FDA Biomarker Learnings and the Future

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What we said in 2004:

A new product development toolkit — containing powerful new scientific and technical methods such as animal or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical evaluation techniques — is urgently needed to improve predictability and efficiency along the critical path from laboratory concept to commercial product. We need superior product development science to address these challenges — to ensure that basic discoveries turn into new and better medical treatments. We need to make the effort required to create better tools for developing medical technologies. And we need a knowledge base built not just on ideas from biomedical research, but on reliable insights into the pathway to patients.

Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products March 2004

What we said in 2006:

1. Biomarker Qualification. The process and criteria for qualifying biomarkers for use in product development should be mapped. Clarity on the conceptual framework and evidentiary standards for qualifying a biomarker for various purposes would establish the path for developing predictive biomarkers. Stakeholders, including industry, researchers, and patient groups would have a clear idea of what needs to be done to adopt a new biomarker for regulatory use. Such a framework could stimulate biomarker development and, consequently, shorten the time necessary to develop a successful marketing application.

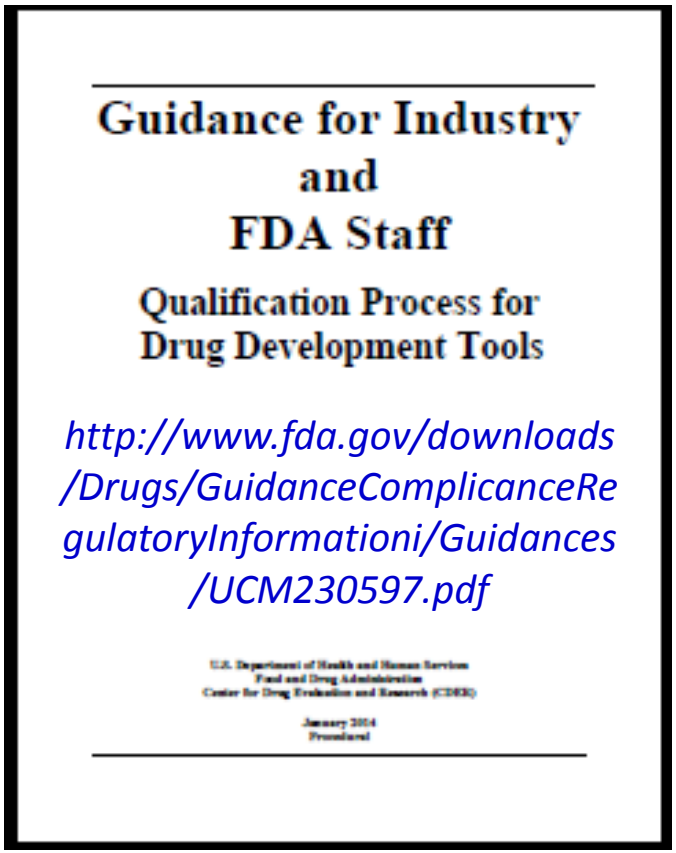
Identifying the framework and evidence needed to qualify biomarkers for different purposes would put an emphasis on correlative and predictive science to accompany the current emphasis on biomarker discovery. Consensus on the following types of questions is needed to put such a framework in place:

- How can biomarker evidence help demonstrate that a candidate product is not too toxic to test in humans?
- How can biomarkers be used to select dose ranges for initial human testing?
- How can biomarkers be used most effectively to evaluate dose response in later trials?
- What biomarker evidence is appropriate to guide selection of patients for clinical testing?
- What types and levels of evidence are needed to accept a biomarker as a surrogate endpoint for product efficacy?

Critical Path Opportunities List March 2006

What has happened since then?

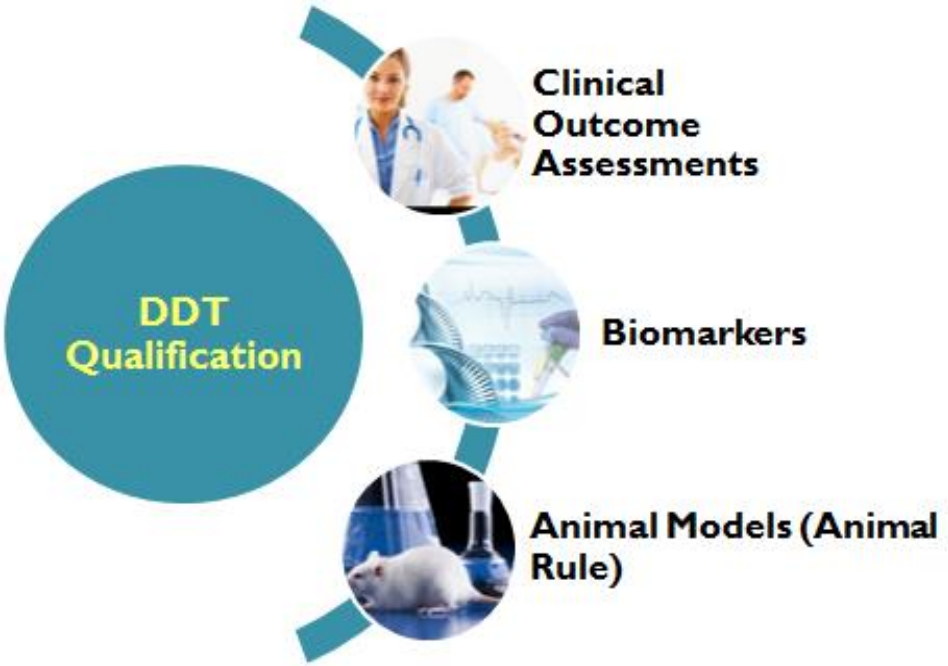
Drug Development Tool Qualification Program



Guidance for Industry
and
FDA Staff

Qualification Process for
Drug Development Tools

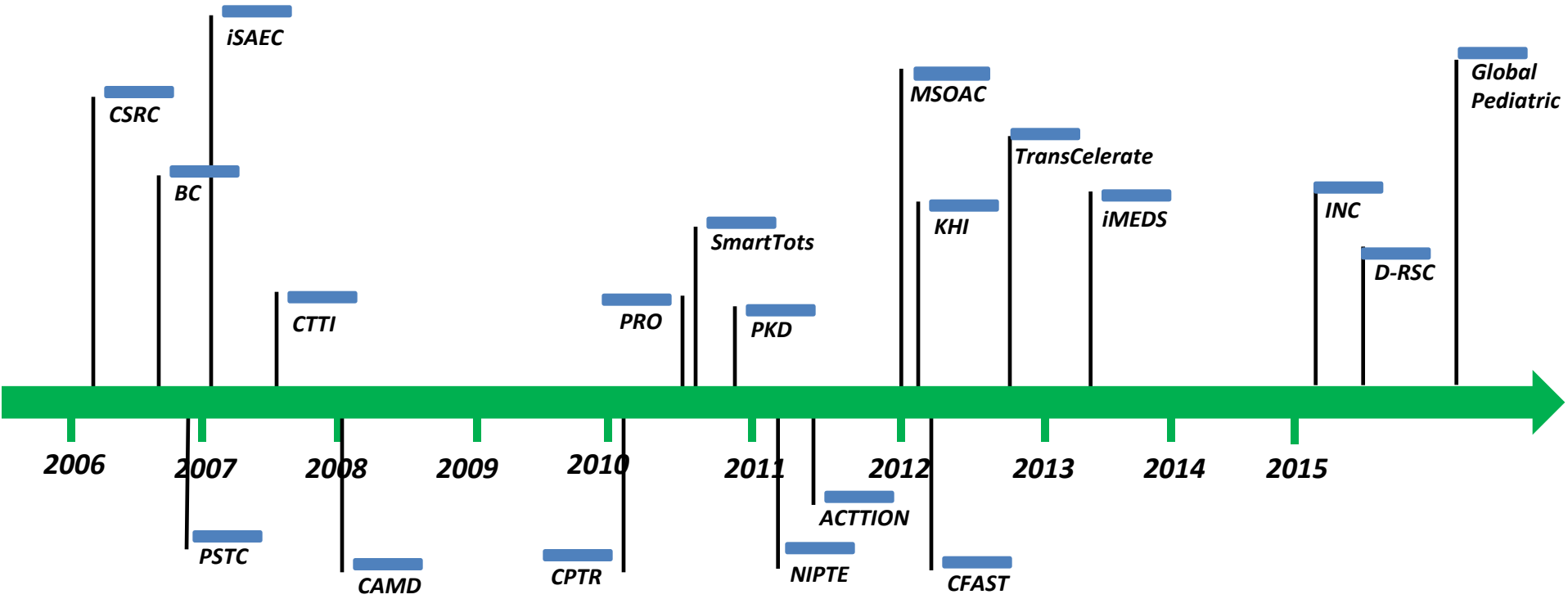
http://www.fda.gov/downloads/Drugs/GuidanceComplicanceRegulatoryInformationi/Guidances/UCM230597.pdf



Why Qualification?

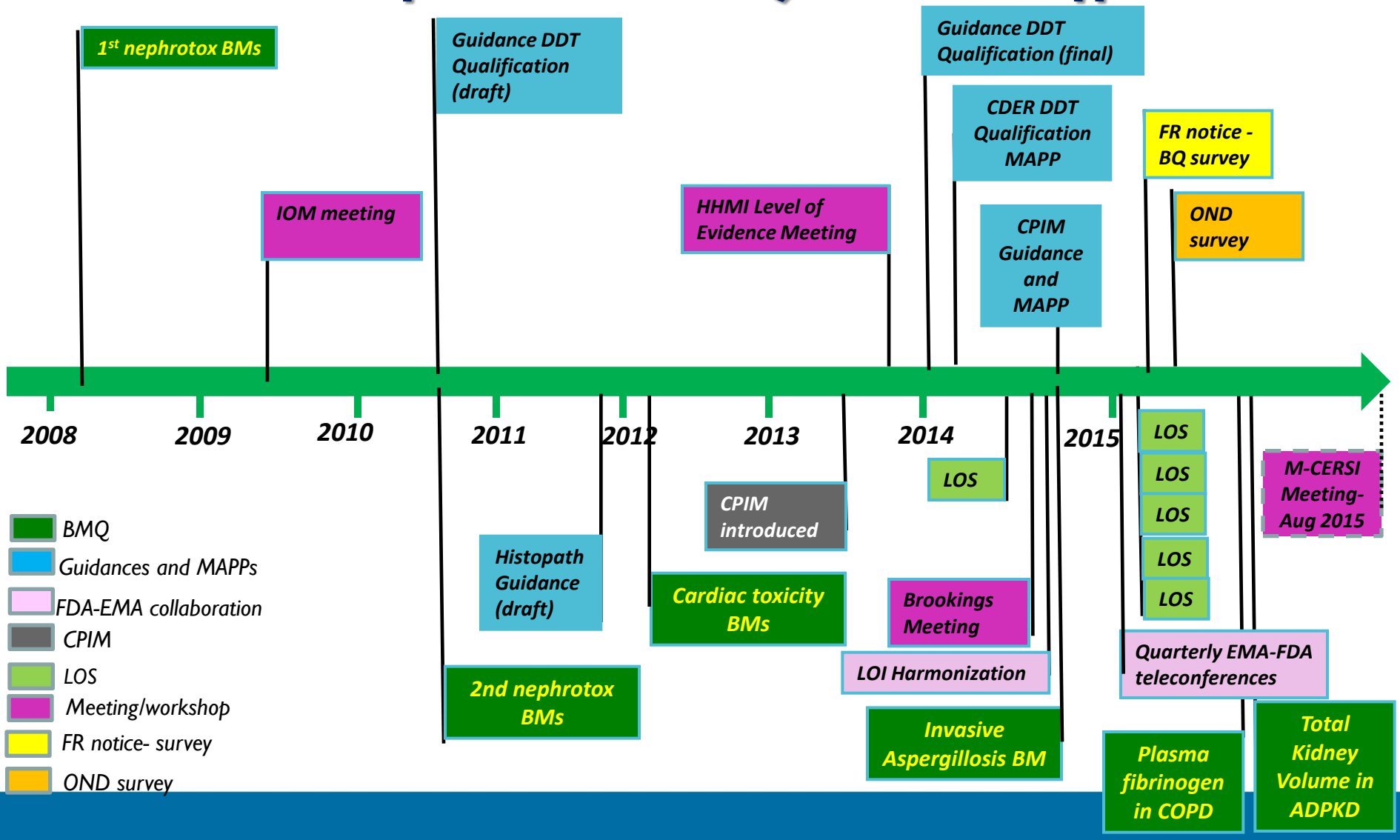
Qualification is a conclusion that within the stated Context of Use (COU), the Drug Development Tool (DDT) can be relied on to have a specific interpretation and application in drug development and regulatory review. The COU describes the way the DDT is to be used and the purpose of the use. Once a DDT has been qualified for a specific COU in drug development, it can be used to produce analytically valid measurements that can be relied on to have a specific use and interpretable meaning. The DDT can be used by drug developers for the qualified context in IND, NDA, and BLA submissions without the relevant CDER review group reconsidering and reconfirming the suitability of the DDT.

Emergence of Consortia



*Cardiac Safety Research Consortium (**CSRC**), Biomarker Consortium (**BC**), Predictive Safety Testing Consortium (**PSTC**), Clinical Trials Transformation Initiative (**CTTI**), Coalition Against Major Disease Consortium (**CAMD**), Critical Path to TB Drug Regimens (**CPTR**) Consortium, Patient Reported Outcomes (**PRO**) Consortium, Polycystic Kidney Disease Outcomes (**PKD**) Consortium, National Institute for Pharmaceutical Technology and Education (**NIPTE**), Analgesic Clinical Trial Translations, Innovations, Opportunities, and Networks Initiative (**ACTTION**), Multiple Sclerosis Outcome Assessments Consortium (**MSOAC**); Kidney Health Initiative (**KHI**), Coalition For Accelerating Standards and Therapies (**CFAST**), Innovation in Medical Evidence Development and Surveillance (**IMEDS**) Program, International Neonatal Consortium (**INC**), Duchenne-Regulatory Science Consortium (**D-RSC**), Global Pediatric Clinical Trials Network Pre-Launch Consortium **(Global Pediatric)***

Timeline for Salient BQ-related Efforts



Current Challenges

- Lack of analytical validation for measuring biomarkers and often a lack of reliable evidence about their performance
- Lack of a common vocabulary and taxonomy for biomarkers
- Inadequate scientific information on the causes, biochemical pathways, and natural histories of many diseases, making identification of disease-specific biomarkers difficult
- Lack of public access to existing research and information on potential biomarkers
- Lack of generally-accepted evidentiary standards for qualifying new biomarkers for particular contexts of use

What have we been doing in the last year?

- Leadership changes for the BQ Program
- Streamlining steps in the process for BQ
- Increased focus on communication with submitters
- Increased focus on communication with CDER staff on the BQRTs
- Harmonization of LOI requirements with EMA
- Setting clear expectations
- Surveys to understand where biomarker development is needed
- Front loading Context of Use discussions
- Letters of Support
- CPIM
- Convening workshops

13 Biomarkers Qualified to Date

General Area	Submitter	Biomarker(s) Qualified for Specific Contexts of Use	Issuance Date with Link to Specific Guidance	Supporting Information
Nonclinical	Predictive Safety and Testing Consortium (PSTC), Nephrotoxicity Working Group (NWG)	Urinary biomarkers: Albumin, β2- Microglobulin, Clusterin, Cystatin C, KIM-1, Total Protein, and Trefoil factor-3	4/14/2008 Drug-induced Nephrotoxicity Biomarkers	Reviews
Nonclinical	International Life Sciences Institute (ILSI)/ Health and Environmental Sciences Institute (HESI), Nephrotoxicity Working Group	Urinary biomarkers: Clusterin, Renal Papillary Antigen (RPA-1)	9/22/2010 Drug-induced Nephrotoxicity Biomarkers	Reviews
Nonclinical	PJ O'Brien, WJ Reagan, MJ York and MC Jacobsen	Serum/plasma biomarkers: Cardiac troponins T (cTnT) and I (cTnI)	2/23/2012 Drug-induced Cardiotoxicity Biomarkers	Reviews
Clinical	Mycoses Study Group	Serum/bronchoalveolar lavage fluid biomarker: Galactomannan	10/24/2014 Patient selection biomarker for enrollment in Invasive Aspergillosis (IA) clinical trials	Reviews
Clinical	Chronic Obstructive Pulmonary Disease (COPD) Biomarker Qualification Consortium (CBQC)	Plasma biomarker: Fibrinogen	7/6/2015 Prognostic biomarker for enrichment of clinical trials in Chronic Obstruction Pulmonary Disease (COPD)	Reviews
Clinical	Polycystic Kidney Disease Outcomes Consortium	Imaging Biomarker: Total Kidney Volume (TKV)	8/17/2015 Prognostic biomarker for enrichment of clinical trials in Autosomal Dominant Polycystic Kidney Disease.	Reviews

Development & Approval Process (Drugs)

Drug Development Tools Qualification Program

Animal Model Qualification Program

Biomarker Qualification Program

Clinical Outcome Assessment Qualification Program

Resources for You

- Biomarker Qualification Context of Use
- Biomarker Qualification FAQ
- Biomarker Qualification Contacts and Submitting Procedures

Letters of Support

What is a Letter of Support?

This is a letter issued to a submitter that briefly describes CDER's thoughts on the potential value of a biomarker and encourages further evaluation. This letter does not connote qualification of a biomarker. It is meant to enhance the visibility of the biomarker, encourage data sharing, and stimulate additional studies.

Why Issue a Letter of Support?

Encouraging the identification and qualification of new drug development tools has been recognized as one of the approaches to overcome hurdles in drug development programs. This approach has the potential to enhance the availability of useful information about drug safety and efficacy. To encourage further development of promising biomarkers which are not yet ready for qualification, FDA may issue a Letter of Support to submitters who have assembled this information about promising biomarkers.

Where Can You Find Issued Letters of Support?

Letters of Support are made publicly available on the FDA's DDT-Biomarker Qualification Program Website.

For more information, please contact CDER-BiomarkerQualificationProgram@fda.hhs.gov.

http://www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugDevelopmentToolsQualificationProgram/ucm412833.htm

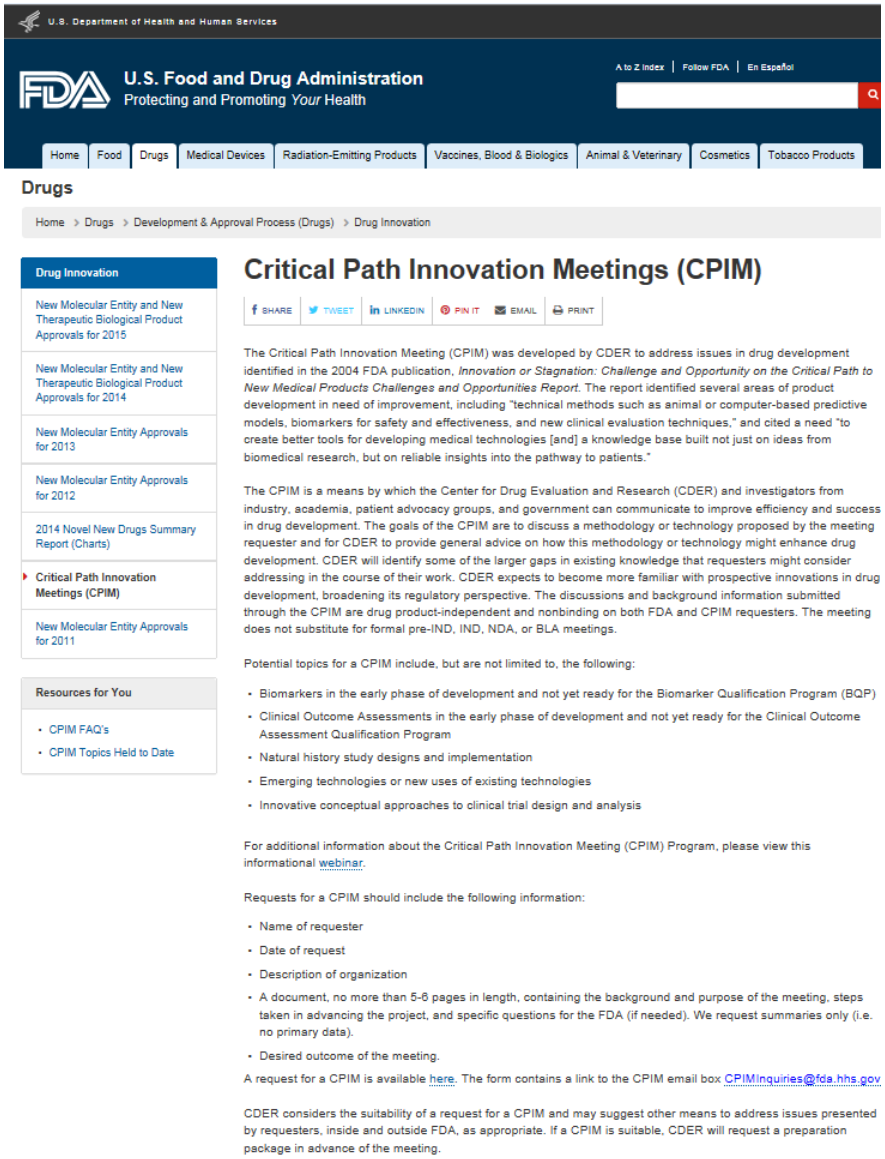
Submitter	Biomarkers	Area(s) for Further Evaluation	Issuance Date with Link to Letter of Support	Submitter Contact
Critical Path Institute's (C-Path) Predictive Safety Testing Consortium (PSTC), Nephrotoxicity Working Group (NWG)	Urinary Biomarkers: Osteopontin and Neutrophil Gelatinase-associated Lipocalin (NGAL)	Early Clinical Drug Development	8/20/2014: Letter of Support (PDF)	Refer to Predictive Safety Testing Consortium Web Site
C-Path, PSTC, Skeletal Muscle Working Group (SMWG)	Serum and Plasma Biomarkers: Myosin Light Chain 3 (Myl3), Skeletal Muscle Troponin I (sTNI), Fatty Acid Binding Protein 3 (FABP3), Creatine Kinase, Muscle Type (CK-M, the Homodimer CK-MM)	Early Clinical Drug Development	1/22/2015: Letter of Support (PDF)	Refer to Predictive Safety Testing Consortium Web Site
C-Path, Coalition Against Major Diseases Consortium (CAMD)	Cerebral Spinal Fluid (CSF) Analyte Biomarkers: $A\beta_{1-42}$, Total tau, Phosphotau	Exploratory Prognostic Biomarkers for Enrichment in Early Stage Alzheimer's Disease Clinical Trials	2/26/2015: Letter of Support (PDF)	Refer to Coalition Against Major Diseases Web Site
C-Path, CAMD	Magnetic Resonance Imaging Biomarker: Low Baseline Hippocampal Volume	Exploratory Prognostic Biomarkers for Enrichment in Early Stage Alzheimer's Disease Clinical Trials	3/10/2015: Letter of Support (PDF)	Refer to Coalition Against Major Diseases Web Site
C-Path, CAMD	Molecular Neuroimaging Biomarker: Dopamine Transporter (DAT)	Exploratory Prognostic Biomarkers for Enrichment in Early Stage Parkinson's Disease Clinical Trials	3/16/2015: Letter of Support (PDF)	Refer to Coalition Against Major Diseases Web Site
C-Path, Polycystic Kidney Disease (PKD) Outcomes Consortium	MRI, Computerized Tomography (CT), or Ultrasound (US) Biomarker: Total Kidney Volume (TKV)	Exploratory Prognostic Biomarker for Enrichment in Autosomal Dominant Polycystic Kidney	4/23/2015: Letter of Support (PDF)	Refer to Polycystic Kidney Disease Outcomes Consortium Web Site
Memorial Sloan-Kettering Cancer Center, Medivation Inc., and Janssen Diagnostics, LLC	Circulating Tumor Cell (CTC) Enumeration	Disease Activity Biomarker for use in Metastatic Castration-resistant Prostate Cancer (mCRPC)	9/25/2015: Letter of Support (PDF)	Debra J. Rasmussen

7 Letters of Support issued to date

Critical Path Innovation Meetings

- Promotes understanding challenges in drug development and innovative strategies to address them
- Potential biomarkers and clinical outcome assessments (COAs) not ready for DDT Qualification Program
- Natural history study design and implementation
- Emerging technologies or new uses of existing technologies
- Novel clinical trial designs and methods
- Nonbinding on FDA and other participants
- No advice on specific approval pathways

Office of Translational Sciences
Critical Path Innovation Meeting



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Drug Innovation

- New Molecular Entity and New Therapeutic Biological Product Approvals for 2015
- New Molecular Entity and New Therapeutic Biological Product Approvals for 2014
- New Molecular Entity Approvals for 2013
- New Molecular Entity Approvals for 2012
- 2014 Novel New Drugs Summary Report (Charts)
- **Critical Path Innovation Meetings (CPIM)**
- New Molecular Entity Approvals for 2011

Resources for You

- CPIM FAQ's
- CPIM Topics Held to Date

Critical Path Innovation Meetings (CPIM)

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The Critical Path Innovation Meeting (CPIM) was developed by CDER to address issues in drug development identified in the 2004 FDA publication, *Innovation or Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products Challenges and Opportunities Report*. The report identified several areas of product development in need of improvement, including "technical methods such as animal or computer-based predictive models, biomarkers for safety and effectiveness, and new clinical evaluation techniques," and cited a need "to create better tools for developing medical technologies [and] a knowledge base built not just on ideas from biomedical research, but on reliable insights into the pathway to patients."

The CPIM is a means by which the Center for Drug Evaluation and Research (CDER) and investigators from industry, academia, patient advocacy groups, and government can communicate to improve efficiency and success in drug development. The goals of the CPIM are to discuss a methodology or technology proposed by the meeting requester and for CDER to provide general advice on how this methodology or technology might enhance drug development. CDER will identify some of the larger gaps in existing knowledge that requesters might consider addressing in the course of their work. CDER expects to become more familiar with prospective innovations in drug development, broadening its regulatory perspective. The discussions and background information submitted through the CPIM are drug product-independent and nonbinding on both FDA and CPIM requesters. The meeting does not substitute for formal pre-IND, IND, NDA, or BLA meetings.

Potential topics for a CPIM include, but are not limited to, the following:

- Biomarkers in the early phase of development and not yet ready for the Biomarker Qualification Program (BQP)
- Clinical Outcome Assessments in the early phase of development and not yet ready for the Clinical Outcome Assessment Qualification Program
- Natural history study designs and implementation
- Emerging technologies or new uses of existing technologies
- Innovative conceptual approaches to clinical trial design and analysis

For additional information about the Critical Path Innovation Meeting (CPIM) Program, please view this informational webinar.

Requests for a CPIM should include the following information:

- Name of requester
- Date of request
- Description of organization
- A document, no more than 5-6 pages in length, containing the background and purpose of the meeting, steps taken in advancing the project, and specific questions for the FDA (if needed). We request summaries only (i.e. no primary data).
- Desired outcome of the meeting.

A request for a CPIM is available here. The form contains a link to the CPIM email box CPIMInquiries@fda.hhs.gov

CDER considers the suitability of a request for a CPIM and may suggest other means to address issues presented by requesters, inside and outside FDA, as appropriate. If a CPIM is suitable, CDER will request a preparation package in advance of the meeting.

10 meetings held to date

www.fda.gov/Drugs/DevelopmentApprovalProcess/DrugInnovation/ucm444165.htm

Patient Focused Drug Development Initiatives

Fiscal Year 2013	Fiscal Year 2014	Fiscal Year 2015	Fiscal Year 2016-2017
• Chronic fatigue syndrome/ myalgic encephalomyelitis • HIV • Lung cancer • Narcolepsy	• Sickle cell disease • Fibromyalgia • Pulmonary arterial hypertension • Inborn errors of metabolism • Hemophilia A, B, and other heritable bleeding disorders • Idiopathic pulmonary fibrosis	• Female sexual dysfunction • Breast cancer • Chagas disease • Functional gastrointestinal disorders • **Huntington's disease and Parkinson's disease (September 22)** • Alpha-1 antitrypsin deficiency (September 29)	• Non-tuberculous mycobacterial lung infections (October 15) *To be announced* • Alopecia areata • Autism • Hereditary angioedema • Patients who have received an organ transplant • Psoriasis • Neuropathic pain associated with peripheral neuropathy • Sarcopenia

Targeted Drug Development: Why Are Many Diseases Lagging Behind?

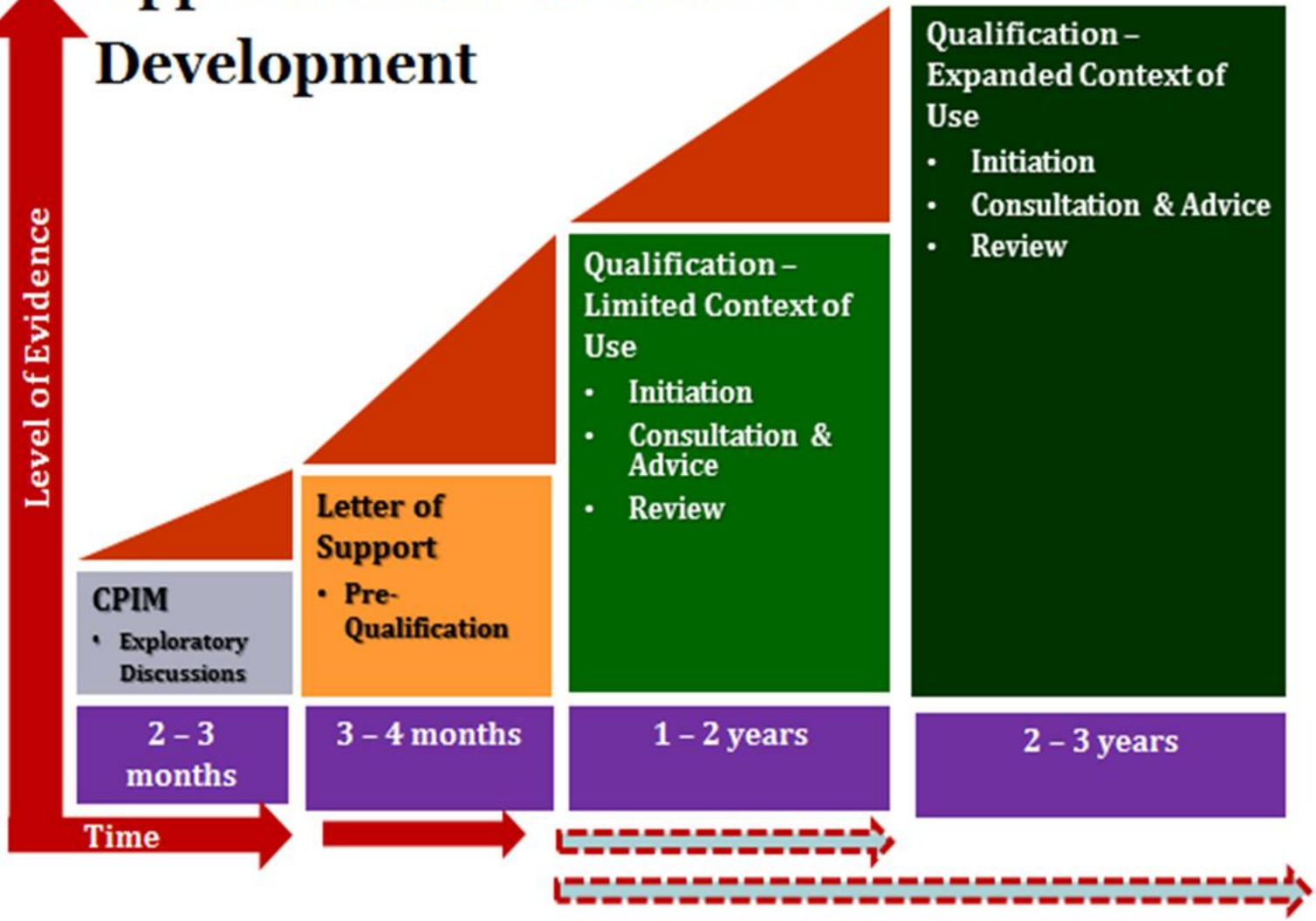
(FDA White Paper, July 2015)

Working with companies. FDA works closely with companies developing Alzheimer's drugs to develop flexible trial designs and more easily interpretable endpoints and, for small companies with less experience, to help them move efficiently through the regulatory process.

Use of unqualified biomarkers for enriched trial designs. To increase the chance that a clinical trial will correctly identify an effective drug in early-stage Alzheimer's, FDA encourages drug sponsors to "enrich" the study population with patients most likely to progress to overt dementia. FDA allows companies to use possible biomarkers, such as amyloid plaque imaging, brain image region measurement, and protein in cerebrospinal fluid as aids to enriching clinical studies with patients who may improve the ability to show the effectiveness of the drug.

Biomarker development. FDA scientists are collaborating with groups including the Alzheimer's Disease Neuroimaging Initiative and the Coalition Against Major Diseases (within the Critical Path Institute) to find biomarkers that can (1) identify Alzheimer's patients before they show symptoms; (2) distinguish among those whose disease will progress more slowly or more quickly; and (3) predict the clinical outcomes of interventions.

Opportunities for Biomarker Development



Drug Development Tools Qualification Programs

Animal Model Qualification Program

Biomarker Qualification Program

Clinical Outcome Assessment Qualification Program

Biomarker Survey Results

FDA Survey to Identify Potential Biomarkers for Qualification

On February 13, 2015, FDA published a survey in the Federal Register entitled, "Identifying Potential Biomarkers for Qualification and Describing Contexts of Use to Address Areas Important to Drug Development." The purpose of the survey was to seek information to inform the development and qualification of biomarkers in areas related to human drug therapeutics. A total of 74 responses were received either online or via submissions to the docket which closed on May 14, 2015.

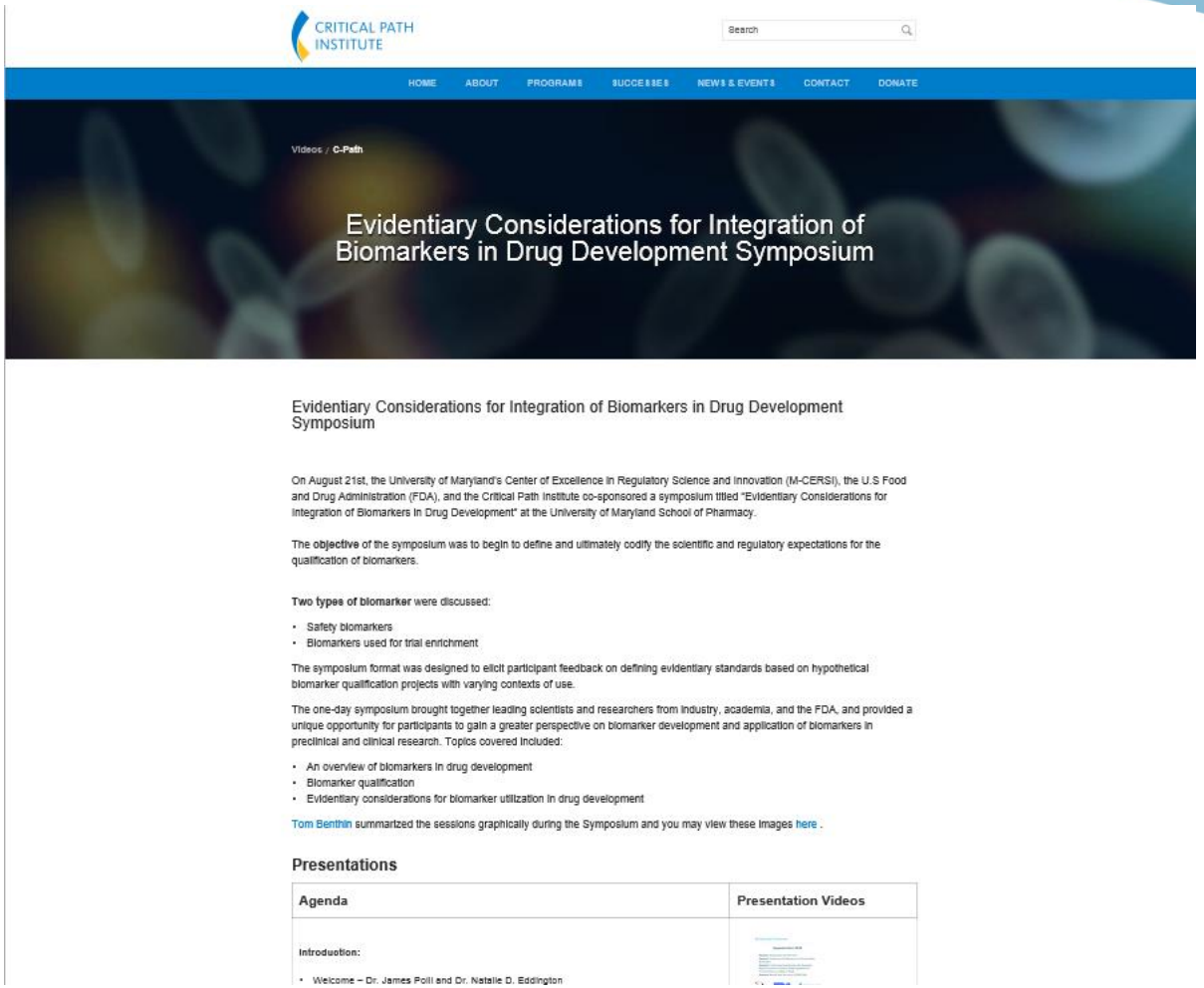
The link to the table below provides a summary of the survey results. It will be linked to an additional larger table that contains more detailed information.
Compiled Survey Results (PDF - 207KB)

If additional information is received, the tables will be updated accordingly.

Comments sent to the docket can be found at: http://www.regulations.gov/#!docketBrowser;rpp=25;po=0;D=FDA-2014-N-2187

Page Last Updated: 08/20/2015
Note: If you need help accessing information in different file formats, see Instructions for Downloading Viewers and Players.

Disease area/Organ toxicity	Specific Areas in Critical Need for Biomarker Development	Biomarker Names	Context of Use	Why Is The Biomarker Useful in Drug Development?
Neurological and Neuropsychiatric Diseases	Alzheimer's disease, Mood Disorders, Epilepsy, Huntington's disease, Alcohol Dependence, Schizophrenia and Parkinson's disease	Tau, Neurofilament proteins, alpha-synuclein, calbindin, Neuron-Specific Enolase, S100b, PSD95, drebrin.	i) Using Some custom biomarker panels (Imaging , Efficacy, Predictive, Diagnostic) as a link to imaging results and to longer term clinical assessments of disease activity/progression. ii) By serving as early signals of efficacy for more rapidly evaluating the impact of potential disease-modifying therapies.	Both neurological and neuropsychiatric disorders suffer from a lack of disease-relevant markers that could be used to assess early signals of efficacy in a clinical setting. Other than imaging modalities like MRS, DTI, PET and MRI, there is a definite need to better monitor drug-mediated effects on neurodegeneration, neuroinflammation and cognition including markers for synaptic plasticity and neurogenesis.
		Neuro-immunology markers	Diagnosis, stratification and outcome measures.	For patient stratification in clinical trials.
		Tau imaging markers		AD diagnosis and staging; progression monitoring, PD measurement.
		Genetic & epigenetic biomarker signatures (AD and Mood disorders)		Disease risk
		Screening biomarkers for AD, Mood disorders (blood tests, neurofunctional and behavioral measurements)		Patient enrichment for clinical trials.



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Evidentiary Considerations for Integration of Biomarkers in Drug Development Symposium

Evidentiary Considerations for Integration of Biomarkers in Drug Development Symposium

On August 21st, the University of Maryland's Center of Excellence in Regulatory Science and Innovation (M-CERSI), the U.S Food and Drug Administration (FDA), and the Critical Path Institute co-sponsored a symposium titled "Evidentiary Considerations for Integration of Biomarkers in Drug Development" at the University of Maryland School of Pharmacy.

The **objective** of the symposium was to begin to define and ultimately codify the scientific and regulatory expectations for the qualification of biomarkers.

Two types of biomarker were discussed:

- Safety biomarkers
- Biomarkers used for trial enrichment

The symposium format was designed to elicit participant feedback on defining evidentiary standards based on hypothetical biomarker qualification projects with varying contexts of use.

The one-day symposium brought together leading scientists and researchers from industry, academia, and the FDA, and provided a unique opportunity for participants to gain a greater perspective on biomarker development and application of biomarkers in preclinical and clinical research. Topics covered included:

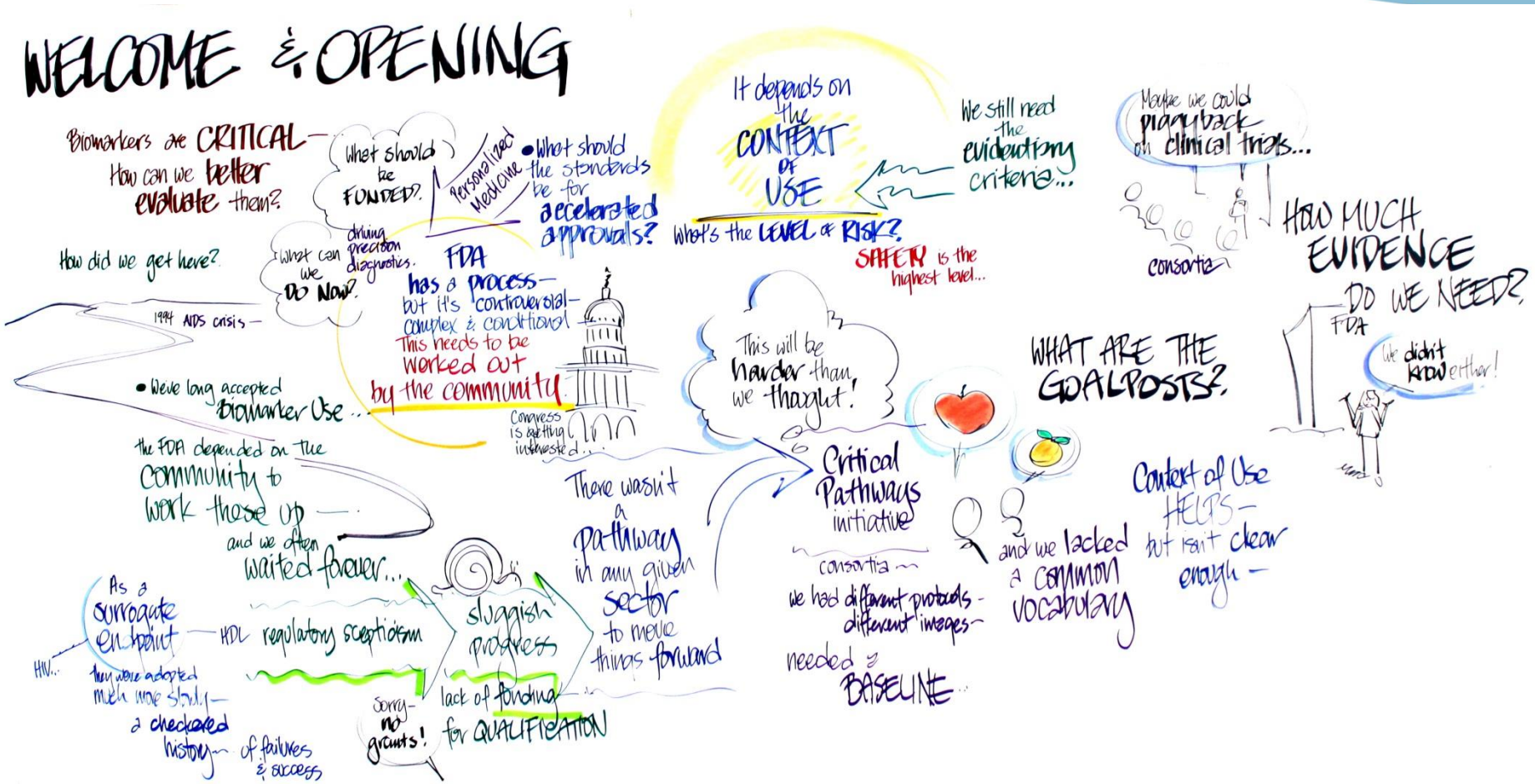
- An overview of biomarkers in drug development
- Biomarker qualification
- Evidentiary considerations for biomarker utilization in drug development

Tom Benthin summarized the sessions graphically during the Symposium and you may view these images here .

Presentations

Agenda	Presentation Videos
Introduction: • Welcome – Dr. James Polli and Dr. Natalie D. Eddington	

http://c-path.org/evidentiary-considerations-for-integration-of-biomarkers-in-drug-development-symposium/


WELCOME & OPENING
Biomarkers are CRITICAL — How can we better evaluate them?
What should be FUNDED?
Personalized Medicine
What should the standards be for accelerated approvals?
It depends on the CONTEXT of USE
What's the LEVEL of RISK?
SAFETY is the highest level...
We still need the evidentiary criteria...
Maybe we could piggyback on clinical trials...
consortia
HOW MUCH EVIDENCE DO WE NEED?
FDA
We didn't know either!
How did we get here?
1984 AIDS crisis —
What can we DO Now?
driving precision diagnostics.
FDA has a process — but it's controversial — complex & conditional
This needs to be worked out by the community.
Congress is getting interested...
• We've long accepted Biomarker Use...
the FDA depended on the community to work these up — and we often waited forever...
This will be harder than we thought!
WHAT ARE THE GOALPOSTS?
Critical Pathways initiative
consortia
we had different protocols - different images - needed a BASELINE...
and we lacked a common vocabulary
Context of Use HELPS — but isn't clear enough —
There wasn't a pathway in any given sector to move things forward
As a surrogate endpoint — HDL
HIV...
they were adopted much more slowly — a checkered history — of failures & success
regulatory skepticism
sluggish progress
lack of funding for QUALIFICATION
sorry — no grants!

Clearing a Path Forward

- Internal biomarker survey *(done)*
- External biomarker survey *(results published on the internet)*
- PhRMA survey *(done)*
- Inventory of biomarkers used in pivotal trials for approved drugs (2007-present) (being compiled)
- Meeting with University of MD and CPath on evidentiary standards *(done)*
- Proposal to the Biomarker Consortium to host an evidentiary standards workshop *(next year)*
- ConsortiaPedia launch (this week)
- Data/specimen repositories *(discussions underway)*

consortia-pedia
a FasterCures project
A CENTER OF THE MILKEN INSTITUTE

Find consortia

You can search the 400+ consortia profiles based on disease area, stakeholder, and other criteria

Go

Help us improve the Consortia-pedia! Do you know of a consortium in medical research that we should include? Do you have edits or updates to an existing profile? Can you tell us how you are using Consortia-pedia?

Submit comments →

Click on the consortium name below to view the full profile

1 | A | B | C | D | E | F | G | H | I | J | K | L | M | N | O | P | Q | R | S | T | U | V | W

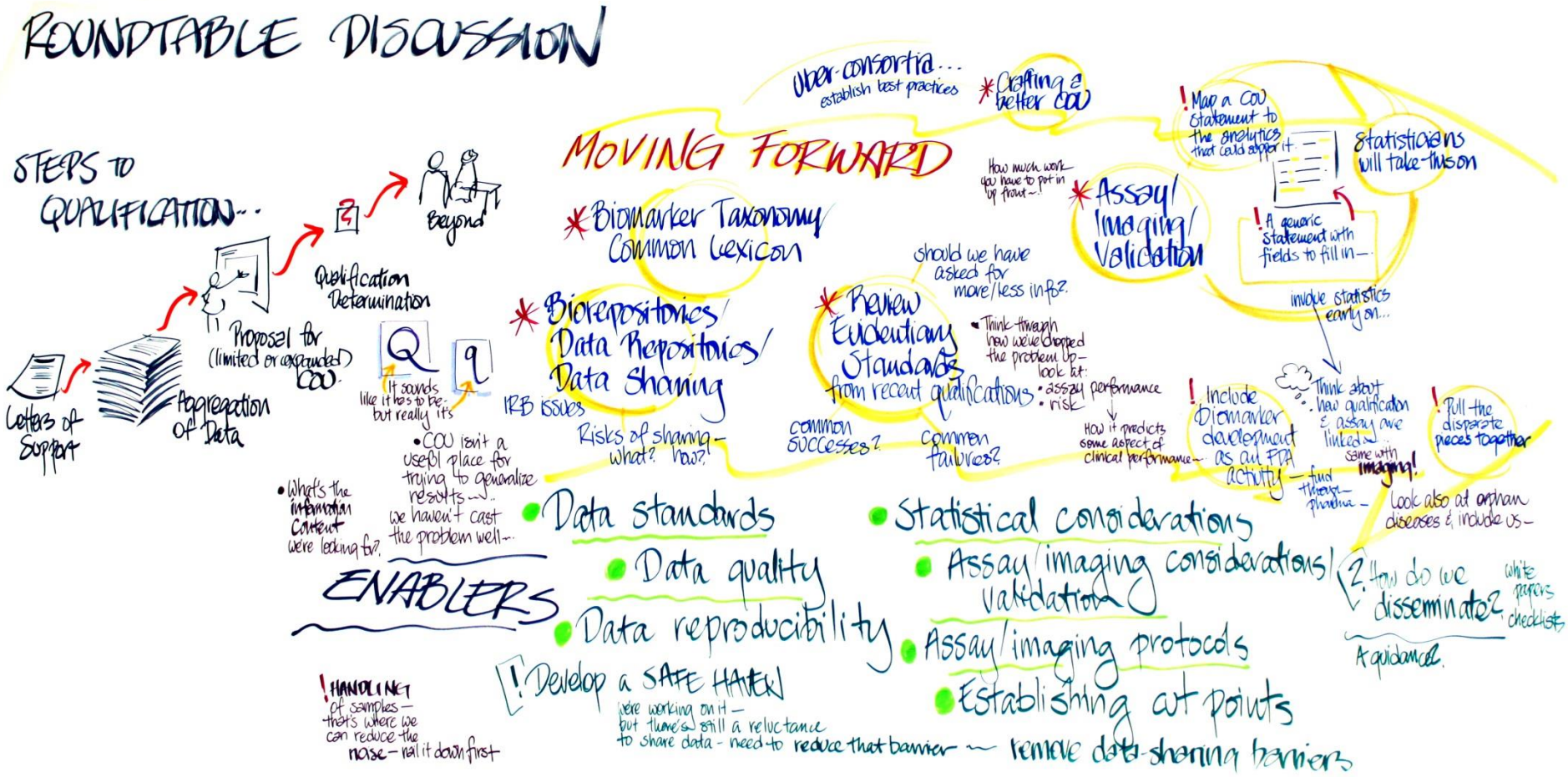
	Tool development	Biomarker research	Basic research	Data-sharing enabler	Product development
1000 Genomes	✓	✓		✓	
Academic Drug Discovery Consortium (ADDC)	✓			✓	
Accelerating Medicines Partnership - Alzheimer's		✓	✓	✓	
Accelerating Medicines Partnership - Autoimmune		✓	✓	✓	
Accelerating Medicines Partnership - Diabetes		✓		✓	
AddNeuroMed	✓	✓			
Advanced Immunization	✓		✓		

Opportunities for Collaboration

- Develop evidentiary standards for context-of-use-specific biomarker qualification
- Prioritize specific diseases and respective biomarkers whose development and qualification would advance drug development and satisfy unmet medical needs
- Expand qualification by developing and maintaining an accessible database for collecting biomarker data, and a repository for samples
- Develop standards for biomarker measurement tools…Reproducibility initiatives…
- Encourage and fund biomedical research that is necessary as the basis for development of new biomarkers
- Coordinate existing partnerships and consortia so that they effectively direct their efforts toward development and qualification of priority biomarkers
- Train investigators on regulatory considerations for biomarker development

Next Steps…What is Needed

- Enhanced data sharing and collaborative efforts among consortia
- Qualification packages that don't try to "boil the ocean"
  - Limited vs Expanded Context of Use
- Data/specimen repositories which can support expanded contexts of use for biomarkers once additional data is aggregated (data standards)
- Up front conversations around context of use—which drives the level of evidence needed
- More communication about the value and progress made by consortia efforts
- Greater clarity around levels of evidence for qualification—this takes the entire scientific community—not just FDA
- Patience…we are in this together and we are learning as we go…


ROUNDTABLE DISCUSSION
STEPS TO QUALIFICATION...
Letters of Support
Aggregation of Data
Proposal for (limited or expanded) COU
Qualification Determination
Beyond
MOVING FORWARD
Uber-consortia... establish best practices
Crafting a better COU
Biomarker Taxonomy/ Common Lexicon
Biorepositories/ Data Repositories/ Data Sharing
IRB issues
Risks of sharing – what? how?
Common successes?
Common failures?
Review Evidentiary Standards from recent qualifications
How much work you have to put in up front...!
Assay/ Imaging/ Validation
Map a COU statement to the analytics that could support it
Statisticians will take-thuson
A generic statement with fields to fill in...
Should we have asked for more/less info?
Think through how we've chopped the problem up – look at: assay performance, risk
How it predicts some aspect of clinical performance
Include Biomarker development as an FDA activity – fund therapy-pharma
Think about how qualification & assay are linked... same with imaging!
Involve statistics early on...
Pull the disparate pieces together
Look also at orphan diseases & include us
What's the information content we're looking for?
COU isn't a useful place for trying to generalize results...
We haven't cast the problem well...
ENABLERS
Data standards
Data quality
Data reproducibility
Develop a SAFE HAVEN
We're working on it – but there's still a reluctance to share data – need to reduce that barrier
HANDLING of samples – that's where we can reduce the noise – nail it down first
Statistical considerations
Assay/imaging considerations/ validation
Assay/imaging protocols
Establishing cut points
How do we disseminate?
White papers
checklists
A guidance?
remove data-sharing barriers

- Shashi Amur
- Jim Kaiser
- Chris Leptak
- Suzie McCune
- Marianne Noone
- Mike Pacanowski
- Ameeta Parekh
- Sarmistha Sanyal
- Alicia Stuart
- Janet Woodcock

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