Annual Clinical Outcome Assessment Qualification Update and Other FDA News

COA Qualification Program,
Office of Strategic Programs,
Center for Drug Evaluation and Research, U.S. FDA
Oncology Center of Excellence, U.S. FDA

8th Annual PRO Consortium Workshop
Bethesda, MD
April 26, 2017
Disclaimer

The views expressed in this presentation are those of the speaker, and do not necessarily represent an official FDA position.
Presenters

• Theresa Mullin, PhD
  – Director, Office of Strategic Programs
• Paul G. Kluetz, MD,
  – Associate Director, Oncology Center of Excellence
• Michelle Campbell, PhD
  – Reviewer and Scientific Coordinator, COA Staff
• Elektra Papadopoulos, MD, MPH
  – Associate Director, COA Staff
21st Century Cures Act:
Subtitle A: Patient Focused Drug Development

Theresa Mullin
Director, Office of Strategic Programs

PRO Consortium Workshop
April 26, 2017
Some Background Before 21st Cures Act
Patient-Focused Drug Development (PFDD)

- FDA began developing a more systematic way of gathering patient perspective on their condition and available treatment options
  - Patient perspective helps inform our understanding of the context for the assessment of benefit-risk and decision making for new drugs

- Patient-Focused Drug Development is part of FDA commitments under PDUFA V
  - Conduct 20 public meetings each focused on a specific disease area
  - Each meeting results in a **Voice of the Patient** report that faithfully captures patient input from the various information streams
    - [https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm368342.htm](https://www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm368342.htm)
  - 21 PFDD meetings conducted by FDA as of May 2017

- 21st Century Cures Act of 2016 includes new statutory provisions for Patient Focused Drug Development (under Title III Subtitle A)
- Patients with chronic serious disease are experts on what it is like to
  live with their condition. Their “chief complaints” may not be
  factored into drug development and data collection plans
- FDA plans to engage wider community of patients, researchers and
  drug developers to discuss methodologically sound approaches
  that:
  - Bridge from initial PFDD meetings to more systematic collection of
    patients’ input
  - Generate meaningful input on patients’ experiences and
    perspectives to inform drug development and B-R assessment
  - Are “fit for purpose” in drug development and regulatory context
- FDA plans to provide regulatory guidance
  - On pragmatic and methodologically sound strategies, pathways, and
    methods to gather and use patient input
SEC. 3001. PATIENT EXPERIENCE DATA

STATEMENT OF PATIENT EXPERIENCE

(1) IN GENERAL.—Following the approval of an application that was submitted under section 505(b) of this Act or section 351(a) of the Public Health Service Act at least 180 days after the date of enactment of the 21st Century Cures Act, the Secretary shall make public a brief statement regarding the patient experience data and related information, if any, submitted and reviewed as part of such application.

(2) DATA AND INFORMATION.—The data and information referred to in paragraph (1) are—(A) patient experience data; (B) information on patient-focused drug development tools; and (C) other relevant information, as determined by the Secretary.
Section 3001: Patient Experience Data

SEC. 3001. PATIENT EXPERIENCE DATA (cont.)

(c) PATIENT EXPERIENCE DATA.—For purposes of this section, the term ‘patient experience data’ includes data that—

(1) are collected by any persons (including patients, family members and caregivers of patients, patient advocacy organizations, disease research foundations, researchers, and drug manufacturers); and

(2) are intended to provide information about patients’ experiences with a disease or condition, including—

(A) the impact of such disease or condition, or a related therapy, on patients’ lives; and

(B) patient preferences with respect to treatment of such disease or condition.
Section 3002: Patient-Focused Drug Development Guidance

(a) PUBLICATION OF GUIDANCE DOCUMENTS. Secretary shall... issue draft and final versions of one or more guidance documents, over a period of 5 years, regarding the collection of patient experience data, and the use of such data and related information in drug development.

CONTENTS.—The guidance documents described in subsection (a) shall address—

(1) methodological approaches that a person seeking to collect patient experience data for submission to, and proposed use by, the Secretary in regulatory decision-making may use, that are relevant and objective and ensure that such data are accurate and representative of the intended population, including methods to collect meaningful patient input throughout the drug development process and methodological considerations for data collection, reporting, management, and analysis;

(2) methodological approaches that may be used to develop and identify what is most important to patients with respect to burden of disease, burden of treatment, and the benefits and risks in the management of the patient’s disease;

(3) approaches to identifying and developing methods to measure impacts to patients that will help facilitate collection of patient experience data in clinical trials;
Section 3002: Patient-Focused Drug Development Guidance

PUBLICATION OF GUIDANCE DOCUMENTS — CONTENTS (cont.)

(4) methodologies, standards, and technologies to collect and analyze clinical outcome assessments for purposes of regulatory decision-making;

(5) how a person seeking to develop and submit proposed draft guidance relating to patient experience data for consideration by the Secretary may submit such proposed draft guidance to the Secretary;

(6) the format and content required for submissions under this section to the Secretary, including with respect to the information described in paragraph (1);

(7) how the Secretary intends to respond to submissions of information described in paragraph (1), if applicable, including any timeframe for response when such submission is not part of a regulatory application or other submission that has an associated timeframe for response; and

(8) how the Secretary, if appropriate, anticipates using relevant patient experience data and related information, including with respect to the structured risk-benefit assessment framework described in section 505(d) of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 355(d)), to inform regulatory decision-making.
**Proposed for PDUFA VI: Enhancing Incorporation of Patient’s Voice in Drug Development and Decision-Making**

Develop systematic approaches to bridge from patient-focused drug development meetings to fit-for-purpose tools to collect meaningful patient input that can be incorporated into regulatory review.

**Proposed:**

- Conduct public workshops and develop series of guidance documents on:
  1. Collecting comprehensive patient-community input on burden of disease and current therapy (FY18)
  2. Development of holistic set of disease or treatment impacts most important to patients (FY19)
  3. Development of measures for an identified set of impacts (FY20)
  4. Clinical outcome assessments and better ways to incorporate COAs into endpoints (FY21)
- Revise MAPPs and standard operating procedures and policies (SOPPs) as needed to incorporate increased patient focus
- Repository of info on publicly available tools and ongoing efforts
- Conduct public workshop to gather experiences and recommendations of patients and caregivers on approaches to enhance engagement in clinical trials (FY19)
- Enhance staff capacity to facilitate development and use of patient-focused methods to inform drug development and regulatory decisions
What sorts of topics would we anticipate addressing in the proposed PDUFA VI guidance?

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 trials.
   - 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
Update on Clinical Outcome Assessments in Cancer Clinical Trials and Oncology Center of Excellence

Paul G. Kluetz
Associate Director
Oncology Center of Excellence
Debrief: 2nd Annual C-Path-FDA Workshop on COA in Cancer Trials- April 25, 2017

“Assessing Tolerability of Cancer Treatments: Optimizing the Role of Patient-Reported Data”
Cancer Drug Development Endpoints

• Unlike many other therapeutic areas, cancer is **life threatening** and we can accurately measure the disease

• Most common oncology endpoints
  – Overall Survival
  – Progression Free Survival
  – Objective Response Rate

• For an anti-cancer therapy, PRO measures can play an important, but more complementary role (than a disease whose only manifestation may be symptoms)
Clinical Outcome Assessment

• “Prolongation of life, a better life or an established surrogate for either of the above”

• In addition to survival and tumor measures, clinical outcome assessments including patient reported outcome (PRO) measures have received a lot more attention.
PRO Measures Should Target a Clinical Trial Research Objective

- **Efficacy**: Does the drug provide superior improvement in disease related symptoms or functional deficits?
  - Pain, Total Symptom Score, Performance related outcomes
  - Supports a claim of treatment benefit
  - Requires Substantial evidence from formal statistical analysis (statistical superiority)

- **Safety/Tolerability**: Describe the patient’s experience while exposed to anti-cancer therapy?
  - Patient-reported symptomatic toxicities
  - If not claiming a treatment benefit of comparative tolerability, may use descriptive statistics as is done with CTCAE data
Tolerability =
PRO Measurement Opportunity

• Symptomatic adverse events are best assessed by patients

• Safety and Tolerability- important in all phases of development

• PRO measures can offer different but complementary data to current clinician reported safety data

• PRO measures can be systematically and longitudinally obtained including a baseline measure
2017 Workshop on COA in Cancer Clinical Trials focused on TOLERABILITY

• Session 1: Defining Safety versus Tolerability
• Session 2: Assessment of Safety and Tolerability
• Session 3: Analysis and Display of PRO Tolerability Data
• Session 4: From Individual Symptoms to Overall Side Effect Burden
A Star-Studded Cast of Characters

- **Critical Path Institute PRO Consortium Friends!** Stephen Coons, Sonya Eremenco, Theresa “T” Griffey, Theresa Hall, Sarah Mann, Christian Noll, and Margo Panke
- **Moderators** Bindu Kanapuru, Steven Lemery and Laura Lee Johnson
- **Oncology Program Colleagues** Valerie Vashio and Dianne Spillman
- **Patient Representatives** Randy Hillard, Karen Arscott, Chris Blackburn, Mary Lou Smith
- **Academic Experts** Crystal Denlinger, Lori Minasian, Gita Thanarajasingam, Diane Fairclough, Corneel Coens, Sandy Mitchell, David Cella, Charlie Cleeland, Galina Velikova and Ethan Basch
- **International Regulators** Dan O’Connor, Kathy Soltys and Yolanda Barbachano
- **Industry Scientists** Eric Rubin, Sheetal Patel, Anna Ryden and Joe Cappelleri
- **FDA Colleagues:** Selena Daniels, Raji Sridhara, Michelle Campbell
• An Update on the Oncology Center of Excellence
Oncology Center of Excellence (OCE)

- FDA Inter-center Institute as Part of 21st Century Cures Act
- Integrated approach to clinical evaluation of cancer products
- Leverages combined skills of regulatory scientists and reviewers from the 3 key centers who review cancer products

Center for Drug Evaluation and Research (CDER)
- Drugs and Antibodies.
- Office of Hematology and Oncology Products

Center for Biologics Evaluation and Research (CBER)
- Cellular and Gene Therapies, Vaccines.

Center for Devices and Radiologic Health (CDRH)
- Devices, In Vitro Diagnostics, Diagnostic and Therapeutic Radiologics.
• The OCE is dedicated to advancing patient-focused drug development (PFDD), establishing a PFDD program as part of its initial roll-out.
OCE’s PFDD Program- Mission Statement:

Cancer therapies are developed to treat patients, not their disease. Cancer patients experience disease symptoms and symptomatic treatment side effects that can impact their ability to function and other aspects of their health related quality of life.

The Oncology Center of Excellence PFDD program fosters collaboration between FDA Centers and external stakeholders involved in patient outcomes research in cancer populations. The OCE PFDD program focuses on three key areas: Actively engaging with patients and advocacy groups, fostering research into measurement of the patient experience, and generating science-based recommendations for regulatory policy. The overarching goal is to identify rigorous methods to assess the patient experience that will complement existing survival and tumor information to better inform a cancer therapy’s effect on the patient.

https://www.fda.gov/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/OCE/ucm544143.htm
Oncology Center of Excellence (OCE)
Patient Focused Drug Development (PFDD) Program

PFDD Education and Outreach
- Symposia/Workshops
- Reviewer Education
- Patient Engagement

PFDD Science
- Analysis and Presentation Methods
- Real-World COA Data
- Preference Data
- Wearable Devices

PFDD Regulatory Policy
- Consistency of advice
- SOPs, Guidances
- Review Practices

CDRH

CBER

CDER
- COA Staff
- Office of Biostatistics
- Office of Prescription Drug Promotion
- Office of Strategic Programs
- Office of Medical Policy
Oncology PFDD Education and Outreach
- Symposia/Workshops
- Reviewer Education
- Patient Engagement
Patient Engagement Should be a Dialogue

**Patients**
- Experts in how they experience their disease
- Identify what matters most to patients
- Identify areas to make clinical trials more patient-friendly

**Clinicians/Trialists/Health Policy Leaders**
- Experts in clinical trial design and conduct
- Medical expertise
- Assess feasibility of trial modifications and outcome measures

Patient-centered
Scientifically Rigorous
Drug Development
PFDD Education-Patient Engagement

• **FDA- Educating the Patient Community:**
  – Annual FDA101 Patient and Advocate Workshop under development

• **The Patient Community- Educating the FDA:**
  – Recurring Disease Focused Patient Advocate Symposia

• **Building FDA Reviewer Expertise in PRO Measurement Science**
  – Clinical Outcome Assessment New Reviewer Training
  – Educational Repositories for COA/Preference Lectures, Podcasts, Videos
Oncology PFDD Science
- Analysis and Presentation Methods
- Real-World COA Data
- Preference Data
- Wearables and Other New Technologies
Efforts at Advancing PRO and other Clinical Outcomes Requires Multiple Disciplines in the Room

**Clinicians**
Disease and Treatment Expertise

**Social Scientists**
Measurement Tool and Analytic Expertise

**Statisticians**
Trial Design and Data Analytic Expertise

**Patients**
Oncology PFDD Regulatory Policy
- Consistency of advice
- Guidance Development
- Standard Review Practices
Some Exciting Work Underway

• **Bringing Together Experts in PRO Measurement Science**
  – Annual COA in Cancer Clinical Trials Workshop

• **Contributing to Multiple International Collaborations**
  – SPIRIT-PRO
  – SISAQOL

• **Exploring Methods to Leverage Technology**
  – Encouraging work in ePRO, PRO data standards, wearable devices

• **FDA Oncology PFDD Science Core**
  – FDA social scientists, statisticians and clinicians working together!

• **Exploring “Real World” PRO Data**

• **Contributing to Guidance and Leading an Effort to Standardize Oncology PRO Review Practices**
Conclusion

• Great momentum to advance the science of PRO measurement, analysis and presentation

• Oncology Center of Excellence has prioritized patient-focused drug development as one of its initial programs

• We will continue to seek international collaboration to advance measurement of the patient experience
Clinical Outcome Assessment Drug Development
Tool Qualification Program Updates

Michelle Campbell, PhD
Reviewer and Scientific Coordinator
COA Staff
Office of New Drugs
Center for Drug Evaluation and Research
COA Staff

• **Associate Director for Clinical Outcome Assessments:**
  – Elektra Papadopoulos, MD, MPH

• **Regulatory Project Manager:**
  • Susan Pretko, PharmD, MPH, BCPS

• **DDT Qualification Scientific Coordinator:**
  – Michelle Campbell, PhD

• **Team Lead**
  – Selena Daniels, PharmD, MS

• **Reviewers:**
  – Michelle Campbell, PhD
  – Wen-Hung Chen, PhD
  – Yasmin Choudhry, MD
  – Ebony Dashiell-Aje, PhD
  – Sarrit Kovacs, PhD
  – Nikunj Patel, PharmD

• **ORISE Fellow:**
  – Paula Chakravarti, MPH, MS, MA
# COA Qualification Projects

<table>
<thead>
<tr>
<th>COA DDT Qualification Program Stage</th>
<th>Number in Stage as of Q1-2017</th>
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<td>Initiation Stage</td>
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<td>Consultation and Advice (C&amp;A) Stage</td>
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<td>Review Stage</td>
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<td>Total</td>
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<td>Qualified for Use in Exploratory Studies</td>
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### Number of DDT Projects by Division as of Q1-2017

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DDT Submissions 2014-2016

Number of submissions received requiring a full QRT meeting, excluding teleconferences and meeting requests

<table>
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<th>Year</th>
<th>Number of Submissions Received</th>
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<td>32</td>
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<td>2015</td>
<td>37</td>
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<td>2016</td>
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**Legend:**
- DDT: Dopamine D2/3 Receptors
- QRT: Quality Research Team
Qualification Program Activity 2014-2016

Number of Actions Received/Taken

- 2014: 32
- 2015: 37
- 2016: 59
FOLLOW UP FROM LAST YEAR
Quantitative Analysis Plans

Last Year our goal was to develop an outline of what minimum evidence required in these plans
Clinical Outcome Assessment (COA): Frequently Asked Questions

1. What is the standard of evidence for COA qualification?

The measurement principles of content validity, reliability, construct validity, and ability to detect change apply to all types of COAs. The PRO guidance, while developed for patient-reported outcomes, provides many recommendations that are applicable to the development of all COAs, including clinician-reported outcome (ClinRO) assessments, observer-reported outcome (ObsRO) assessments, and performance outcome (PerFO) assessments. In addition, we often refer instrument developers to the ISPOR Task Force publications on content validity.

The COA Wheel and Spokes (PDF - 1MB) provided here identifies the key components of various stages of instrument development and the points at which qualification may occur.

2. What quantitative information is useful to provide for Agency review to support a clinical outcome assessment (COA) drug development tool (DDT) for qualification for exploratory use? (Spoke III)

This stage of instrument development typically involves cross-sectional quantitative (psychometric) analysis. The primary objective of these analyses, in conjunction with the qualitative data, is to select items and refine the conceptual framework of the instrument for further confirmatory evaluation. Each quantitative analysis planned should provide evidence that the items perform well psychometrically and together they assess what the instrument is intended to assess, i.e., the concept(s) described in the COA conceptual framework. The analysis results should inform the retention or removal of items, refinement of the conceptual framework, and the development of provisional scoring algorithm(s). The Agency encourages the submitter to focus on basic analyses and build the evidence methodically using a systematic approach. The quantitative evidence described below should be gathered in a sample of patients with characteristics consistent with the targeted patient population expected in trials and targeted context of use:

1. Item descriptive statistics including frequency distribution of all item responses and overall scores, floor and ceiling effect, and percentage of missing response
2. Inter-item relationships and dimensionality analysis (e.g., factor analysis or principal component analysis and evaluation of conceptual framework)
3. Item inclusion and reduction decisions, identification of subscales (if any), and modification to conceptual framework
2. What quantitative information is useful to provide for Agency review to support a clinical outcome assessment (COA) drug development tool (DDT) for qualification for exploratory use? (Spoke III)

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1. Item descriptive statistics including frequency distribution of both item response and overall scores, floor and ceiling effect, and percentage of missing response
2. Inter-item relationships and dimensionality analysis (e.g., factor analysis or principal component analysis and evaluation of conceptual framework).
3. Item inclusion and reduction decisions, identification of subscales (if any), and modification to conceptual framework
4. Preliminary scoring algorithm (e.g., include information about evaluation of measurement model assumptions, applicable goodness-of-fit statistics). The scoring algorithm should also include how missing data will be handled.
5. Reliability
   a. Test-retest (e.g., intra-class correlation coefficient)
   b. Internal consistency (e.g. Cronbach’s alpha)
   c. Inter-rator (e.g. kappa coefficient)
6. Construct validity
   a. Convergent and discriminant validity (e.g., association with other instruments assessing similar concepts)
   b. Known groups validity (e.g., difference in scores between subgroups of subjects with known status)
7. Score reliability in the presence of missing item-level and if applicable scale-level data
8. Final instrument, conceptual framework, provisional scoring algorithm for exploratory use, and plans for further revision and refinement
Remember - Letter of ???

- Instrument is accepted into the qualification program and is currently in the advice and consultation stage

- FDA has reviewed and agrees with successful development of a draft instrument based on strong qualitative research that includes completion of both concept elicitation and cognitive debriefing

- There is a preliminary conceptual framework and preliminary scoring algorithm available

**Currently:**

- Name: Letter of Continued Progress

- On-hold until implementation of 21st Century Cures is determined
COA Compendium

• FDA review of public comments to dockets:
  – Thirty three (n=33) public comment submissions (academia, industry, CROs, professional societies, independent consultants, and patient advocacy groups) received
  – Majority of the comments supportive of the COA Compendium initiative
  – Range of ideas about COA Compendium expansion and how to optimize its utility

• Goal: Prospectively expand the COA Compendium
Critical Path Innovation Meetings*

• Provides an example of how FDA has responded to the request for earlier COA communications

• Voluntary process that can be used as a venue for a discussion of the potential approaches to developing COAs to provide evidence of treatment benefit

• What it’s not:
  • Not a venue for regulatory advice on a specific product development program

*CPIM topics can include: biomarkers, COAs, natural history studies, innovative approaches to clinical trial design and analysis and others.
CDER Clinical Outcome Assessment Program Updates

PRO Consortium 8th Annual Workshop
April 26, 2017

Elektra J. Papadopoulos, MD, MPH
Associate Director Clinical Outcome Assessments Staff
Office of New Drugs
Center for Drug Evaluation and Research
Outline

• Efforts toward leveraging PROMIS® measures for unmet public health needs

• Expert workshops in emerging areas of need (2016-2017)

• Highlights of 21st Century Cures Act Section 3011

Patient–Reported Outcome Measurement Information System (PROMIS®)
http://www.nihpromis.com/default
Leveraging PROMIS® for unmet public health needs
A Win for Patient Voice in Rheumatoid Arthritis

• FDA agreed (10/2016) to accept the Letter of Intent for a fatigue severity PRO measure in adults with clinically defined rheumatoid arthritis

• PROMIS® Fatigue Short Form 7a (and/or computer adaptive tests) proposed
• Working with Northwestern University to develop a PROMIS® short form for assessment of physical function for use in patients with advanced solid tumors and hematologic malignancies
PROMIS® in Pediatrics

• Pediatric Patient Reported Outcomes in Chronic Diseases (PEPR) Consortium

• Letters of Intent currently under review:
  – DDT 92 PROMIS® Pediatric SF Fatigue (Crohn’s disease)
  – DDT 93 PROMIS® Pediatric Crohn’s Disease Short Form - Pain Interference
  – DDT 95 PROMIS® Pediatric Short Form - Fatigue (children with chronic kidney disease)
Duke-Margolis Center for Health Policy
Expert workshops
2016-2017
Performance Outcome Assessments (PerfOs)

• Duke Margolis Expert PerfO Meeting (Dec 7 and 8, 2016)*
• Some key take-aways:
  – PerfO definition is in evolution
  – Evidentiary considerations for a PerfO include:
    • How closely it is linked to real-world functioning?
    • What is the context (e.g., multinational trials, special populations, demographic characteristics, etc.)?
  – Input from a wide range of stakeholders is valuable
  – Training and standardization is emphasized
    • Patients and administrators should be able to understand the PerfO tasks and perform them consistently
  – Interpretation of score change: Similar considerations to PROs, but with additional challenges
• White paper in progress

* https://healthpolicy.duke.edu/events/developing-and-implementing-performance-outcome-assessments-evidentiary-methodological-and
2017 Duke-Margolis Expert Workshops

• April 4, 2017: COAs: Establishing and Interpreting Meaningful Within-Patient Change
• April 5, 2017: Developing Personalized COAs

– Meeting summaries to be posted
21\textsuperscript{st} Century Cures Act Section 3011
21st Century Cures Act

• Signed into law: December 13, 2016

• Adds new section 507 to the Food, Drug, and Cosmetic Act (FD&C Act) concerning the qualification of DDTs
  – Subtitle B—Advancing New Drug Therapies
  – Sec. 3011. Qualification of drug development tools

• Legislation establishes new processes for qualification of DDTs (biomarkers and clinical outcome assessments)

Key provisions of sec. 3011

- Process
- Engagement of external experts
- Transparency
Process:
Three Submission Milestones

• Letter of Intent

• Qualification Plan

• Qualification Package
Prioritization of Qualification Review

• (i) as applicable, the severity, rarity, or prevalence of the disease or condition targeted by the drug development tool and the availability or lack of alternative treatments for such disease or condition; and

• (ii) the identification, by the Secretary or by biomedical research consortia and other expert stakeholders, of such a drug development tool and its proposed context of use as a public health priority.
Engagement of External Experts

• The Secretary may, for purposes of the review of qualification submissions, through the use of cooperative agreements, grants, or other appropriate mechanisms, consult with biomedical research consortia and may consider the recommendations of such consortia with respect to the review of any qualification plan submitted...
Transparency

Secretary shall make publicly available, and update on at least a biannual basis, on the Internet website of the Food and Drug Administration the following:

‘‘(A) Information with respect to each qualification submission under the qualification process...

(i) stage of the review process applicable to the submission;
(ii) date of the most recent change in stage status
(iii) whether external scientific experts were utilized in the development of a qualification plan or the review of a full qualification package
(iv) submissions from requestors under the qualification process under subsection (a), including any data and evidence contained in such submissions, and any updates to such submissions.
Timing

- Not later than 3 years after the date of the enactment of this Act, the Secretary shall issue draft guidance...

- The Secretary shall issue final guidance on the implementation of such section not later than 6 months after the date on which the comment period for the draft guidance closes
Summary

• Legislation highlights the role of DDT qualification as a way of encouraging innovation and streamlining drug development
• Emphasizes the importance of partnering with external stakeholders, including consortia
• Increases transparency of the qualification process by calling for public posting of information on FDA website
  – BUT: FDA thinking on extent and timing of posting of information still under development
• Increases predictability through inclusion of timelines
Questions or Comments?