U.S. FOOD & DRUG ADMINISTRATION

Public Workshop on Patient-Focused Drug Development

Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision-Making

December 6, 2019

#PFDD

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Disclaimer

The views expressed in the following presentations are those of the individual speakers and do not necessarily represent an official FDA position.

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Welcome

Meghana Chalasani, MHA
Office of the Center Director
Center for Drug Evaluation and Research

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Opening Remarks

Theresa Mullin, PhD
Associate Director for Strategic Initiatives
Center for Drug Evaluation and Research

Incorporating Clinical Outcome Assessments into Endpoints for Regulatory Decision Making

PFDD Guidance 4 Public Workshop
December 6, 2019

Theresa Mullin, PhD
Associate Director for Strategic Initiatives
FDA Center for Drug Evaluation and Research

Background: 5+ Years of Listening to Patients' Perspectives in PFDD Meetings

- Patients are uniquely positioned to inform FDA understanding of the clinical context
- PFDD meetings provided a more systematic method of obtaining patients' point of view on
  - Burden of disease
  - Burden of available treatment
  - What patients would value most in a new treatment
- We have heard from patients in meetings spanning a wide range of conditions *(26 PFDD, 30 EL-PFDD)*

PFDD Learnings

- **Patients** with chronic serious disease **are experts** on what it is like to live with their condition
- Patients **"chief complaints" may not be factored explicitly into medical product development** plans, including measures of medical product benefit planned in clinical studies
- Patients want to be as **active as possible** in the work to develop and evaluate new treatments
- PFDD meetings help elicit broader patient input for a disease to better inform clinical context of BR assessment. Patient stakeholders also started asking: **What's next?**
  - Not expecting FDA to address all current gaps in patient engagement but want FDA to provide clear actionable guidance on what they and others need to do
  - Concerned that many efforts underway may be duplicative and not coordinated

PFDD "What's Next"

Series of Methodological Guidance to enable stakeholders to go beyond powerful narrative and collect data that can serve as study endpoints and be used as a basis for marketing decisions

Dimension	Evidence and Uncertainties	Conclusions and Reasons
Analysis of Condition	PFDD Meetings and Reports provide powerful narrative that gives regulators insights about clinical context and what matters to patients	
Current Treatment Options		
Benefit	Using measures & tools (COAs) to systematically capture what matters most during clinical trials can turn narrative into evidence for regulatory decision making	
Risk and Risk Management		
Benefit-Risk Summary and Assessment		

Included in FDA Next Steps

Conduct public workshops and develop series of guidance documents on

1. Collecting comprehensive patient community input on burden of disease and current therapy
    - How to engage with patients to collect meaningful patient input?
    - What methodological considerations to address ?
2. Development of holistic set of impacts (e.g., burden of disease and burden of treatment) most important to patients
    - How to develop a set of impacts of the disease and treatment?
    - How to identify impacts that are most important to patients?
3. Identifying and developing good measures for the identified set of impacts that can then be used in clinical studies
    - How to best measure impacts (e.g., endpoints, frequency) in a meaningful way?
    - How to identify measure(s) that matter most to patients?
4. **Incorporating measures (COAs) into endpoints considered significantly robust for regulatory decision making**
    - Topics including technologies to support collection through analysis of the data

Today's Workshop Informs Development of Guidance 4 in the Series

PDUFA VI Commitment

"By the end of FY 2021, FDA will publish a draft guidance on clinical outcome assessments, which, when final, will, as appropriate, revise or supplement the 2009 Guidance to Industry on Patient-Reported Outcome Measures. The draft guidance will also address technologies that may be used for the collection, capture, storage, and analysis of patient perspective information. The guidance will also address methods to better incorporate clinical outcome assessments into endpoints that are considered sufficiently robust for regulatory decision-making."

21st Century Cures Section 3002(c)(4)

[guidance shall address] "methodologies, standards, and technologies to collect and analyze clinical outcome assessments for purposes of regulatory decision making;"

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Overview of FDA's Approach to Patient-Focused Drug Development Guidance 4

Scott Komo, DrPH
Office of Translational Sciences
Center for Drug Evaluation and Research

Introduction

- Will cover methodologies, standards, and technologies to collect and analyze clinical outcome assessments (COA) for purposes of regulatory decision making
- Guidance 4 continues on from Guidance 3
  - Now that you have developed a fit-for-purpose COA, how do you create an endpoint using COA data?
  - Important: COA is not the same as the endpoint

Primary Audience

- Stakeholders involved in the design, conduct, analysis, and review of clinical studies incorporating COAs
- Useful for statistical, data management, and related audiences
- Medical product sponsors, clinical research organizations, industry consultants and other researchers who provide professional services in this area, academic and other researchers, FDA reviewers, and patient groups
- Other audiences include organizations involved in development of registries, natural history studies, and endpoint or COA development

Discussion Document Overview

- Introduction
- Estimand framework
- Meaningful within-patient change (will not be discussed today)
- Additional considerations
- Two examples

Discussion Document Format

- Sections contain
  - Section summary aimed at a broader audience
  - Technical summary
  - Technical details

➢ QUESTION: Do you find this formatting approach helpful in understanding the material?

Factors to Consider When Constructing COA-based Endpoints (1)

- Each COA-based endpoint stated as part of a clinical study objective
- COAs are fit-for-purpose and sensitive to detect meaningful changes
- Effect of disease type (e.g., acute, chronic) on endpoint selection
- Treatment objective (e.g., cure, symptom management)
- Clinical study duration is adequate to support COA objectives
- Frequency and timing of COA administration is appropriate given patient population, study design and objectives, and COA measurement properties

Factors to Consider When Constructing COA-based Endpoints (2)

- Scoring algorithm is specified and consistent with tool development including handling of missing data
- Plans for COA measurement after treatment discontinuation are driven by the research questions
- Effect of blinding (interpretation and use of COA-based endpoints in open-label or single-blind trials)
- Considerations when using a nonrandomized or nonconcurrent control

➢ QUESTION: What other factors should be included and why?

Estimand Framework

- Estimand: quantity used to define a treatment effect for a study objective in a clinical study
- Aims to align the study design, endpoint, and analysis with the clinical study objective to improve study planning and the interpretation of analyses

Estimand Attributes Discussed Today

- Target population for the study
- Endpoint (e.g., what variables will be used including which time points)
- Events precluding observation or affecting interpretation be accounted for in the analyses, (e.g., dropouts, use of rescue medication, not following prescribed regimen)
- Population level summary (e.g., comparing means, hazard ratios)

Estimand Attributes

- Attributes present (implicitly or explicitly stated) in every data analysis
- Choices made strongly impact interpretation of the analysis, power, and data collected

Heterogeneity in Symptoms and/or Functional Status

Considerations in endpoint construction when there is heterogeneity in symptoms and/or functional status

- Between patients
- Within the same patient over time

Topics Not Discussed Today

- Meaningful within-patient change
- Computerized adaptive testing
- Formats for submissions

➢ If you have comments on the discussion document relating to these topics, please submit to the docket

https://www.regulations.gov/document?D=FDA-2019-N-4900-0001

Docket Comments

- Docket closes at 11:59 PM ET Feb 4, 2020
- Topics could include but are not limited to
  - Content (e.g., lack of clarity, missing, suggested modifications)
  - Level of technical detail
  - Formatting
  - Examples for online materials
  - Questions in the document (e.g., computerized adaptive testing)
  - Any additional comments for the guidance series

Send us your comments!

If you have examples, information, feedback or comments, please submit to the public docket for this workshop!

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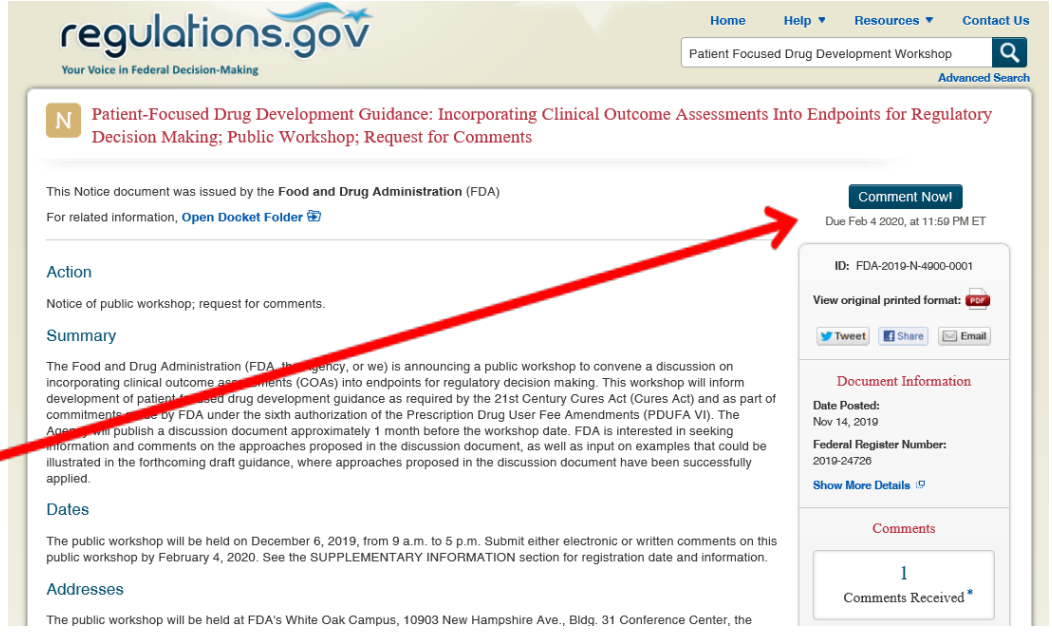

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Session I: General Considerations for Developing an Endpoint From COA Data

Moderator: Martin Ho, MS
Office of Biostatistics and Epidemiology
Center for Biologics Evaluation and Research

PANEL SESSION 1

- **Fraser Bocell**, *Office of Strategic Partnerships and Technology Innovation, CDRH, FDA*
- **Kendra Hileman**, *Vice President, Head of Clinical Research and Development, Alcon*
- **Hylton Joffe**, *Office of New Drugs, CDER, FDA*
- **Larissa Lapteva**, *Office of Tissues and Advanced Therapies, CBER, FDA*
- **Gianna (Gigi) McMillen**, *Patient Advocate and Program Administrator, Bioethics Institute at Loyola Marymount University*
- **Linda Nelsen**, *Senior Director and Head, Patient-Centered Outcomes, GlaxoSmithKline*
- **Kevin Weinfurt**, *Professor and Vice Chair for Research, Department of Population Health Sciences, Duke University School of Medicine*

PANEL SESSION 1

Objective: This Workshop's Discussion Document covers several topics and includes factors to be considered when constructing an endpoint based on a fit-for-purpose COA. Explore and discuss at a high level information in the document and suggested areas to include in guidance.

Questions to address:

1. Should the future guidance provide any additional details on the currently proposed factors?
2. What additional factors should be included in the guidance?

AUDIENCE Q&A

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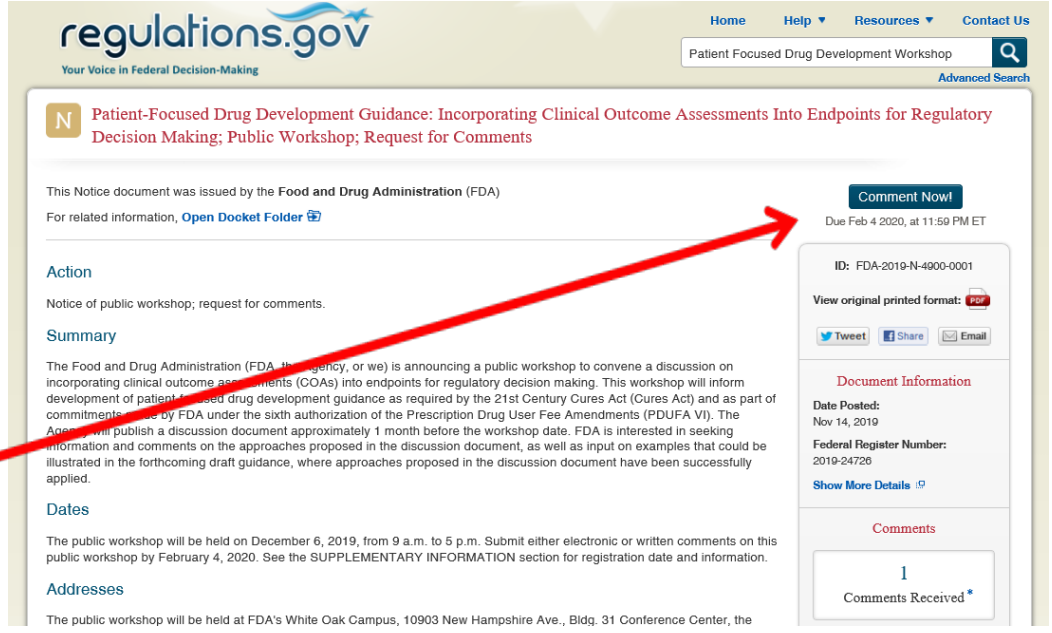

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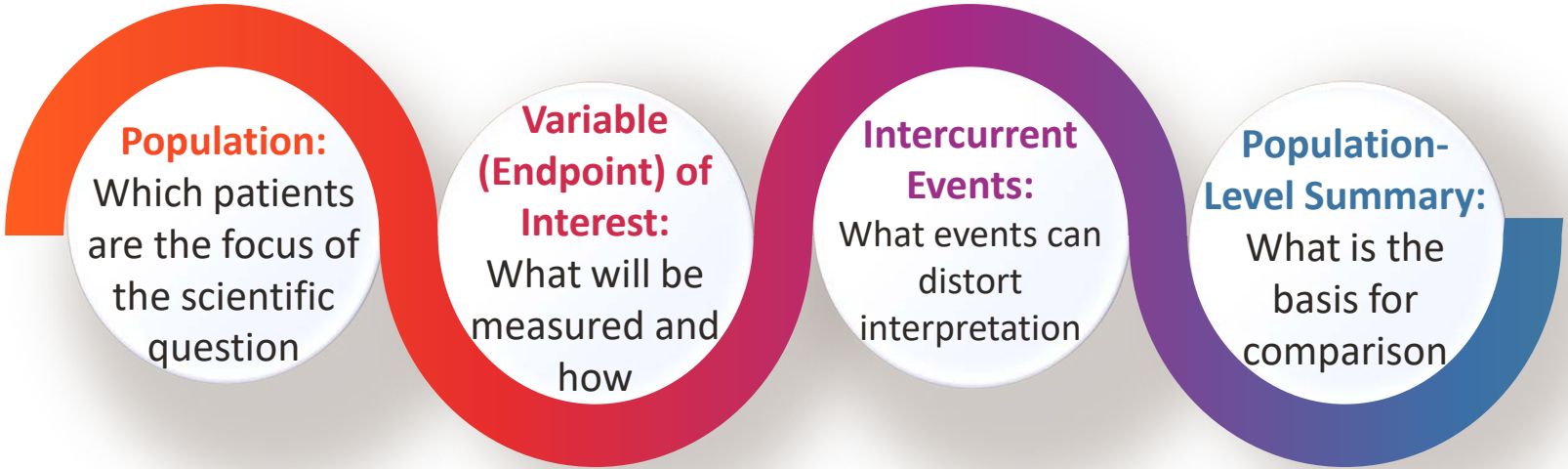
Session II: Using the Estimand Framework to Design, Conduct, and Analyze Data From a Trial with a COA-Based Endpoint

Moderator: Mallorie H. Fiero, PhD
Office of Translational Sciences
Center for Drug Evaluation and Research

PANEL SESSION 2

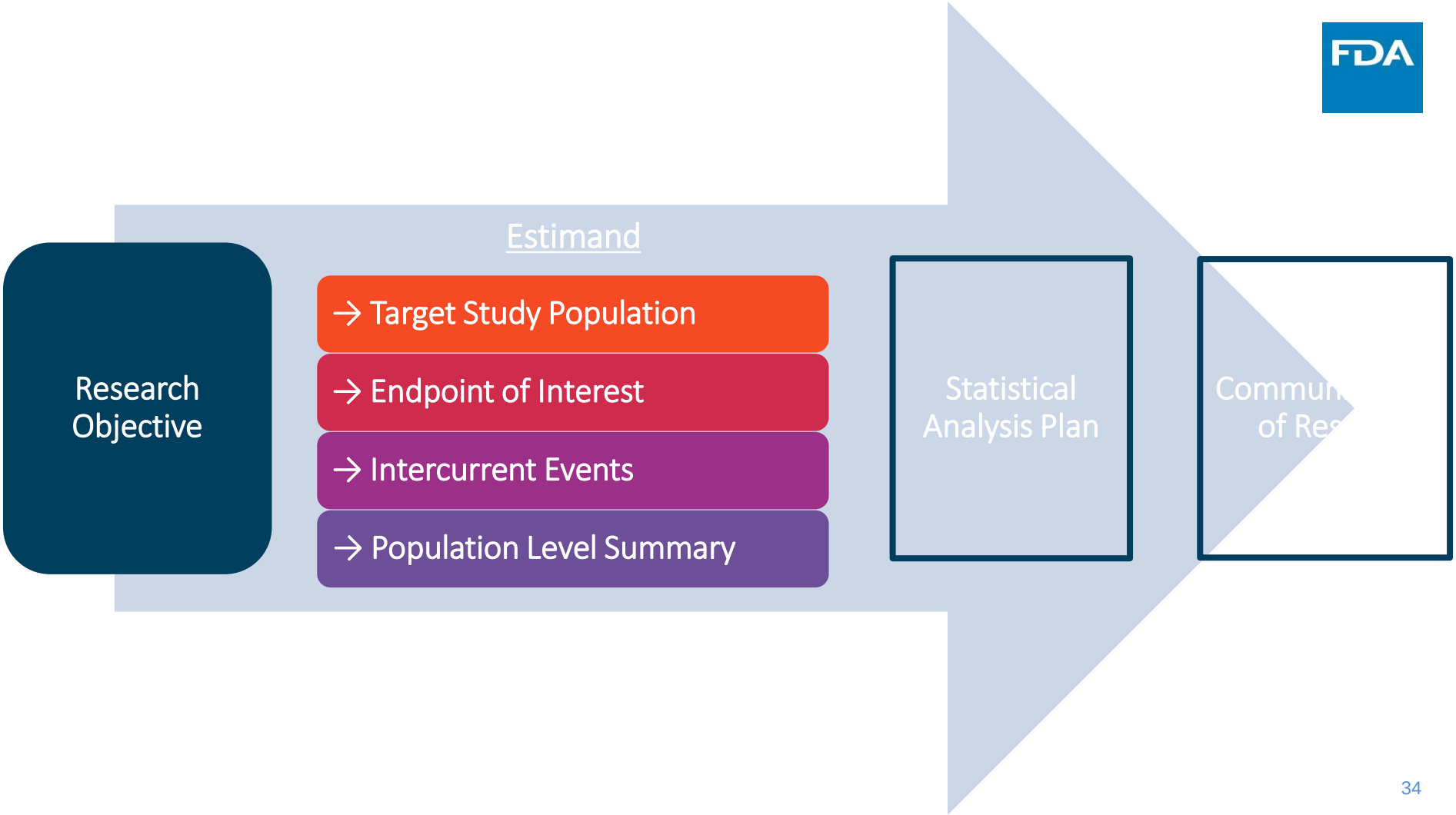
- **Jessica Lee**, *Office of New Drugs, CDER, FDA*
- **Gregory Levin**, *Office of Translational Science, CDER, FDA*
- **John Scott**, *Office of Biostatistics and Epidemiology, CBER, FDA*
- **Daniel Serrano**, *Director of Psychometrics, Pharmerit*
- **Kevin Weinfurt**, *Professor and Vice Chair for Research, Department of Population Health Sciences, Duke University School of Medicine*
- **Lisa Weissfeld**, *Senior Investigator, Statistics Collaborative*

What is an estimand?



Estimand: Target of estimation to address a study's scientific question of interest

Source: 2019 COA-CCT Workshop


Research Objective
Estimand
→ Target Study Population
→ Endpoint of Interest
→ Intercurrent Events
→ Population Level Summary
Statistical Analysis Plan
Commun
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DISCLAIMER

These case studies are **not an endorsement** of a singular study design, outcome, analysis, or visualization; rather they are meant to illustrate principles conceptualizing a COA research question and design

Case Study Clinical Scenario

Scenario	• Metastatic ER/PR+ HER2- breast cancer after progression on 1st line therapy

Epidemiology and Disease Information	• Breast cancer has heterogeneous disease symptoms and many women will be asymptomatic at baseline
	• 2nd line prior studies have shown
	  • Median overall survival (OS) 2-2.5 years with 2nd line therapy alone
	  • Median progression-free survival (PFS) of 10-12 months

Treatment goal	• Addition of target therapy to hormonal agent will improve PFS by 6-8 months

Source: https://www.uptodate.com/contents/treatment-approach-to-metastatic-hormone-receptor-positive-her2-negative-breast-cancer-endocrine-therapy-and-targeted-agents

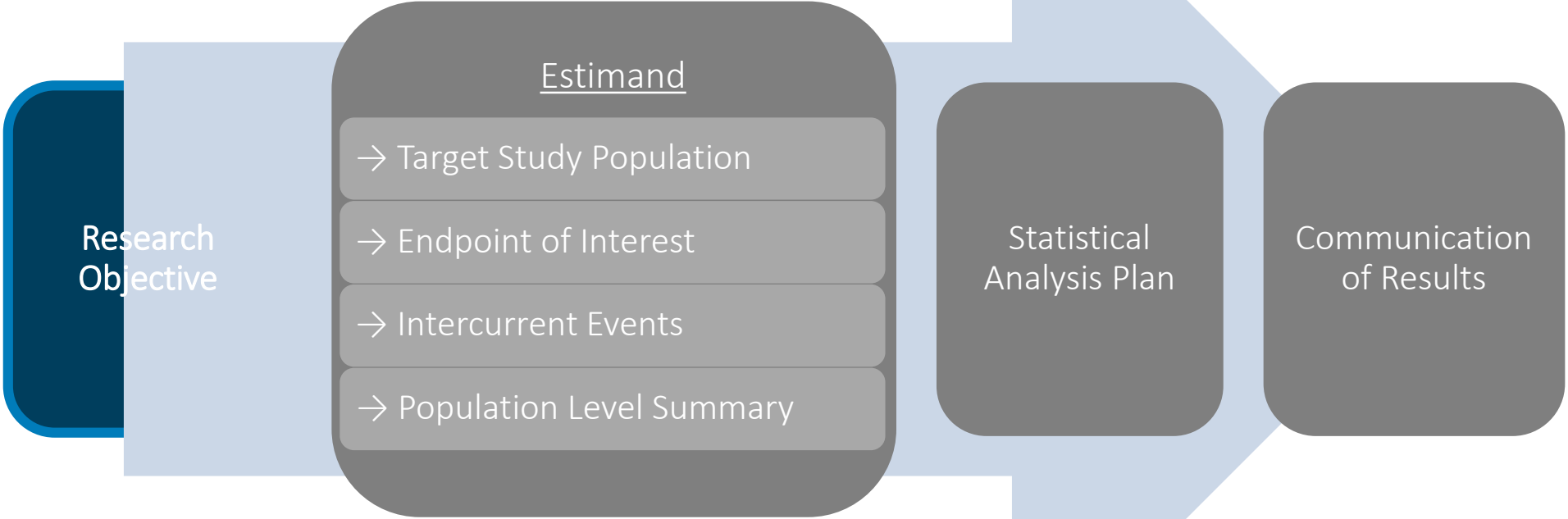
Case Study Clinical Scenario

Study Design

- Randomized controlled trial
  - Treatment: Standard of care + oral targeted investigational agent
  - Control: Standard of care + placebo

Expected Outcomes

- Expected Efficacy: 6-8 month progression-free survival benefit
  - Overall survival may be impacted if patients initiate subsequent therapy
  - **Physical function score using well-defined measurement tool collected at every treatment cycle**
- Expected Safety: Symptomatic toxicities including diarrhea, fatigue and rash on investigational arm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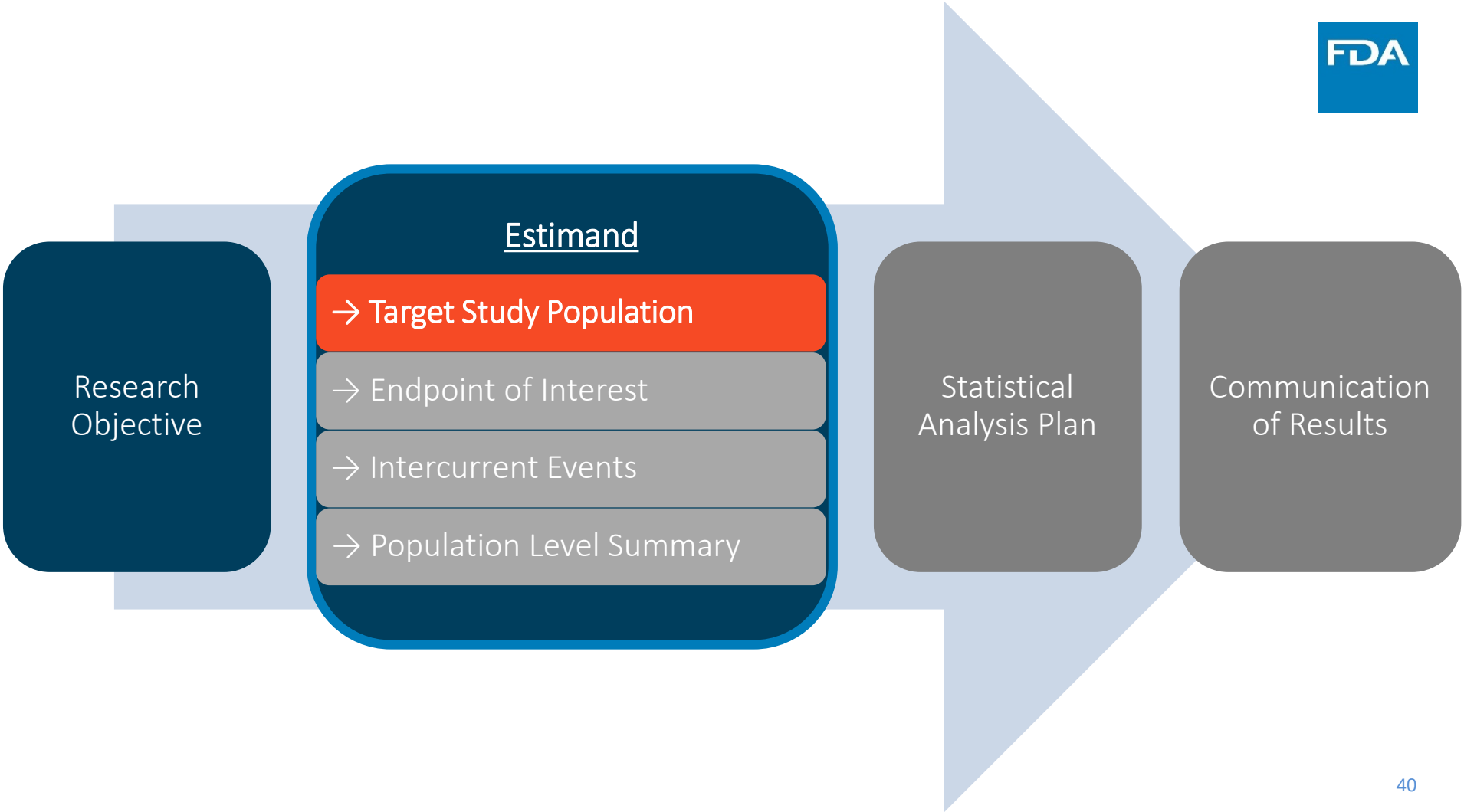
Define COA Scientific Research Question *A Priori*

Broad COA Research Objective

Evaluate efficacy related to physical function

Scientific Research Question

Is the average change in physical function from baseline to Week 28 better (superior) in the investigational arm compared to the control arm?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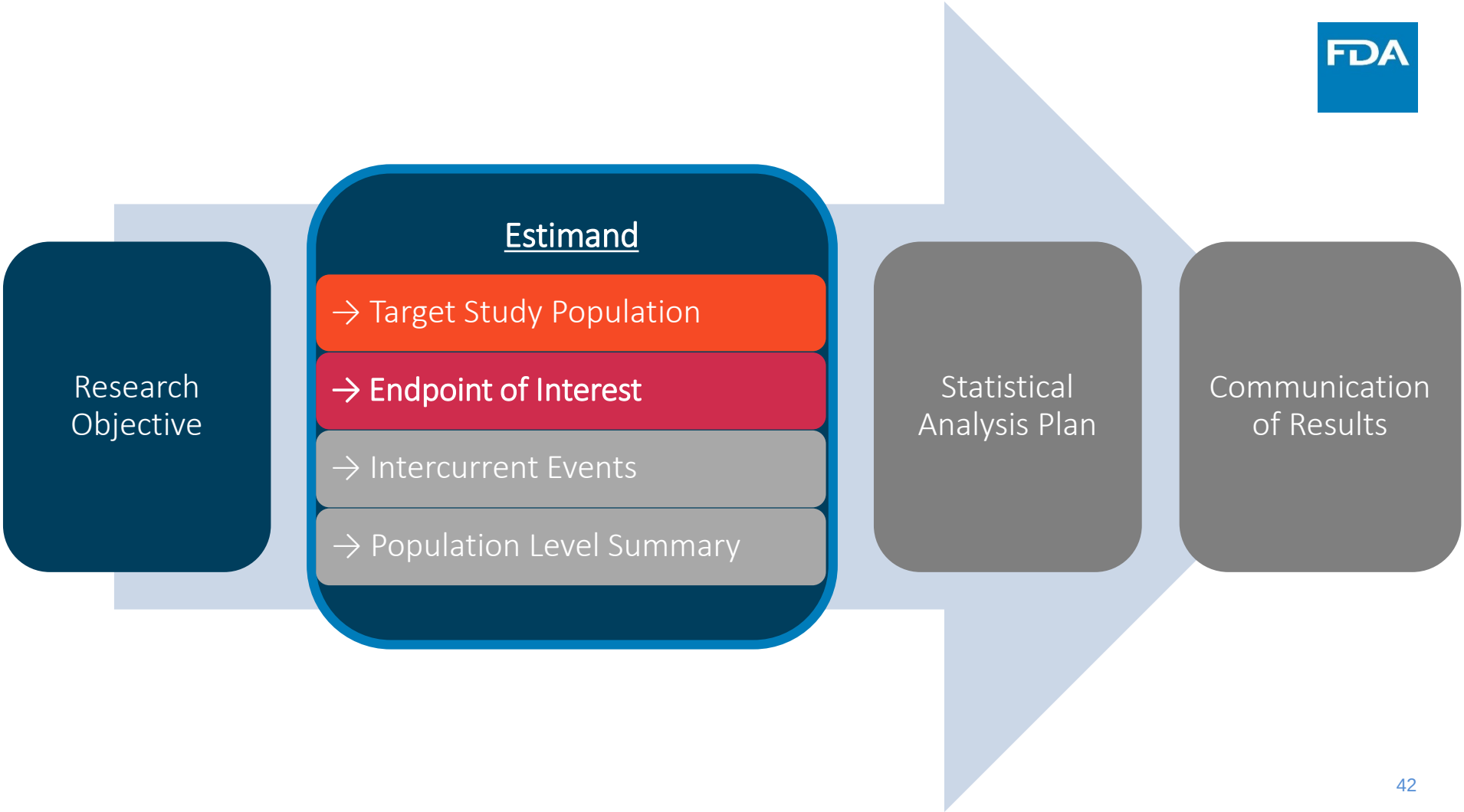
Define Target Study Population Based on Research Question *A Priori*

Scientific Research Question

Is the average change in physical function from baseline to Week 28 better (superior) in the investigational arm compared to the control arm?

Target Study Population

Defined through inclusion/exclusion criteria to reflect the targeted patient population for medical product approval.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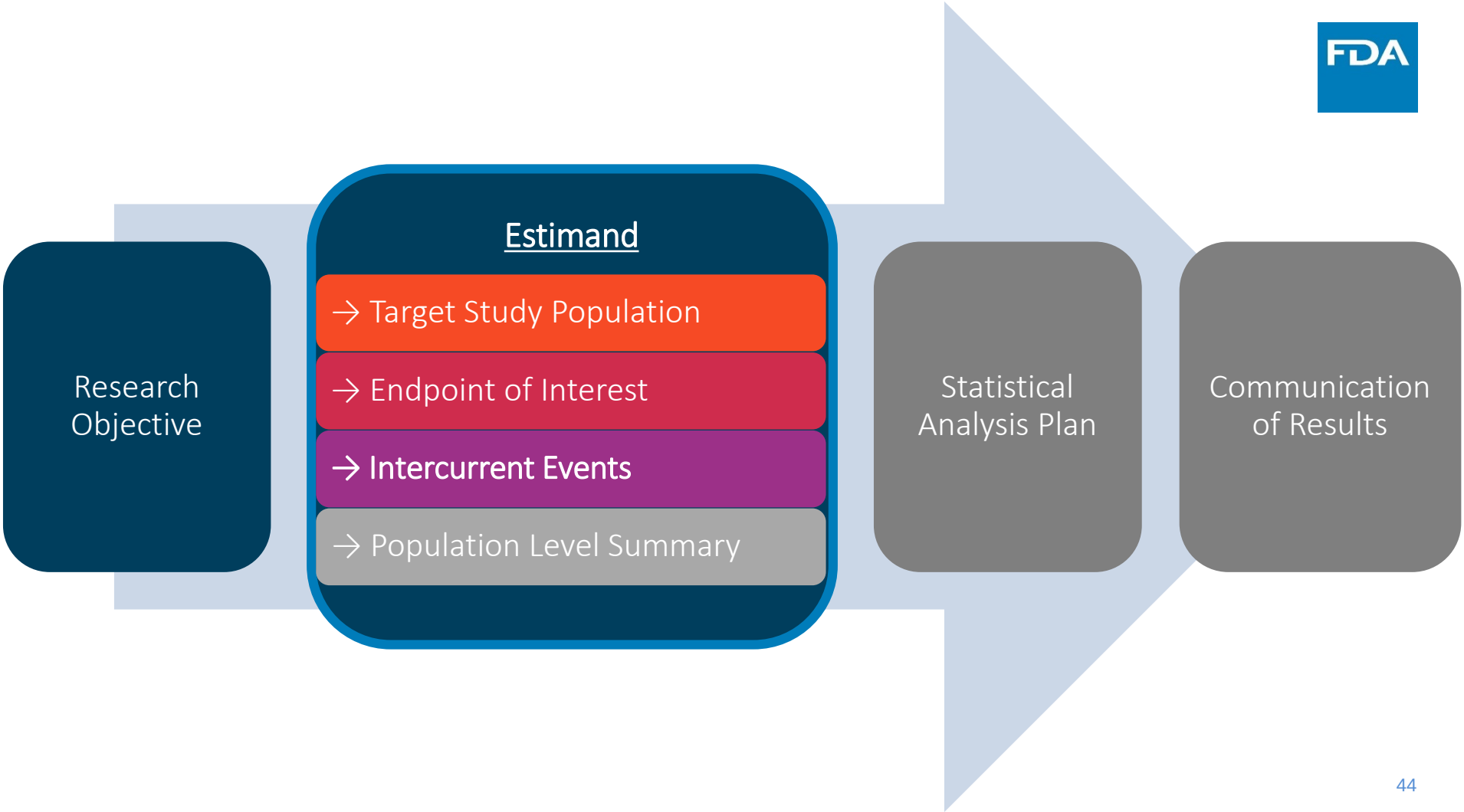
Define Endpoint of Interest Based on Research Question *A Priori*

Scientific Research Question

Is the average change in physical function from baseline to Week 28 better (superior) in the investigational arm compared to the control arm?

Endpoint of Interest

Change from baseline in physical function score using well-defined measurement tool. Use measurements at baseline and at Week 28.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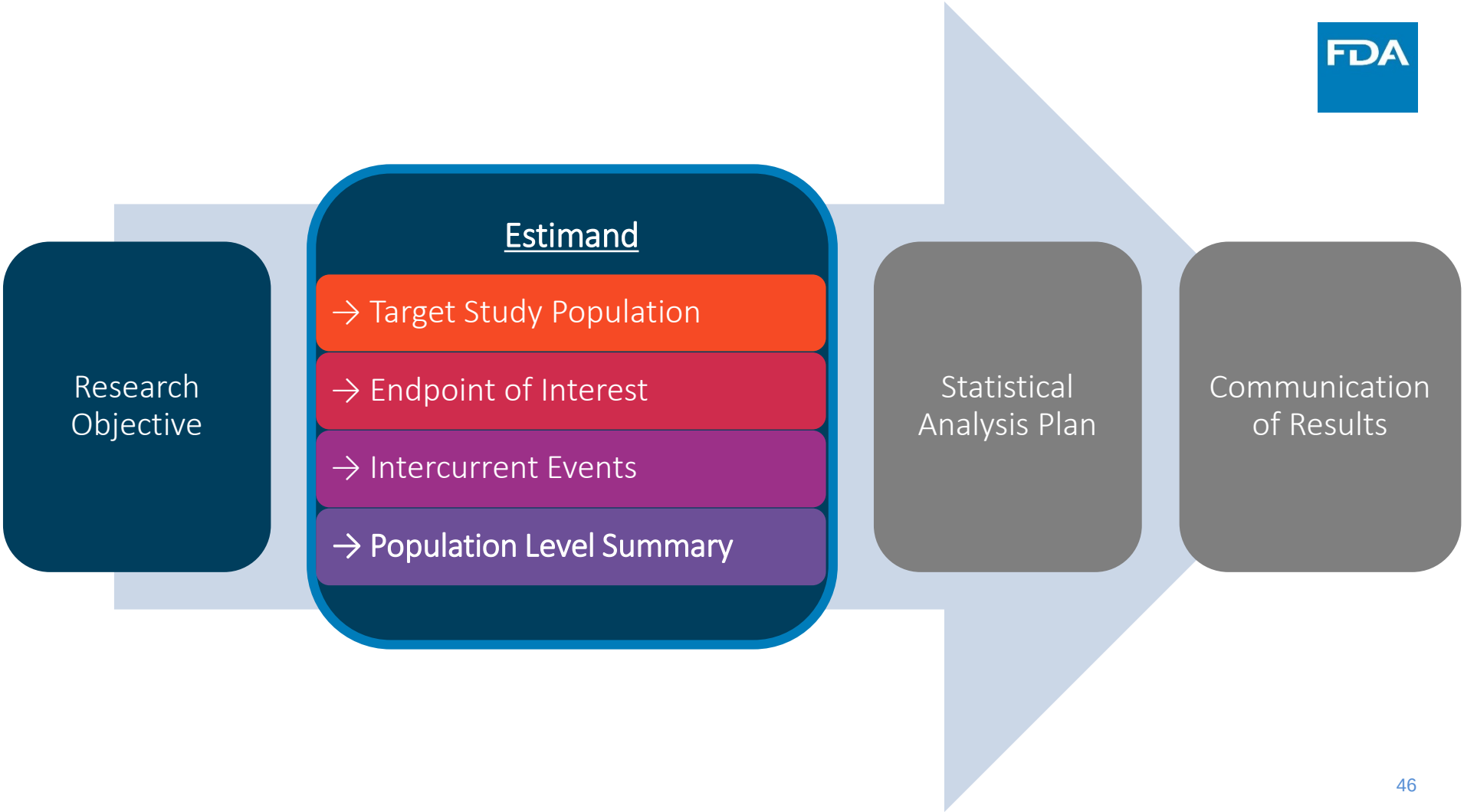
Address Intercurrent Events in Alignment with Research Question

Scientific Research Question

Is the average change in physical function from baseline to Week 28 better (superior) in the investigational arm compared to the control arm?

Intercurrent event	Addressing intercurrent event
• Discontinuation of treatment • Disease progression • Physical therapy • Subsequent therapy	Physical function collected and included in analysis regardless of whether intercurrent event occurs
• Death	Address in the analysis plan; may be included as part of the endpoint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Define Population Level Summary Based on Research Question *A Priori*

Scientific Research Question

Is the average change in physical function from baseline to Week 28 better (superior) in the investigational arm compared to the control arm?

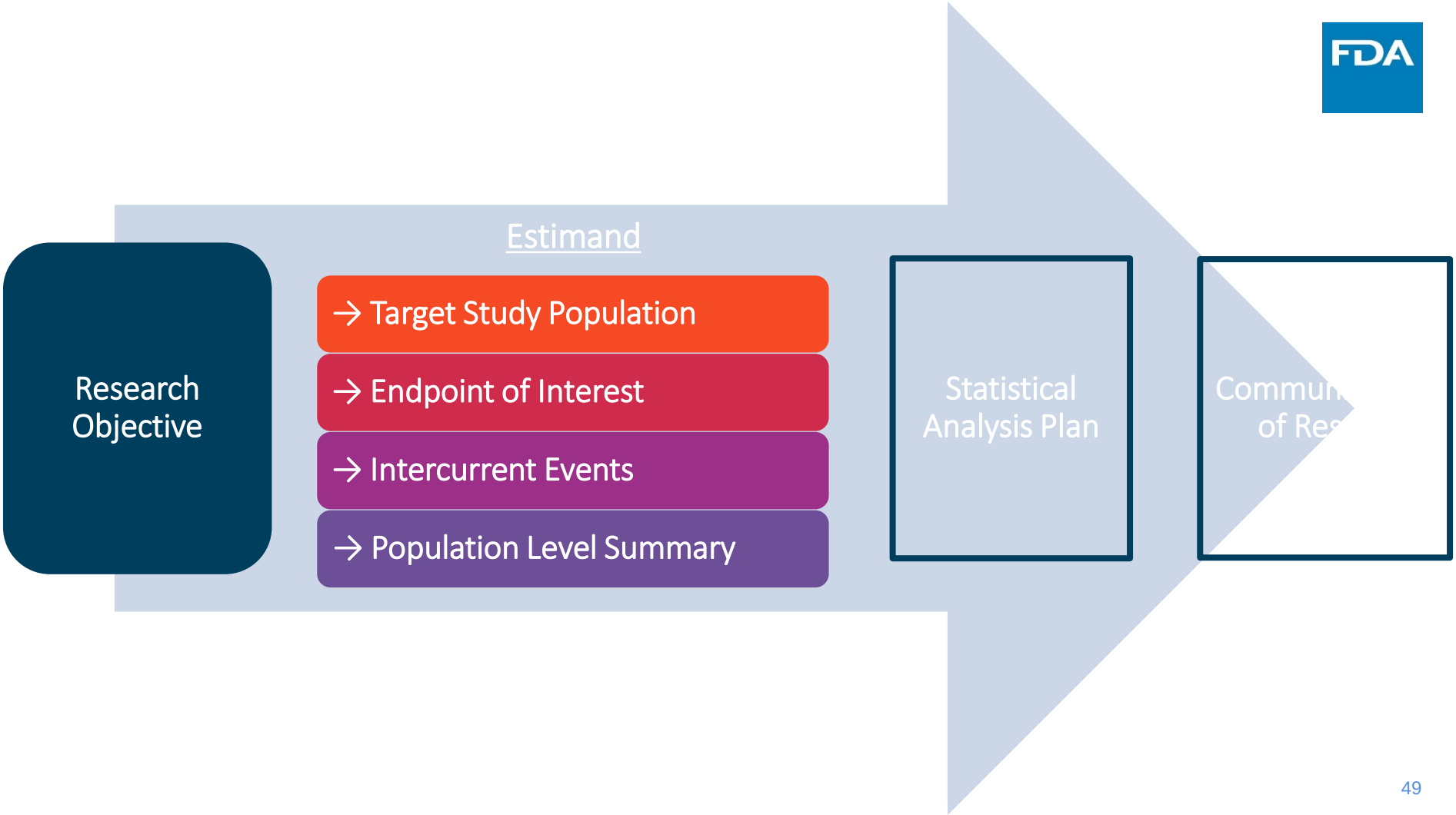
Population Level Summary

Difference between treatment arms in mean change from baseline in physical function score using baseline and Week 28 measurements.

Summary of Estimand Attributes for this Case Study

Estimand attributes	Decisions to better define research objectives
Target population	Defined through inclusion/exclusion criteria to reflect the targeted patient population for approval.
Endpoint of interest	Change from baseline in physical function score using well-defined measurement tool. Use measurements at baseline and at Week 28.
Addressing intercurrent events	
• Disease progression • Treatment discontinuation • Physical therapy • Subsequent therapy	Physical function collected and included in analysis regardless of whether intercurrent event occurs.
• Death	Address in the analysis plan; may be included as part of the endpoint
Population level summary	Difference between treatment arms in mean change from baseline in physical function score using baseline and Week 28 measurements.

These case studies are **not an endorsement** of a singular study design, outcome, analysis, or visualization; rather they are meant to illustrate principles conceptualizing a COA research question and design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PANEL SESSION 2

Objective: Introduce and discuss approaches for identifying the appropriate analysis population, determining clinical study duration and timing of COA administration, and adjusting for potential confounders or intercurrent events

Questions to address:

1. What do you foresee as real-life challenges when using the estimand framework for a COA research objective?
   - In addition, please discuss considerations in addressing intercurrent events

PANEL SESSION 2

Objective: Introduce and discuss approaches for identifying the appropriate analysis population, determining clinical study duration and timing of COA administration, and adjusting for potential confounders or intercurrent events

Questions to address:

2. How does a treatment's mechanism of action, disease's natural history, etc. impact study duration and timing/frequency of assessments for COA endpoints?

AUDIENCE Q&A

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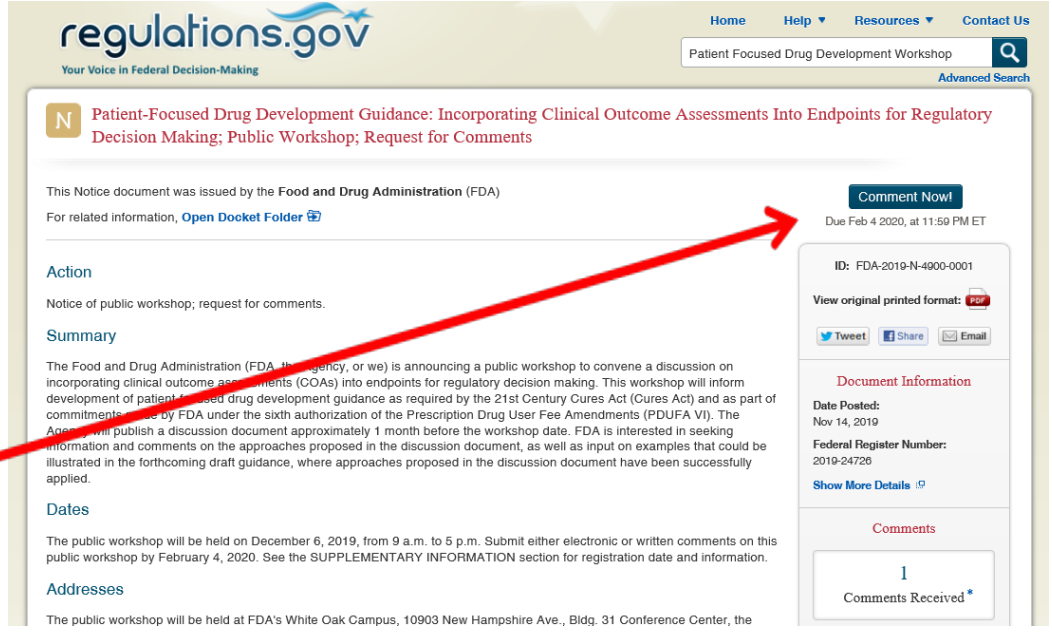

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Session III: Considerations When There Is Heterogeneity in Disease Symptoms and Functional Status Between Patients and Within the Same Patient Over Time

Moderator: Lili Garrard, PhD
Office of Translational Sciences
Center for Drug Evaluation and Research

PANEL SESSION 3

- **Lisa Kammerman,** *Regulatory Statistics and PRO Consultant, Kammerman Consulting, LLC*
- **Elektra Papadopoulos,** *Office of New Drugs, CDER, FDA*
- **Tejashri Purohit-Sheth,** *Office of Tissues and Advanced Therapies, CBER, FDA*
- **David Reasner,** *Head of Data Science & Analytics, Imbria Pharmaceuticals*
- **Steve Roberds,** *Chief Scientific Officer, Tuberous Sclerosis Alliance*
- **Patroula Smpokou,** *Office of New Drugs, CDER, FDA*
- **R.J. Wirth,** *President and Managing Partner, Vector Psychometric Group*

UNDERSTANDING HETEROGENEITY

- Example variability in disease
  - Genotypic, e.g. mtDNA/nDNA mutations
  - Phenotypic
    - May range from monosymptomatic to multisystemic diseases
    - Disease manifestations
    - Rate of disease progression
    - Baseline severity of symptoms and functional status
  - Waxing and waning nature
  - Wide age range, etc.
- Challenging to assess a single concept of interest across all patients

PANEL SESSION 3

Objective: Discuss considerations for COA measurement and analysis for diseases with heterogeneous patient populations and/or variable manifestations

Questions to address:

1. What factors should be considered when developing a COA-based endpoint for diseases with heterogeneous patient populations and variable manifestations?
   - ➢ Include potential analysis and interpretation issues

PANEL SESSION 3

Objective: Discuss considerations for COA measurement and analysis for diseases with heterogeneous patient populations and/or variable manifestations

Questions to address:

2. What factors should be considered when constructing personalized/individualized endpoints for use in studies?
   - ➢ Include what personalized/individualized endpoints mean to you
   - ➢ Include potential analysis and interpretation issues

AUDIENCE Q&A

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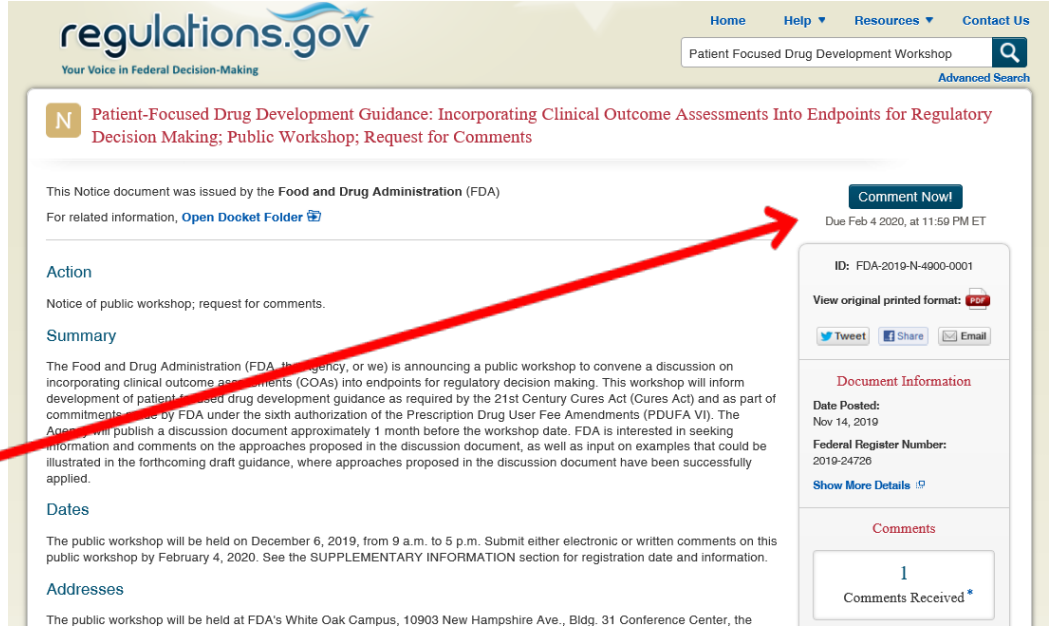

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Session IV: Pulling It All Together – An Example Across Guidances

Moderator: Ebony Dashiell-Aje, PhD
Office of New Drugs
Center for Drug Evaluation and Research

PANEL SESSION 4

- **Bill Byrom**, *Vice President of Product Strategy and Innovation, Signant Health*
- **Michelle Campbell,** *Office of New Drugs, CDER, FDA*
- **Andrea Coravos**, *Co-founder and Chief Executive Officer, Elektra Labs*
- **Matthew Diamond**, *Office of Strategic Partnerships and Technology Innovation, CDRH, FDA*
- **Mark Frasier,** *Senior Vice President, Research Programs, The Michael J. Fox Foundation for Parkinson's Research*
- **Abigail Luo**, *Office of Biostatistics and Epidemiology, CBER, FDA*
- **Andrew Potter**, *Office of Translational Sciences, CDER, FDA*
- **Diane Stephenson**, *Executive Director, Critical Path for Parkinson's Consortium, Critical Path Institute*

Session Objective

- Discuss a working example – Information from this panel session will inform the development of an case study illustrating important concepts for consideration in the collection of COA data using digital health technologies (DHTs) within the clinical study context

Session Outline

- DHTs to Evaluate Clinical Benefit: A few guiding principles
- Panel Discussion: Case examples and input
- Audience Q&A

DHTs to Evaluate Clinical Benefit

Terminology:
Digital Health Technologies* (DHTs)

- Technologies that use computing platforms, connectivity, software, and/or sensors for healthcare and related uses
  - DHTs span a wide range of uses, from applications in general wellness to applications as a medical device
  - DHTs are also used as companion diagnostics, companion therapeutics or adjuncts to other medical products (devices, drugs, and biologics)
- They may also be used to develop or study medical products

(*Derived from CDRH definition)

DHTs Include (But Are Not Limited To)

- Wearable, implantable, or ingestible sensors
  - *Accelerometers, continuous glucose monitors, heart rate monitors*
- Environmental sensors placed in the subject's home
  - *Motion sensors*
- Software applications
  - *Apps that collect COAs*
- Other general purpose hardware
  - *Mobile phone camera*
- Specialized hardware
  - *Handheld spirometers*

DHT Use

- Assess existing endpoints or novel endpoints
- May be used to collect data remotely
- Can perform
  - Passive data capture (e.g., accelerometer, cardiac rhythm measurement throughout the day)
  - Active data capture
    - Measurement during task performance (e.g., finger tapping test)
    - Patient responses (e.g., an electronic PRO [ePRO])

Evidentiary Considerations

- Well-defined and reliable (21 CFR 314.126)
- Compliance with FDA regulatory requirements for record keeping, maintenance, and access (21 CFR Part 11)

Guiding Principles

Concept Measurement (Guidance 1-3):

- Determine what are the important concepts to measure by talking to patients and discussing these concepts with FDA review staff

- For the concept/symptom identified, consider if a DHT is an appropriate measurement approach

Guiding Principles

Tool Selection (Guidance 3):

- Assess if the DHT meets performance specifications (including accuracy, reliability, and validity) for the proposed intended use

Guiding Principles

Usability Testing (Guidance 1 & 3):

- Plan to conduct usability studies to ensure that the DHT is usable by patients in the proposed context of use without serious errors or problems

Guiding Principles

Endpoint Measurement (Guidance 4):

- Propose an endpoint using the DHT measurements that captures the important concept previously identified, and then consider the statistical and measurement properties of this endpoint

Guiding Principles

Clinical Study Deployment (Other Guidances):

- Consider how to deploy and use the DHT in the study, including how patients will receive the DHT, how data will be collected from the DHT, and how clinical operations will be adapted

Scenario: Assessing Gait in Parkinson's Disease

- Based on a literature review, a sponsor asserts gait (e.g., ability to walk distances, gait speed) is important to assess in patients with Parkinson's Disease
  - Interested in exploring use of a general purpose consumer accelerometer to measure gait variability to support medical product development
  - Hopes data can be used to demonstrate difference in gait variability between treatment arms in their clinical trial
- Existing methods to assess gait variability in clinical investigations are based on in-clinic performance outcome (PerfO) assessments
  - Can a DHT capture data reflecting how patients function in their daily lives?

PANEL SESSION 4

Objective: Discuss a working example – Information from this panel session will inform the development of an case study illustrating important concepts for consideration in the collection of COA data using digital health technologies (DHTs) within the clinical study context

➢Thinking more broadly, beyond the example

Questions to address:

1. What additional details would be helpful to clearly illustrate the guiding principles (as applied to DHTs) when the data is intended for use as an endpoint in clinical trials?

PANEL SESSION 4

Objective: Discuss a working example – Information from this panel session will inform the development of an case study illustrating important concepts for consideration in the collection of COA data using digital health technologies (DHTs) within the clinical study context

➢Thinking more broadly, beyond the example

Questions to address:

2. How well do the guiding principles illustrate considerations for any type of COA implementation in trials, especially the importance of considering patient input and knowledge of the natural history of the disease when deciding on a target concept (e.g., gait variability)?

Helpful Links

- Guidance for Industry: Patient-Reported Outcome Measures: Use in Medical Product Development to Support Labeling Claims
  - http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/UCM071975.pdf
- Guidance for Industry: Computerized Systems Used in Clinical Investigations
  - http://www.fda.gov/OHRMS/DOCKETS/98fr/04d-0440-gdl0002.pdf
- Guidance for Industry: Electronic Source Data in Clinical Investigations
  - http://www.fda.gov/downloads/drugs/guidancecomplianceregulatoryinformation/guidances/ucm328691.pdf
- Clinical Trials Transformation Initiative (CTTI) Novel Endpoints Project
  - https://www.ctti-clinicaltrials.org/projects/novel-endpoints
- Framework for FDA's Real World Evidence Program
  - https://www.fda.gov/media/120060/download
- CDRH and Digital Health Website
  - https://www.fda.gov/medical-devices/digital-health
- CDRH Guidance on Real World Evidence
  - https://www.fda.gov/regulatory-information/search-fda-guidance-documents/use-real-world-evidence-support-regulatory-decision-making-medical-devices

AUDIENCE Q&A

Session V: Identifying Key Themes and Rounding Out the Guidance Series

Moderator: Meghana Chalasani, MHA
Office of the Center Director
Center for Drug Evaluation and Research

PANEL SESSION 5

- **Marc Boutin,** *Chief Executive Officer, National Health Council*
- **Stephen Joel Coons,** *Executive Director, Patient-Reported Outcome Consortium, Critical Path Institute*
- **Katarina Halling,** *Global Head Patient Centered Science, AstraZeneca*
- **Telba Irony,** *Office of Biostatistics and Epidemiology, CBER, FDA*
- **Laura Lee Johnson,** *Office of Translational Sciences, CDER, FDA*
- **Pandu Kulkarni,** *Vice President, Biometrics and Advanced Analytics, Eli Lilly and Company*
- **Michelle Tarver,** *Office of Strategic Partnerships and Technology Innovation, CDRH, FDA*

PANEL SESSION 5

Objective: Reflect on the day's discussion, specifically any themes that emerged throughout the day. Discuss key considerations that should guide FDA's completion of its methodological PFDD guidance series.

Questions to address:

1. What are the key themes and considerations from today's discussions that should guide the development of guidance on these topics?
2. Considering this is the fourth and final guidance in FDA's methodological PFDD guidance series, is there a clear understanding of the big picture and how the pieces fit together?

AUDIENCE Q&A

U.S. FOOD & DRUG ADMINISTRATION

Open Public Comment

Moderator: Mary Jo Salerno, MPH
Office of Translational Sciences
Center for Drug Evaluation and Research

U.S. FOOD & DRUG ADMINISTRATION

Closing Remarks

Laura Lee Johnson, PhD
Office of Translational Sciences
Center for Drug Evaluation and Research

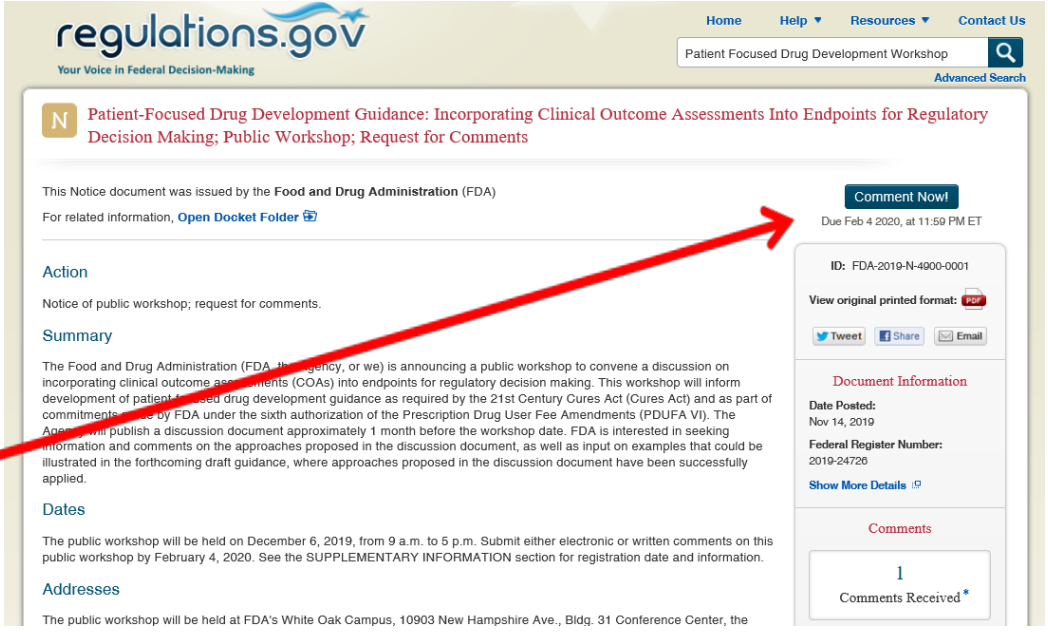
Send us your comments!

If you have examples, information, feedback or comments, please submit to the public docket for this workshop!

The docket will close on February 4, 2020, at 11:59 PM ET.

How do you submit a comment?

- Please visit: https://www.regulations.gov/document?D=FDA-2019-N-4900-0001
- Or search “Patient Focused Drug Development Workshop” on www.regulations.gov
- And **Click Comment Now**!



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Thank you!


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