feedback

- Please use the "raise hand" feature next to your name or the chat feature
- Duke-Margolis also welcomes written comments on these topics.
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Potential mHealth Data Types

User-Reported Data
What people say

Task-Based Measures
Measures effort and physiology

Passive Sensing
What people actually do day to day

User-Reported Data

- Data reported manually by the person themselves (or their caregiver if the person is unable to enter the data)
- Examples include:
  - Questionnaires/surveys
  - Symptom tracking
  - Person-reported outcomes
  - Patient diary entries
- The data could be but is not limited to a validated outcome measure (i.e. Patient-Reported Outcome Measure or PROM)
  - Historically captured through paper-based approaches, web surveys, phone calls, etc.
- Could be collected through mHealth apps (devices can be given out for the study or users can download apps onto their own device)

Task-Based Measures

- Objective measurement of a person's mental and/or physical ability to perform a test consisting of a defined task or set of tasks

- Examples include:
  - Physical functioning (e.g., 6-minute walk test)
  - Cognitive functioning (e.g., digital symbol substitution)
  - Physiological tests performed by the user (e.g., glucose self-measurements)

- Typically collected in a clinical setting with appropriate clinical/task procedure validation

- Could be collected through remote sensors and/or mobile apps which may utilize smartphone hardware, but would require specific instruction and confirmation the task was performed

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Passive Sensing

- A measurement of a person's daily activities/mental state, where the measurement does not interrupt the person's normal activities (i.e. it measures what the person actually does in daily life)

- Could be collected through wearable and remote sensors, mobile devices/apps and other tools that monitor behavior (e.g., social media)

mPower helps decipher Parkinson's disease.

The variability in Parkinson's disease symptoms has left many questions unanswered. So the University of Rochester and Sage Bionetworks created the mPower app to precisely measure data such as dexterity, balance, memory, and gait. This information could help researchers better understand how various symptoms are connected to Parkinson's disease. In turn, participants could start to recognize their own signs and symptoms.
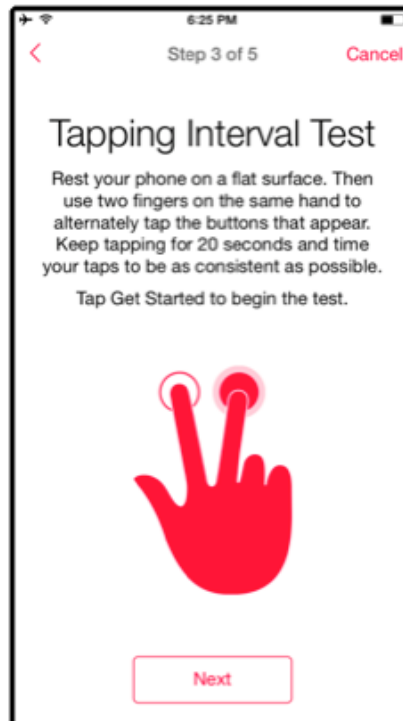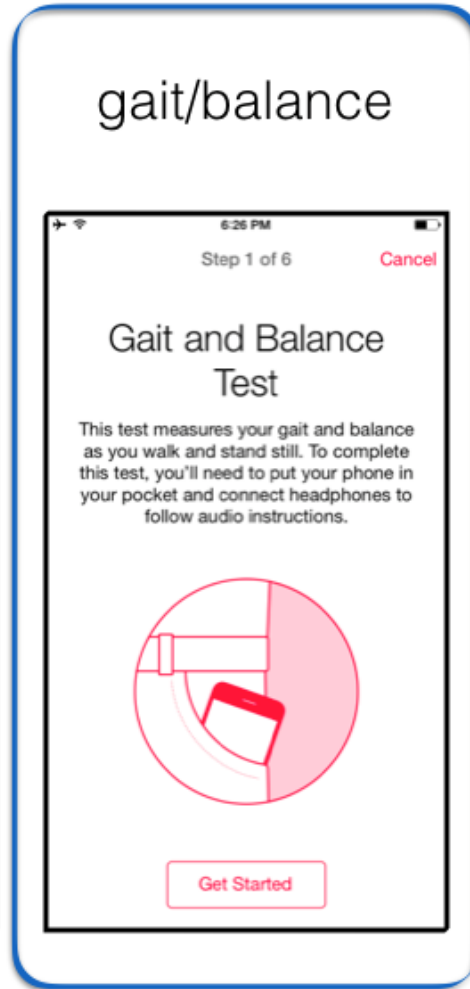
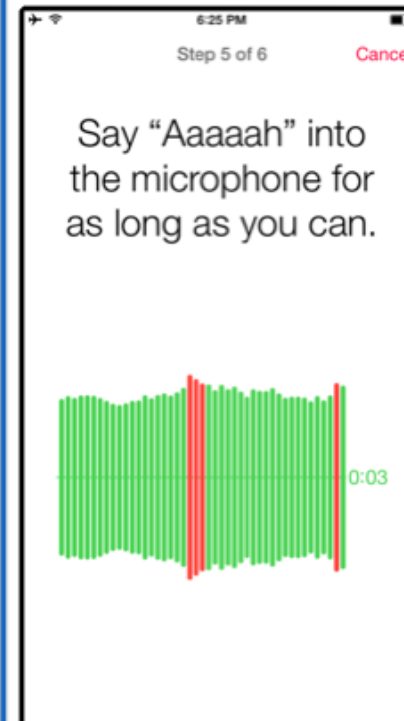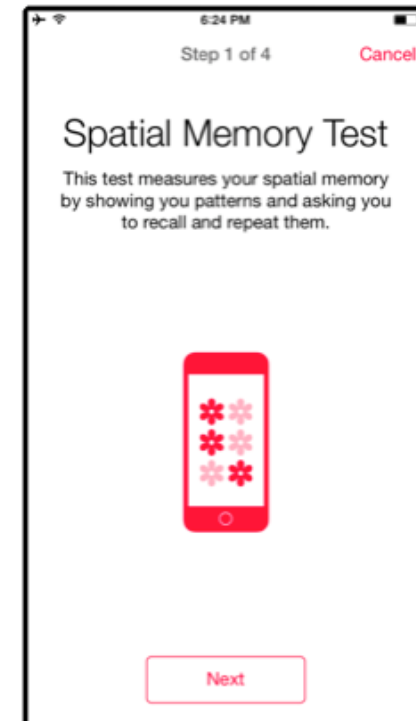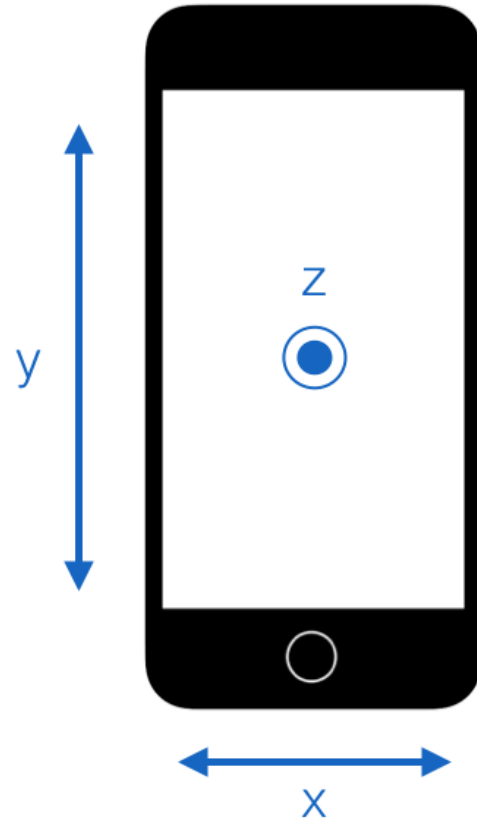|	motor initiation	gait/balance	hypophonia	memory
passive measures	gps - displacement vectors	gps - displacement vectors	-	-
structured activities	tapping activity	walking/ standing activity	voice activity	memory game
surveys	MDS-UPDRS PDQ8	MDS-UPDRS PDQ8	MDS-UPDRS PDQ8	MDS-UPDRS PDQ8

motor initiation

gait/balance

hypophonia

memory

Tapping Interval Test

Rest your phone on a flat surface. Then use two fingers on the same hand to alternately tap the buttons that appear. Keep tapping for 20 seconds and time your taps to be as consistent as possible.

Tap Get Started to begin the test.

Next

Gait and Balance Test

This test measures your gait and balance as you walk and stand still. To complete this test, you'll need to put your phone in your pocket and connect headphones to follow audio instructions.

Get Started

Say "Aaaaah" into the microphone for as long as you can.

0:03

Spatial Memory Test

This test measures your spatial memory by showing you patterns and asking you to recall and repeat them.

Next

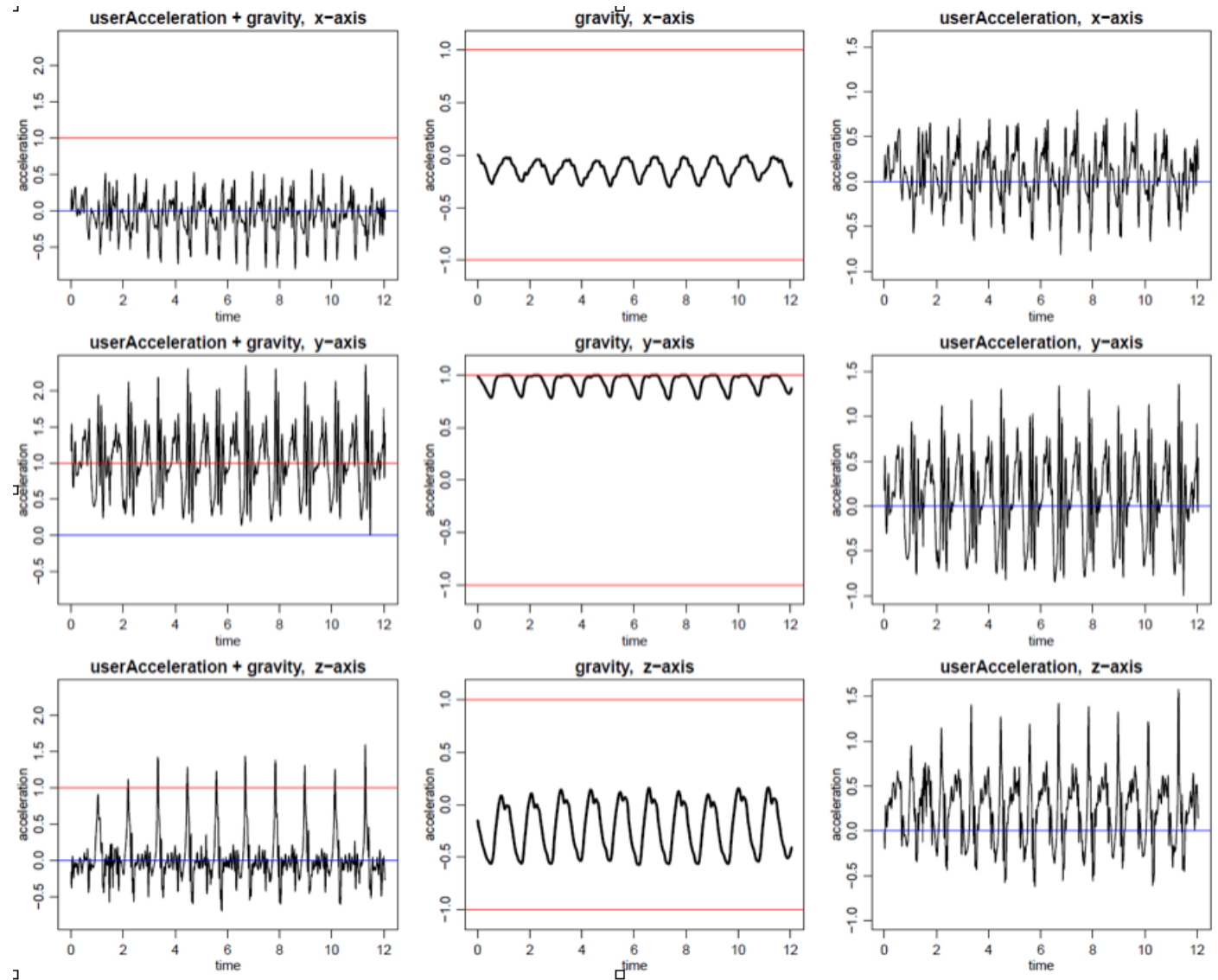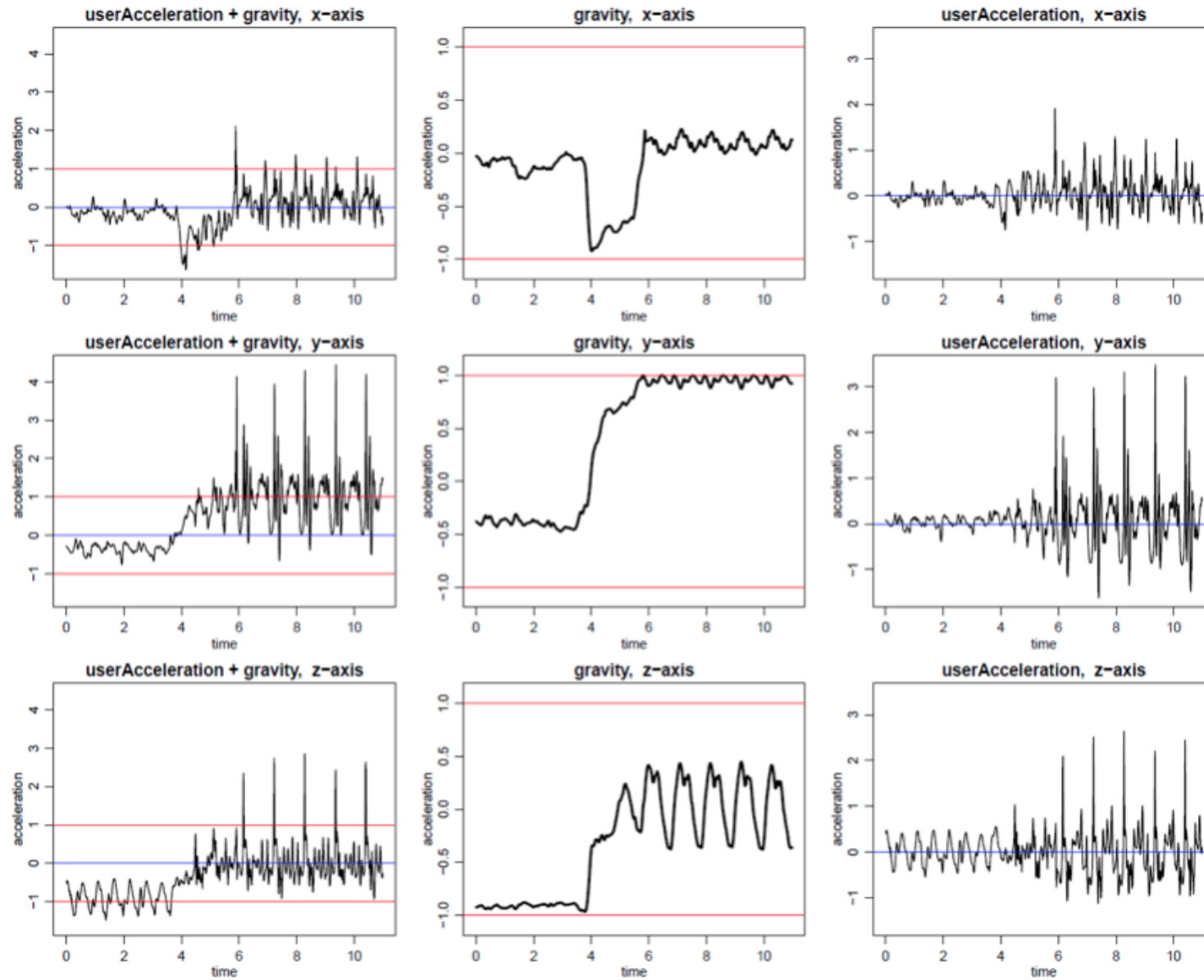**Duke** | **MARGOLIS CENTER** *for* **Health Policy**

gait / balance

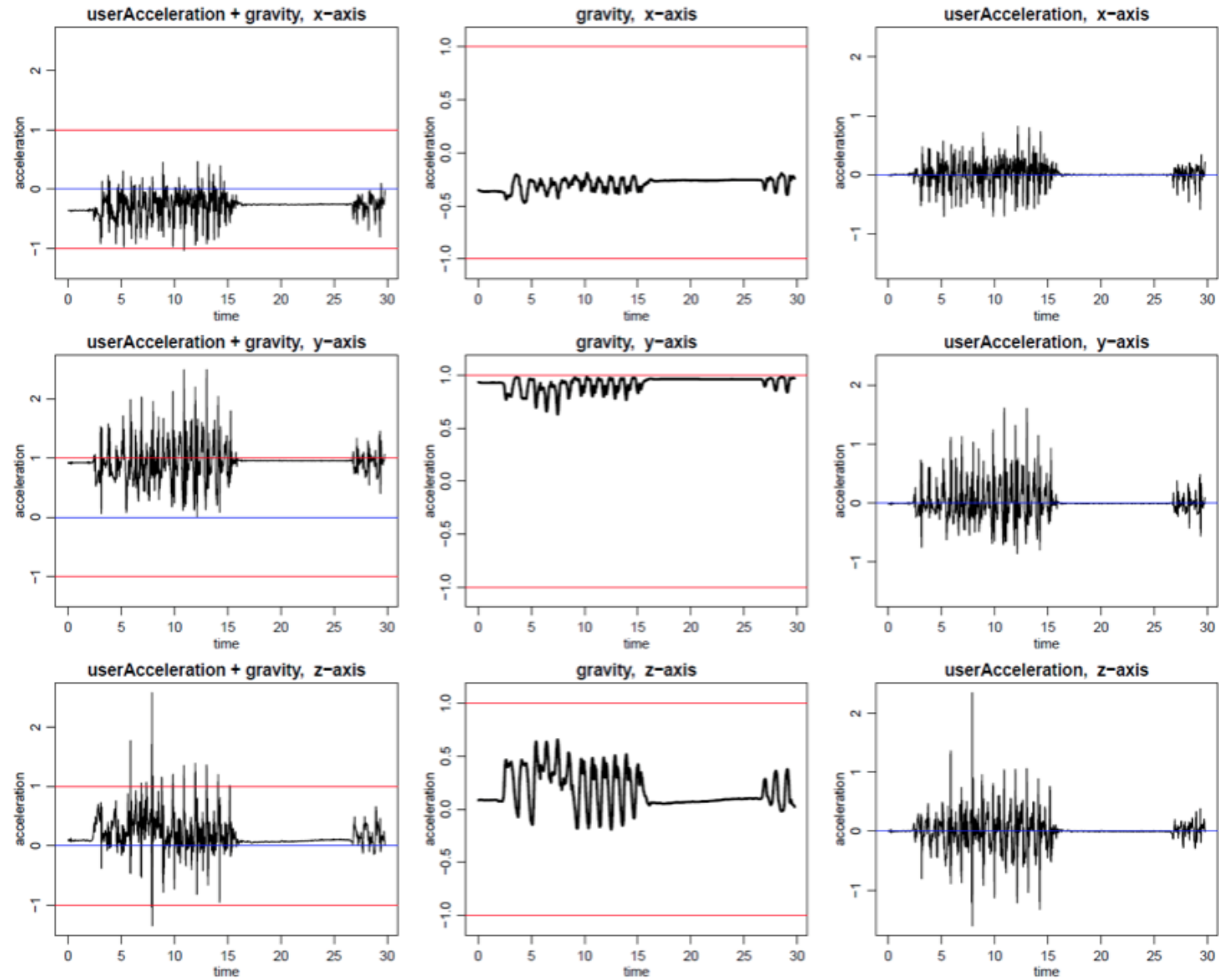userAcceleration

gravity

rotationRate

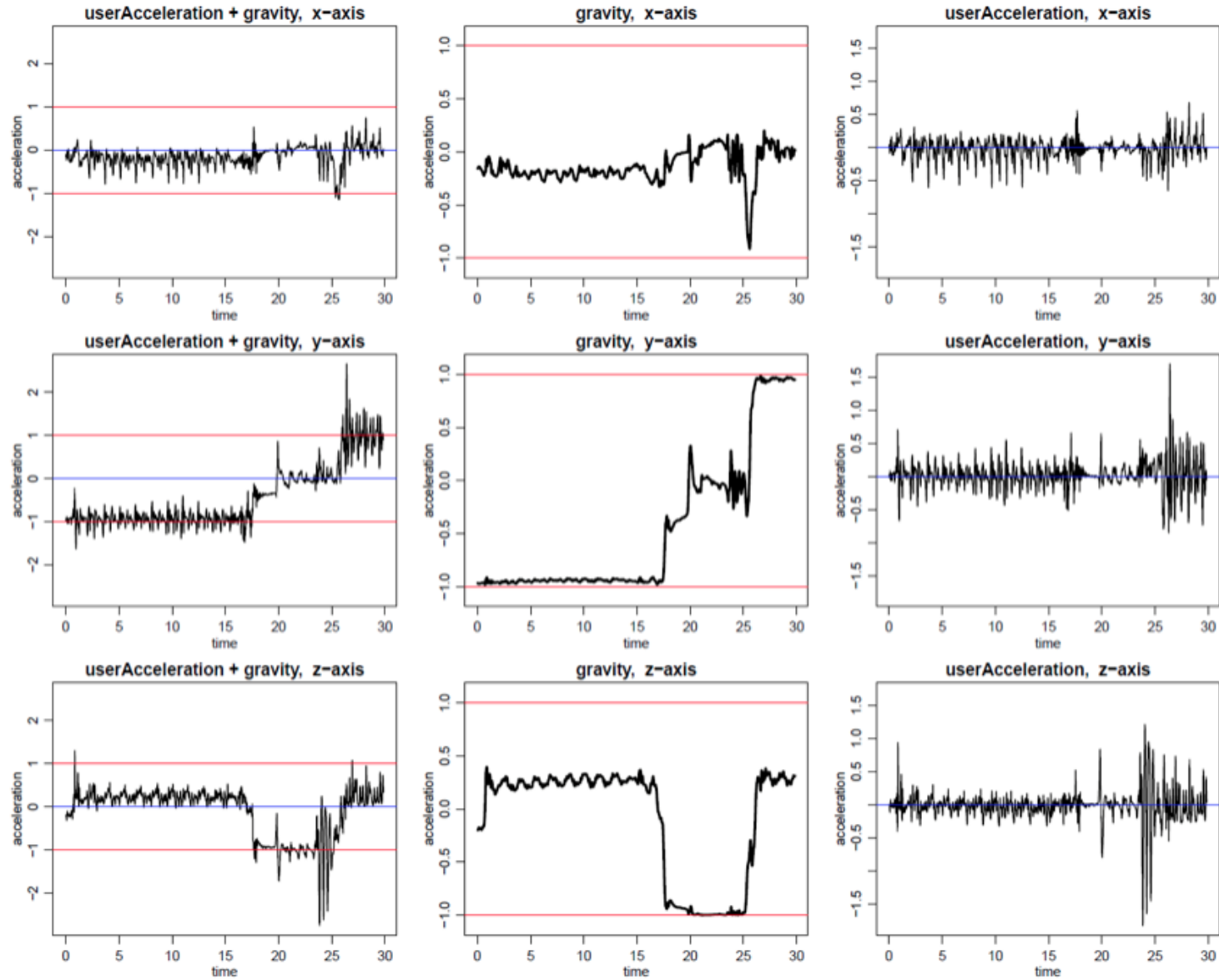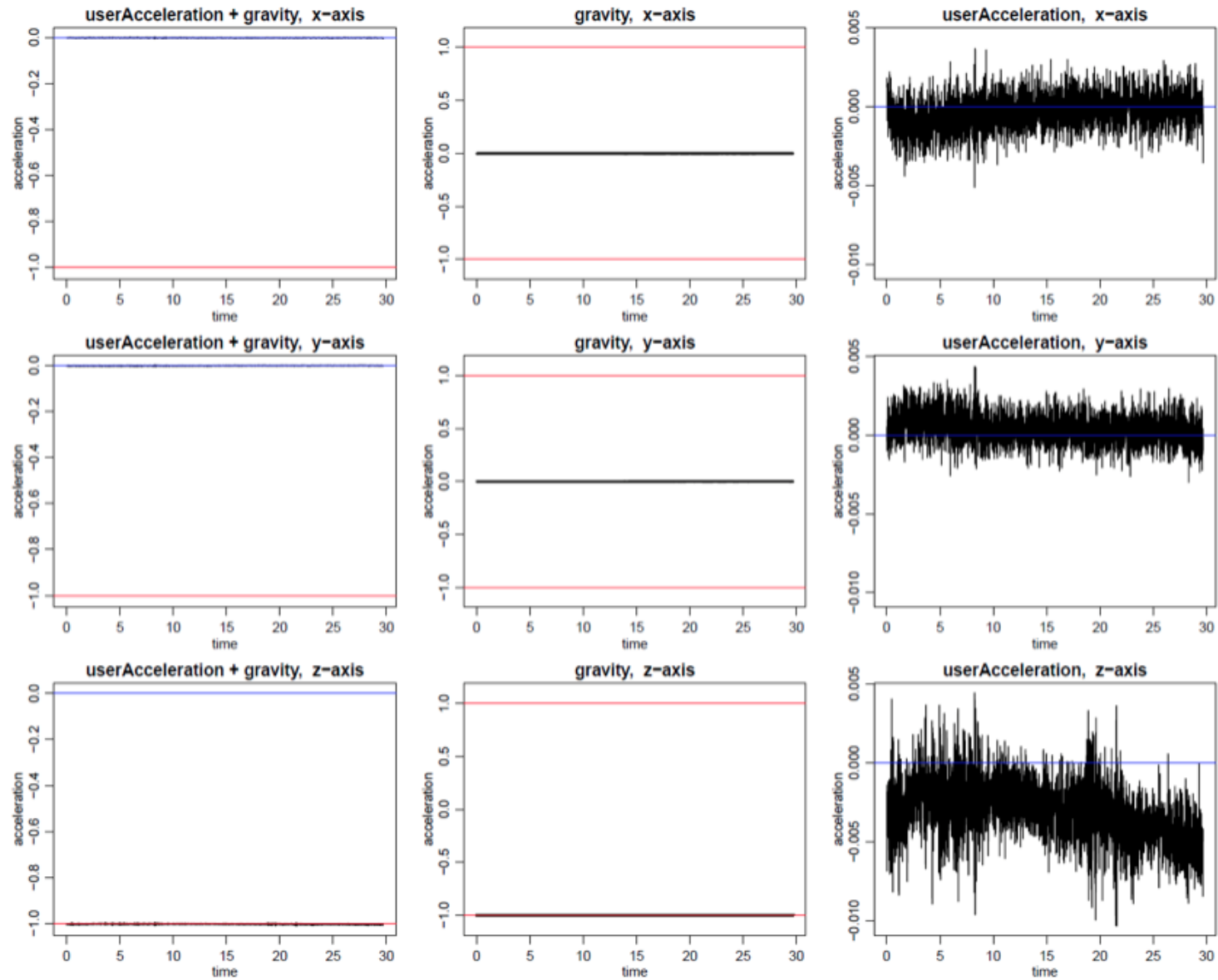attitude
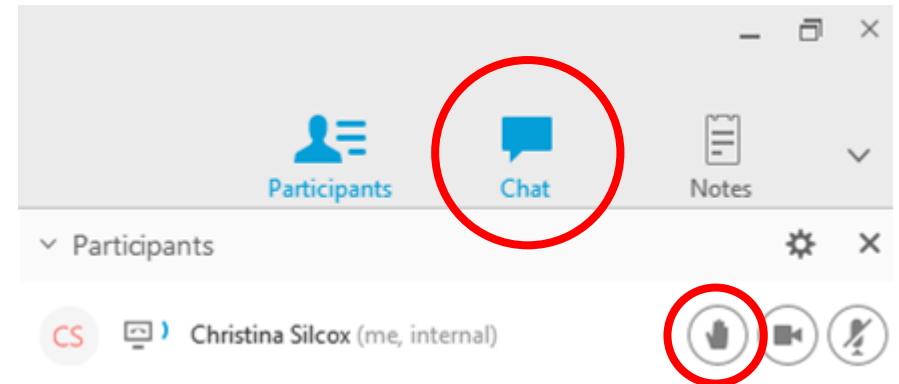
device motion readings at 100 hz

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What will the Action Plan address?

- Contribution of mHealth data for novel real-world evidence generation
  - Role of NEST in encouraging the inclusion and use of mHealth in evidence development for medical devices

- Ways to think about different types of person-facing mHealth technologies, software, and data

- Recommendations for advancing person-facing mHealth adoption and usage
  - User engagement
  - Researcher/sponsor needs
  - mHealth company incentives

- Overarching challenges in digital health data: Current work and resources
  - Best practices for patient-consumer informed consent
  - Data linkages and interoperability
  - Accommodating diversity of wearable technology and application
  - Fit-for-purpose: validation and reliability of data

- Recommended next steps to advance consumer/clinical mHealth technologies as a viable source of reliable data for evidence generation

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Next Steps

- Public Comment period through July 12, 2017
  - Please send your thoughts to margolismhealth@duke.edu
  - Comments will be shared with the working group but will not be made public.
  - The webinar slides and recording will be posted to the Duke-Margolis website within 48 hours.

- Action Plan Recommendations - Public Release Meeting
  - September 15, 2:00-4:00 pm EST
    - In-person at our DC office and webcast
  - Details will be posted on the Duke-Margolis website or you can sign up for event notifications at https://healthpolicy.duke.edu/newsletter

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