SECOND ANNUAL PATIENT-REPORTED OUTCOME (PRO) CONSORTIUM WORKSHOP

March 15, 2011 • Silver Spring, MD

Co-sponsored by

[Logos for CRITICAL PATH INSTITUTE and FDA]
Drug Development Tools Qualification Guidance

ShaAvhrée Buckman-Garner, M.D., Ph.D.
Director
Office of Translational Sciences
CDER/FDA

SECOND ANNUAL
PATIENT-REPORTED OUTCOME (PRO) CONSORTIUM WORKSHOP

March 15, 2011 ■ Silver Spring, MD
Co-sponsored by

CRITICAL PATH INSTITUTE
FDA

collaborate • innovate • accelerate
Outline

- Timeline for development of DDT Guidance
- Key points covered in the Guidance
- Qualification Processes
- Response to draft guidance
- Next steps
## DDT Guidance Development

<table>
<thead>
<tr>
<th>Date</th>
<th>Event</th>
</tr>
</thead>
<tbody>
<tr>
<td>April 2009</td>
<td>Meeting with Dr. Woodcock to discuss PRO/Instrument/Tool Qualification Process</td>
</tr>
<tr>
<td>May 2009</td>
<td>Kick off meeting w/ Working group</td>
</tr>
<tr>
<td>June 2009</td>
<td>Working group created</td>
</tr>
<tr>
<td>Dec 2009</td>
<td>Working group completes Draft Guidance</td>
</tr>
<tr>
<td>July 2010</td>
<td>Guidance circulated and vetted within CDER</td>
</tr>
<tr>
<td>Oct 2010</td>
<td>Docket closed</td>
</tr>
<tr>
<td>Jan 2011</td>
<td>Anticipate finalization of Guidance</td>
</tr>
<tr>
<td>Feb 2011</td>
<td>Working group reviews public comments</td>
</tr>
<tr>
<td>June 2011</td>
<td>Guidance Published as draft</td>
</tr>
</tbody>
</table>
Qualification Process for DDTs

Guidance for Industry

Qualification Process for Drug Development Tools

DRAFT GUIDANCE

This guidance document is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 90 days of publication in the Federal Register of the notice announcing the availability of the draft guidance. Submit comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the Federal Register.

For questions regarding this draft document contact (CDER) Shaniece Gathers, 301-796-2600.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

October 2010
Clinical/Medical

Published October 22, 2010
DDT Guidance

• What the DDT Guidance addresses.....
  • Qualification is a conclusion that within the stated context of use, the results of a DDT measurement can be relied upon to have a stated interpretation and utility

• What the DDT Guidance does not address....
  • Evidentiary standards
  • DDTs as part of regulatory applications for a specific drug development program
Qualification Guidance for Drug Development Tools

- High-level document
- Biomarkers and Patient Reported Outcome Instruments
- Definitions for biomarkers, PROs and qualification
- Rationale for qualification
- Process
- Procedures for making recommendations available
- Does not discuss evidentiary standards
Letter of Intent
(Feasibility Document)

Appropriate to proceed?

Yes

Submission of Scoping Stage Summary Document

Yes

Appropriate to proceed?

Yes

Submission of Qual. Research Summary

Yes

Submission of Quant. Research Summary

PRE-QUALIFICATION

No

Feedback to DDT Sponsor

QUALIFICATION

No

Feedback to DDT Sponsor
Receive Qualification “Dossier”

Initiation of QRT Review

Mid-cycle review meeting held for QRT members

Additional Scientific Discussion Needed?

Yes: Additional Scientific Discussion Needed?

No: Final Review Distributed to Offices for Center Review

CDER Regulatory Briefing

CDER Regulatory Briefing Concurrence with QRT?

Yes: CDER Regulatory Briefing Concurrence with QRT?

No: Advisory Committee or Scientific Workshop for broader scientific discussion

Qualification Letter Developed and Cleared

Qualification Published in FR and posted on website

Scientific Discussion held in appropriate manner
CDER Qualification Program Goals

• To support outside groups who are attempting to establish a DDT for generalizable use

• To provide organized structure for interactions in a consistent and responsive manner while minimizing burdens on product review divisions

• Establishes a process for interactions with DDT sponsors
DDT Guidance: Public Comments

• Does the guidance apply to DDTs developed for proprietary use?
• Does the guidance apply only to PROs and Biomarkers?
• Context of Use
• CDRH involvement
Next Steps

- Finalize DDT guidance
- Animal Model Qualification?
- Supporting the PRO Qualification Program
To contact us:

Office of Translational Sciences/CDER/FDA
301-796-2600

shaavhree.buckman@fda.hhs.gov

Questions?