Advancing the Appropriate Use of Mobile Clinical Trials: The Clinical Trials Transformation Initiative

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CLINICAL TRIALS **TRANSFORMATION** INITIATIVE

October 19, 2016

Agenda

- Introduction to CTTI
- CTTI Mobile Clinical Trials Program Overview
- CTTI Mobile Clinical Trials Projects
  - Use of Mobile Devices
  - Novel Endpoints
  - Legal and Regulatory Issues
  - Stakeholder Perceptions

Introduction to CTTI

CTTI Overview

CLINICAL TRIALS **TRANSFORMATION** INITIATIVE

To develop and drive adoption of practices that will increase the quality and efficiency of clinical trials

Public-Private Partnership
Co-Founded by FDA and Duke
involving all stakeholders
80+ members

CTTI

CTTI Membership

*Version: September 26, 2016

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PROJECT PORTFOLIO *September 2016*	**Systematic approach to evidence generation including use of non-traditional CT data sources & technical innovations**	**Patients as equal partners across the R&D continuum**	**CTs designed with a focus on efficiency & quality**	**Trials that address emerging public health concerns**	**Safe & ethical trials that are streamlined**
Project Recommendations/ Findings Complete	Large simple trials		GCP training Monitoring Quality by Design Recruitment Site metrics	Antibiotic Drug Development (ABDD): • Streamlining HABP/VABP trials Opioid	Central IRB (2) DMCs Informed Consent Safety reporting (3)
Active Projects	Registries State of clinical trials **Mobile in Clinical Trials Program:** • **Mobile Devices** • **Legal & Reg** • **Novel endpoints** • **Stakeholders**	Patient Groups & Clinical Trials	Investigator turnover GCP follow-on (Qualified investigators)	ABDD: • Pediatric clinical trials • Pilot studies • Unmet need	Pregnancy Testing
Completed Collaborations	Uses of electronic healthcare data		Clinical trial survey CV endpoints Investigator training course Patient engagement survey		
Active Collaborations	Supporting IMPACT-AFib			ABDD PTN	

Mobile Clinical Trials Program Overview

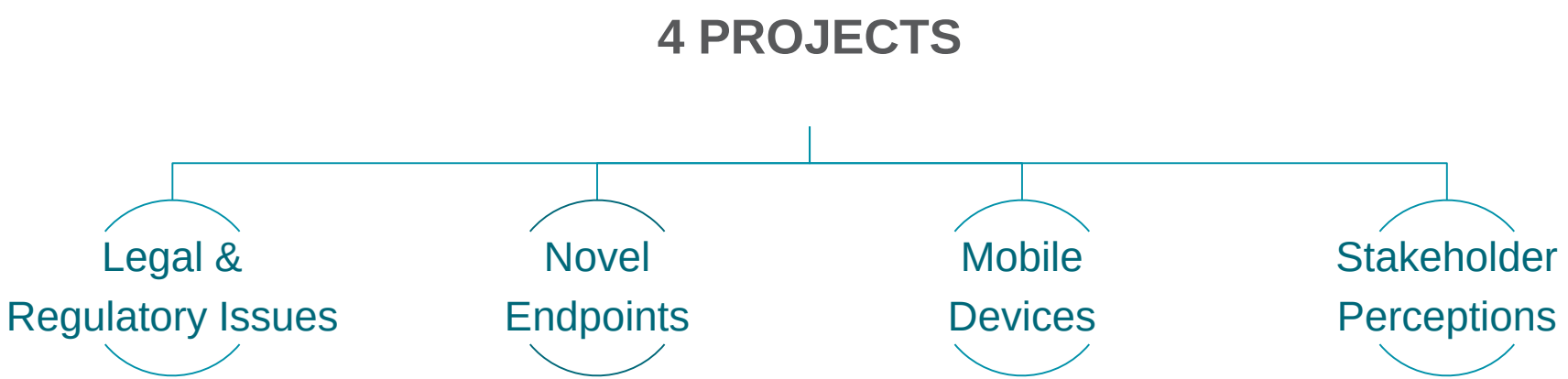
Mobile Clinical Trials (MCT) Program

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



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

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Scientific and Technological Issues Surrounding the Use of Mobile Devices in Clinical Trials

Project Team

Team Leaders	Team Members	Project Manager
Marissa Bolognese (The Life Raft Group) Phil Coran (Medidata) Ray Dorsey (URMC) Cheryl Grandinetti (FDA) Seleen Ong (Pfizer) Kaveeta Vasisht (FDA)	Aiden Doherty (University of Oxford) Ashish Naryan (Northwell Health) Jonathan Helfgott (Stage 2 Innovations) Jane Shen (PMG Research) Matt Kirchoff (NIH) Chris Miller (AstraZeneca) Tom Switzer (Genentech) Adam Amdur (Sleep Apnea Assoc) Dharmesh Patel (FDA) Phillip Kronstein (FDA) Barry Peterson (Philips) Drew Schiller (Validic)	Jen Goldsack (CTTI)

Project Purpose:

- Propose recommendations that address the scientific and technological challenges related to applying mobile devices in clinical trials.

Project Objectives:

- Identify solutions to the data challenges associated with using mobile devices in clinical trials.
- Identify and describe the scientific and technological considerations associated with managing mobile devices for use in clinical trials and develop guiding principles to promote their inclusion.

Issues are Evidence Based

- CTTI Expert Meeting (November 2015)
- Analysis of response to FDA public docket on “Using Technologies and Innovative Methods to Conduct FDA-Regulated Clinical Investigations of Investigational Drugs” (December 2015)
- Review of pertinent guidance documents (“Old made new”)
  - Part 11, Electronic Records; Electronic Signatures — Scope and Application
  - General Principles of Software Validation
  - eSource Data in Clinical Investigations
  - Critical Path Innovation Meetings
  - Use of Electronic Informed Consent in Clinical Investigations
  - Computerized Systems Used in Clinical Investigations
  - Mobile Medical Applications

Scientific and Technological Issues Surrounding the Use of Mobile Devices in Clinical Trials

- Topics and issues we are addressing in this project

Data Challenges	Scientific Considerations
• Data origins & source data • Data integrity • Data collection • Data attribution • Study Monitoring • Data security • Data analysis • Data validation • Audit trail • Data reproducibility	• Providing real-time data to study participants • Monitoring outcomes • Real time safety signals
	Technological Considerations
	• Device validation • Calibration • Device management • BYOD • Device failure • Device reuse

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Primary Products

- Specific recommendations on how to effectively use mobile devices in clinical trials
- Tool kits
  - Guidance for selection of appropriate devices
    - Primarily addressing ‘technological considerations’
  - A framework of pros and cons for investigators / sponsors to consider during protocol design when incorporating mobile devices into regulatory trials
    - Primarily addressing ‘scientific considerations’

Secondary Products

- Manuscripts
- Conference Presentations
- Webinars

Timeline of upcoming activities

- Complete Phase I evidence gathering – Q4 2016
  - Interviews & analysis
- Complete Phase II of evidence gathering – Q1 2017
  - Interviews & analysis
- Expert meeting Q2 2017
- Finalize recommendations and other products Q3 2017

Developing Novel Endpoints Generated by Mobile Technology for Use in Clinical Trials

Project Team

Team Leaders	Team Members	Project Manager
Lauren Bataille (MJFF) Rob DiCicco (GSK) Cheryl Grandinetti (FDA) Will Herrington (Univeristy of Oxford) Martin Landry (University of Oxford) Kaveeta Vasisht (FDA)	Stephen Friend (Apple) Ashish Naryan (Northwell) Elektra Papodopoulous (FDA) Theresa Strong (FPWR) Komathi Stem (ReThynk Consulting/Genentech) Ken Skodacek (FDA) Nirav 'Rav' Sheth (MC10) Andrew Trister (Sage Bionetworks) Marc Walton (Janssen)	Jen Goldsack (CTTI) **CTTI Executive Committee Champion** John Alexander (Duke)

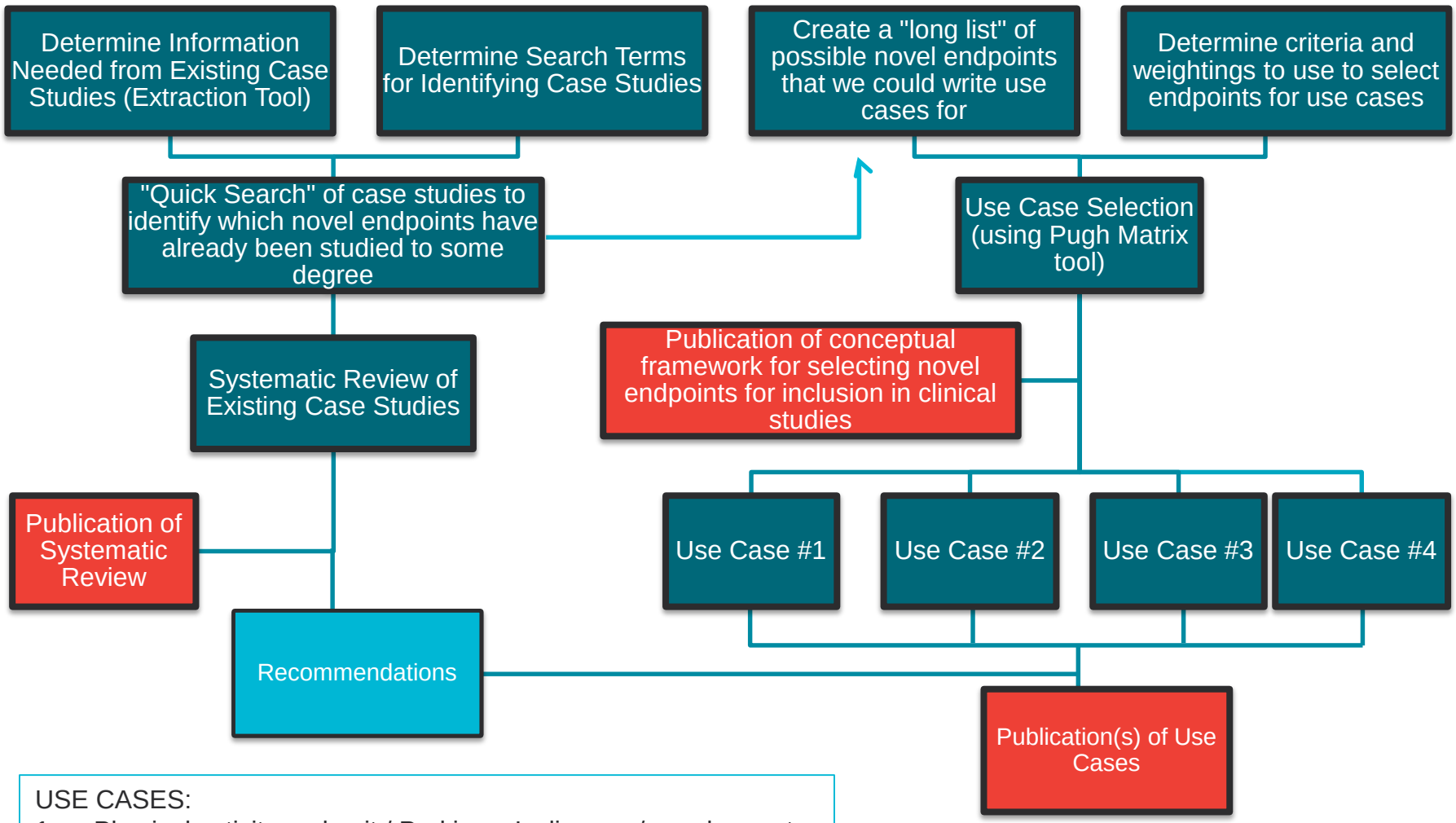
Developing Novel Endpoints Generated by Mobile Technology for Use in Clinical Trials

- **Purpose**:
  - This project aims to issue recommendations that clarify the pathway for developing novel endpoints*, generated using mobile technology, for use in clinical trials.

- **Objective:**
  - Describe best practices for developing novel endpoints, generated using mobile technology, for use in clinical trials.

* We have defined novel endpoints as either 1) new endpoints that are not currently used, or 2) existing endpoints that can now be measured in new and possibly better ways using mobile technology.

Our Approach



USE CASES:

1. Physical activity and gait / Parkinson's disease / accelerometer
2. Physical activity / heart failure / accelerometer
3. Blood sugar level / diabetes / CGM
4. Physical activity / muscular dystrophy / accelerometer

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Upcoming Milestones

- Systematic review of and data extraction from existing use cases is underway
  - Expected completion - Q4 2016
- Expert meeting - Q3 2016
- Completion of use cases - Q4 2016
- Finalize recommendations and other products - Q1 2017

Legal and Regulatory Issues Affecting the Adoption of Mobile Clinical Trials

Project Team

Team Leaders	Team Members	Project Manager
Linda Coleman (Yale University) Gary Grabow (Genentech) Jan Hewitt (FDA CDER) Barak Richman (Duke University)	David Babanian (Quorum Review) Mark Borigini (FDA CDER) Paul Conway (AAKP) Kristin Dolinski (PhRMA) Molly Flannery (FDA CDER) Amy Hummel (Alexion) Gracie Lieberman (Genentech) Leanne Madre (CTTI) Scott McGoohan (BIO) Kristen Miller (FDA) Nicole Miskel (Eli Lilly) Laura Podolsky (Science 37) Vaishali Popat (FDA CDER) Ken Skodacek (FDA CDRH) Marissa Stroo (Duke University) Evan Wearne (FDA/CDER)	Gerrit Hamre (CTTI)

Legal and Regulatory Issues
Project Purpose and Objectives

- **Purpose:**
  - The project aims to propose recommendations to overcome the legal and regulatory barriers that inhibit widespread use of mobile technology in clinical trials
- **Objectives:**
  - Catalog and summarize laws, regulations, and associated organizations that affect the implementation of mobile clinical trials
  - Identify perceived and actual legal and regulatory barriers to conducting mobile clinical trials
  - Identify opportunities to clarify and inform policies that affect the implementation of mobile clinical trials

Legal and Regulatory Issues

- Seven key areas of consideration
  - **Health Authority Receptivity/Readiness****
  - Good Clinical Practice
  - Institutional Review Boards
  - Privacy/Confidentiality
  - Reimbursement
  - Shipping and Receiving of Investigational Agents
  - **Telemedicine****

Upcoming Milestones

- Interviews Step One (Sponsors) – Q4 2016
- Interviews Step Two (FDA, IRBs, GCP Monitors, Associations, etc.) – Q1 2017
- Analysis of findings – Q1 2017
- Expert Meeting – Q2 2017
- Catalogue Summarizing Laws/Regs/Associations – Q3 2017
- Recommendations – Q3 2017

Stakeholder Perceptions

Project Team

Team Leaders	Team Members	Project Manager
Cynthia Geoghegan (Patient Advocate) Steve Morin (FDA/CDER) Virginia Nido (Genentech)	Maria Ali (George Institute) Ricky Bloomfield (Duke) David Borasky (WIRB Copernicus Group) David Brennan (Medstar Research Institute) Terri Hinkley (ACRP) Les Jordan (Target Health) Hassan Kadhim (Boehringer Ingelheim) Amanda Niskar (Arthritis Foundation) Ido Paz-Priel (Genentech) Bill Riley (NIH/OD /OBSSR) Ken Skodacek (FDA/CDRH) Junyang Wang (FDA/PASE)	Zach Hallinan (CTTI)

Project Purpose and Objectives

- **Purpose**:
  - This project will issue recommendations to overcome barriers on the use of mobile technology in clinical trials *as perceived by two key stakeholder groups, potential participants and community providers.*
- **Objectives**:
  - Identify the concerns of key stakeholders when using mobile technology to collect and share personal data in clinical trials and how these concerns can be addressed.
  - Determine key stakeholders' familiarity of and ease with using technology likely to be used in clinical trials.
  - Describe expectations for the ongoing provision of personal study data collected using mobile technology to study participants during clinical trial implementation.
  - Identify key stakeholders' perceptions of the benefits of using mobile technology in clinical trials.

Methodology

- Patient survey following a scenario-based approach, and targeted at 3-5 therapeutic areas. Objectives include:
  - Determine patients' familiarity of and ease with using mobile technologies in general and with mobile technologies likely to be used in clinical trials.
  - Identify perceived benefits and concerns of using mobile technologies to collect and share personal data in clinical trials.
  - Identify preferred and undesirable attributes of mobile technologies and with how mobile technologies are used in clinical trials.
- Survey of community providers planned but not yet initiated.
- Additional qualitative research may be considered based on findings of surveys.

Upcoming Milestones

- Survey development and data collection through Q2 2017
- Analyze/discuss survey results, consider additional info gaps – Q2-Q3 2017
- Expert Meeting – Q3 August 2017
- Recommendations – Q4 September 2017

Thank you.

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