WITH STANDARDS – UNLOCK THE POWER OF DATA

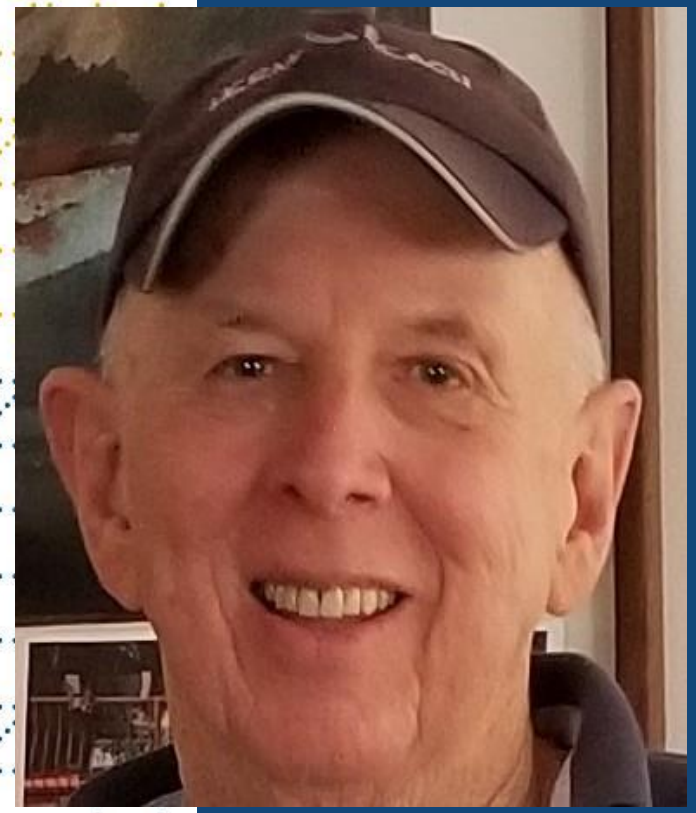
cdisc

2022
JAPAN
INTERCHANGE

13-14 JUNE | VIRTUAL EVENT

FDA CDER Presentation

Presented by Steve Wilson, Senior Staff Fellow, Office of Biostatistics, FDA/CDER/OTS



Meet the Speaker

Steve Wilson, Dr.P.H. (Biostatistics), CAPT USPHS (ret.)

Title: Senior Staff Fellow

Organization: US Food and Drug Administration (FDA)

In 2017, after serving for 30 years as a mathematical statistician, Dr. Wilson, a Captain in the US Public Health Service Commissioned Corps, retired from the FDA. In 2019 Steve returned to the Agency as a Senior Staff Fellow in CDER's Office of Biostatistics.

Dr. Wilson received his doctorate in Biostatistics from the University of North Carolina, Chapel Hill, in 1984. His professional experience also includes positions with the East West Center, the Indonesian Central Bureau of Statistics, the University of North Carolina, the Federated States of Micronesia and the World Bank. His professional interests and activities are currently focused on issues related to the improvements in clinical trials science and practice, data standards, and the regulatory review of drug and biological therapies.

CDISC Disclaimer and Disclosures

- *The views and opinions expressed in this presentation are those of the author(s) and do not necessarily reflect the official policy or position of CDISC.*

- *No disclosures or conflicts of interest to report*

OB/AIS, PDUFA VII and Collaboration: An FDA CDER Presentation

Steve Wilson, Dr.P.H. (Biostatistics)*

Senior Staff Fellow
Office of Biostatistics (OB), OTS/CDER
Food and Drug Administration

June 13, 2022

*Acknowledgement: Matilde Kam, Ph.D., OB/AIS

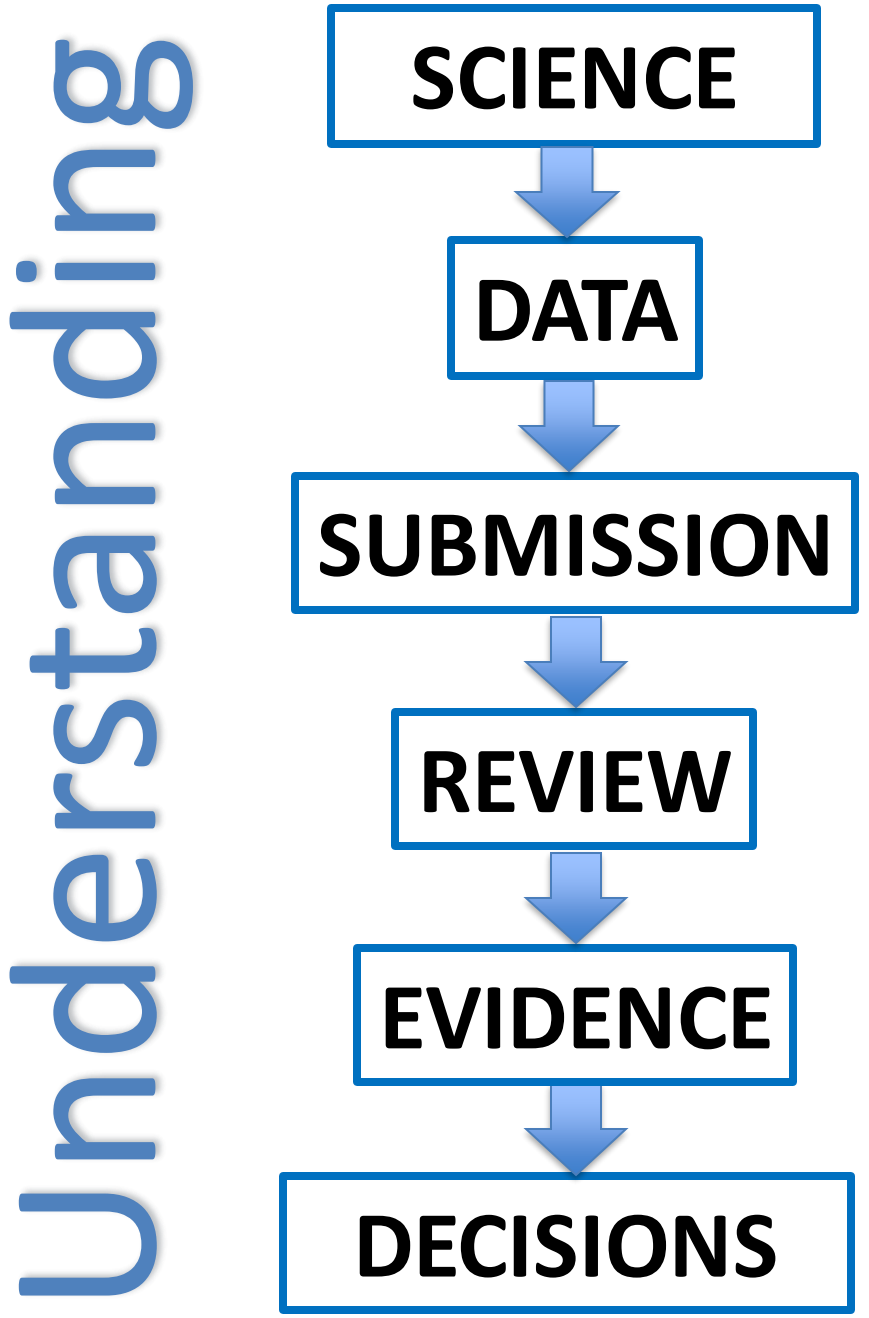
FDA Disclaimer

The views and opinions presented here represent those of the speaker and should not be considered to represent advice or guidance on behalf of the U.S. Food and Drug Administration.

Topics/Agenda

- This Statistical Reviewer's Perspective: Science, Collaboration and Understanding
- 2022 Japan Interchange Program – A CDISC Virtual Meeting
- FDA/CDER/Office of Translational Science (OTS)/Office of Biostatistics (OB)/ Analytics and Informatics Staff (AIS)
- The Evolving Science/Data/Evidence/Decision Landscape
- FDA/CDER Plans and Actions
- Prescription Drug User Fee Act (PDUFA) VII
  - Real World Data/Real World Evidence (RWD/RWE)
  - Rare Diseases
  - Patient Focused Drug Development (PFDD)
  - Digital Health Technology (DHT)
- Collaboration: Opportunities & Experiences
  - PRO-CTCAE
  - Technical Specifications
  - Working with Japanese Colleagues on Data Standards: Some Personal Reflections and Thanks
- CDISC RWD Connect /Report of a Qualitative Delphi Survey
- Closing Comments

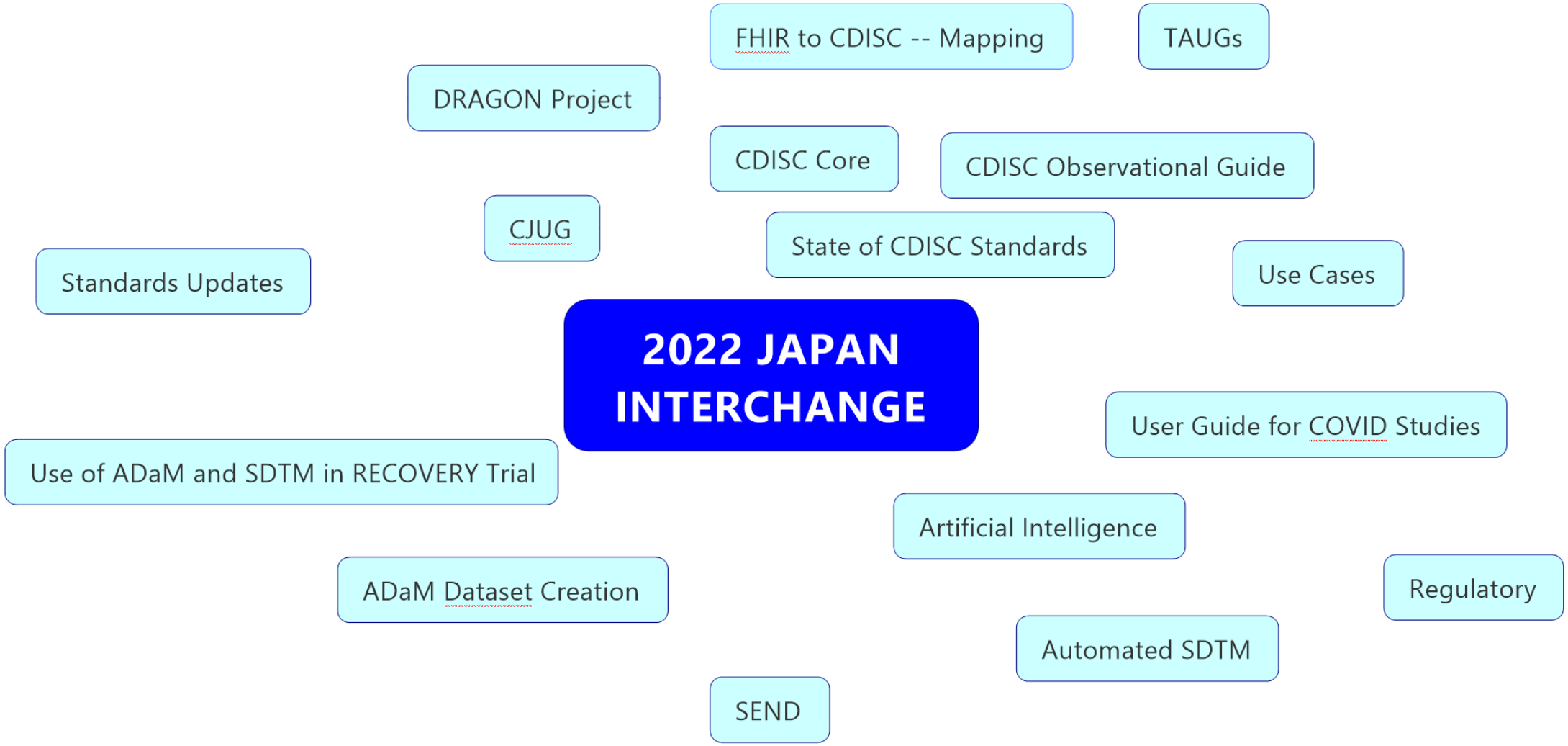
This FDA Statistical Reviewer's Perspective



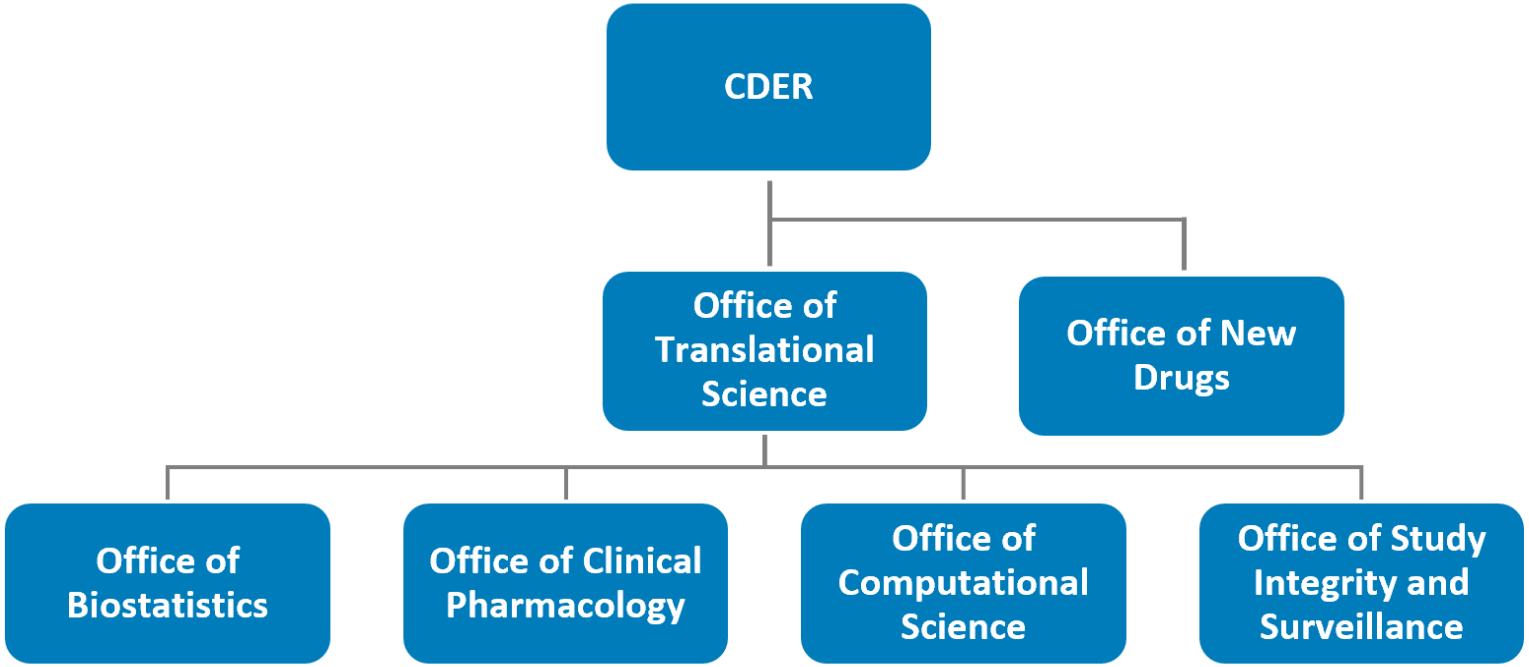
2022 CDISC Japan Interchange Program

- **Welcome Address & State of the CDISC Union**
- Dave Evans, CDISC President and CEO
- **ELSI (Ethical, Legal, and Social Implications) Aspects of Health Database in Japan**
- Ryuichi Yamamoto, MD. PhD. Chief Director, Medical Information System Development Center
- **Session 2: CDISC Updates**
- Takuhiro Yamaguchi, J3C, Tohoku University
- **State of CDISC Standards**
- Bess Leroy, CDISC
- **FHIR to CDISC: Mapping Real World Data Connections**
- Rebecca Baker, CDISC
- **DRAGON Project and the CDISC Observational Guide**
- Jon Neville and Kit Howard, CDISC
- **Session 3: COVID-19 Related Topics**
- Hidetoshi Misawa, J3C, Pfizer
- **Experience with Interim User Guide for COVID Studies & Guidance for Ongoing Studies**
- Arvind Sri Krishna Mani, Zifo RnD Solutions
- **Second Keynote Presentation: Use of SDTM and ADaM in the RECOVERY Trial of Treatments for COVID-19**
- Dr. Will Stevens, Ph.D, Oxford University
- **Session 4: Implementation & Use Cases**
- Hideto Yokoi, J3C, Kagawa University
- **Slashing my first mapping, five years later!**
- Shizuko Takahara, Kanazawa University
- **SDTM Basics for ADaM Dataset Creation**
- Seiko Yamazaki and Chikaaki Nakao, Pinnacle 21
- **Session 5: Regulatory Presentations, Part I**
- Naoto Awaji, J3C, Chugai Pharmaceutical
- **PMDA Presentation**
- Dr. Yuki Ando, Senior Scientist for Biostatistics; and Dr. Daisuke Iwata, Senior Reviewer, Pharmaceutical and Medical Devices Agency (PMDA)
- **EMA Presentation**
- Nick Halsey, Scientific Administrator; and Eftychia-Eirini Psarelli, Seconded National Expert, Methodology Workstream, Data Analytics and Methods Task Force, European Medicines Agency (EMA)
- **Session 6: Regulatory Presentations, Part II**
- Dr. Toshiki Saito, J3C Vice Chair, Nagoya Medical Center
- **FDA CDER Update on CDISC SEND Submissions**
- Jesse Anderson, Lead Project Manager, Regulatory Review Services Team, Center for Drug Evaluation and Research (CDER), FDA
- **FDA CDER Presentation**
- Dr. Stephen Wilson, Senior Staff Fellow, Office of Biostatistics, Center for Drug Evaluation and Research (CDER), FDA
- **Regulatory Q&A Session**
- Dr. Stephen Wilson & Jesse Anderson, Center for Drug Evaluation and Research (CDER), FDA
- **Session 7: Automation Topics**
- Hidemi Hasegawa, J3C Vice Chair, Boehringer Ingelheim
- **Use of Artificial Intelligence(ML/NLU/NLP/NLG) in Regulatory(scientific) Documents Authoring**
- Farha Feroze, Symbiance Inc.
- **Validation of Programs in Automated SDTM Datasets Creation**
- Kunihito Ebi, Fujitsu Limited
- **An Efficient Approach to Automatically Trace Back Clinical Data from Analysis to Source**
- Haiqiang Luo, Hengrui Pharma
- **Session 8: CDISC Standards**
- Hideaki Kosaka, J3C, EPS Corporation
- **Upgrade/Downgrade to define.xml v2.0 for SDTM**
- Hajime Shimizu, Independent
- **Introduction to the CJUG CDASH Team**
- Yuko Tamura, Noriyuki Furuya, CJUG CDASH Team
- **Session 9: Closing Plenary**
- Yoshiteru Chiba, J3C, UMIN
- **SDTM Mapping for the T1Dexi Trial Using the T1D TAUG**
- John Owen, CDISC
- **CDISC CORE Status and Next Steps**
- Peter Van Reusel, CDISC
- **Q&A**
- Peter Van Reusel and John Owen, CDISC
- **Closing Remarks**

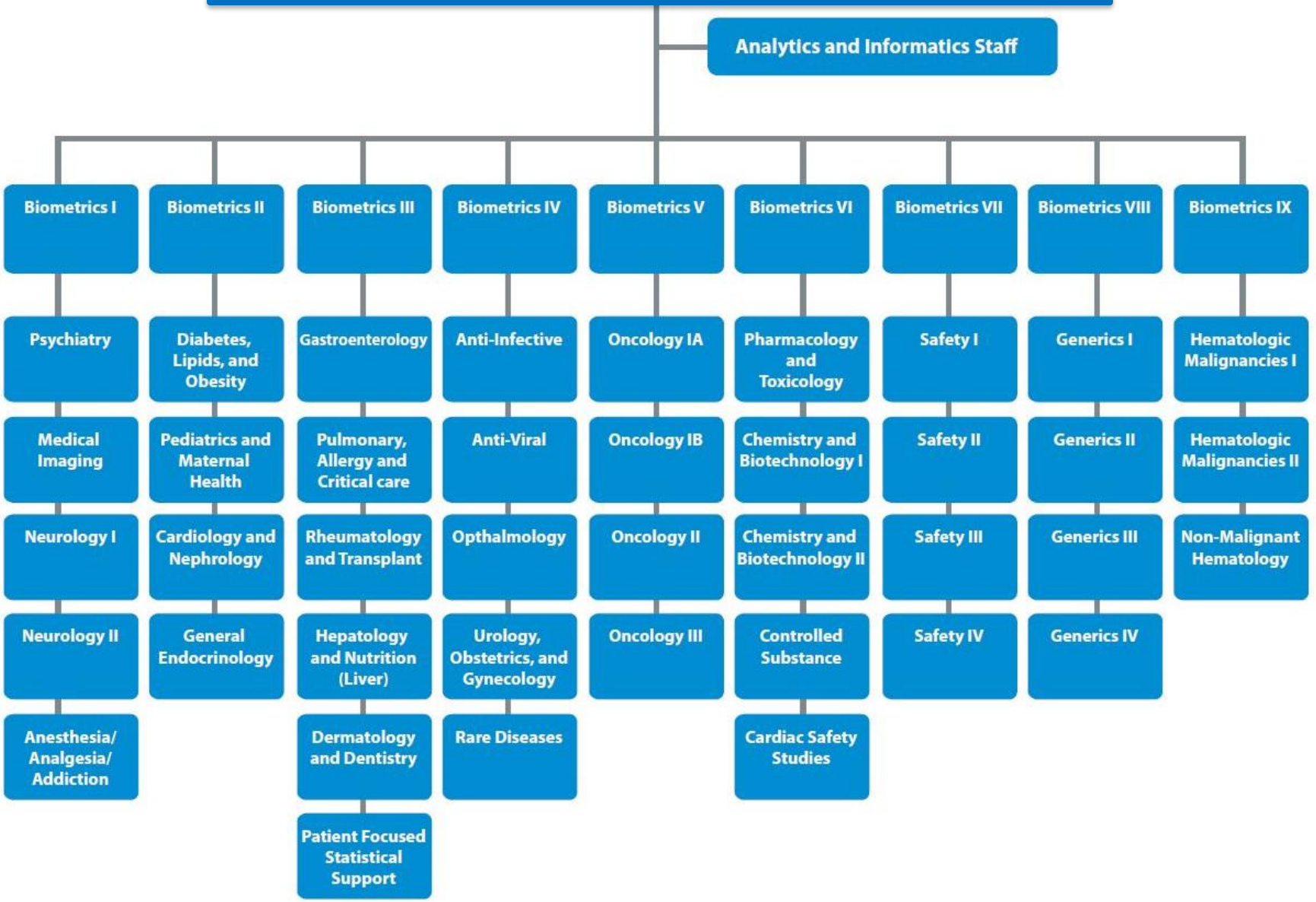
The Program


FHIR to CDISC -- Mapping
TAUGs
DRAGON Project
CDISC Core
CDISC Observational Guide
CJUG
State of CDISC Standards
Standards Updates
Use Cases
2022 JAPAN INTERCHANGE
User Guide for COVID Studies
Use of ADaM and SDTM in RECOVERY Trial
Artificial Intelligence
ADaM Dataset Creation
Regulatory
Automated SDTM
SEND

Center for Drug Evaluation and Research (CDER)



Office of Biostatistics (OB)



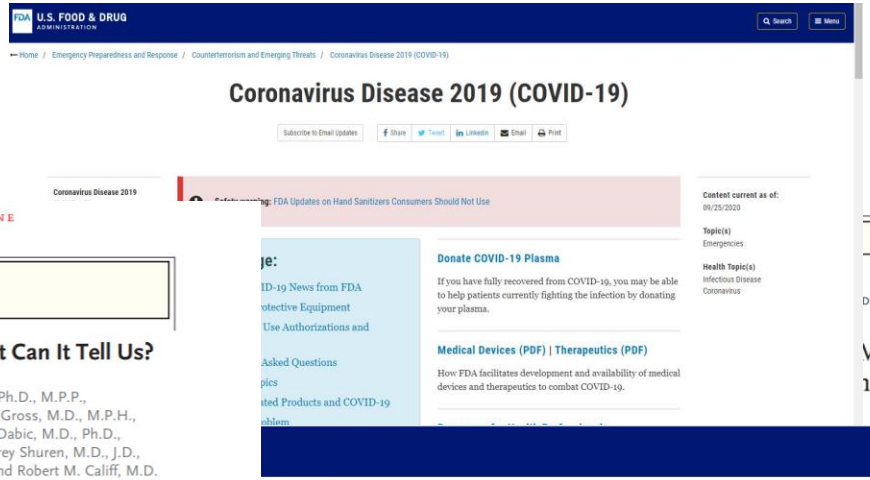
Analytics and Informatics Staff

- **Works jointly with the 9 Review Divisions of Biometrics (DB)**
- **Provides leadership in the areas of:**
  - Data Standards
  - Data Integrity and Data Quality
  - Data Visualization and other Data Tools
  - Scientific Computing and Statistical Programming
  - IT Tools Development and Support (CDERwiki, ColdFusion)
- **Special Skill Sets**
  - *Master's degree in Statistics/Biostatistics*
  - Statistical Programming (R, SAS)
  - GitLab Pilot
  - Shiny app development
  - Statistical Modeling and Simulation

(Some) Current OB/AIS Activities

- Supports Statistical Reviews of INDs/NDAs/BLAs
- Develops Training Materials for Data Standards
- Supports Statistical/Scientific Software System Maintenance and Development
- Represents OB on FDA/CDER IT and Data Standards and Review Planning Committees
- Assists OB Management With the Development of OB Review Management Systems, Programs and SOPs
- Works with OB Divisions to Design and Develop Software Tools and Specifications for Statistical Review
- Collaborates with the CDISC SDS QRS Subteam to Develop High Priority Instrument Supplements

The Evolving Regulatory Landscape



The NEW ENGLAND JOURNAL of MEDICINE

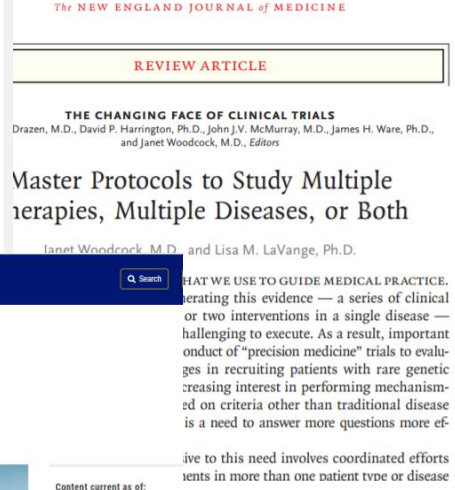
SOUNDING BOARD

Real-World Evidence — What Is It and What Can It Tell Us?

Rachel E. Sherman, M.D., M.P.H., Steven A. Anderson, Ph.D., M.P.P., Gerald J. Dal Pan, M.D., M.H.S., Gerry W. Gray, Ph.D., Thomas Gross, M.D., M.P.H., Nina L. Hunter, Ph.D., Lisa LaVange, Ph.D., Danica Marinac-Dabic, M.D., Ph.D., Peter W. Marks, M.D., Ph.D., Melissa A. Robb, B.S.N., M.S., Jeffrey Shuren, M.D., J.D., Robert Temple, M.D., Janet Woodcock, M.D., Lilly Q. Yue, Ph.D., and Robert M. Califf, M.D.

The term "real-world evidence" is widely used by those who develop medical products or who study, deliver, or pay for health care, but its specific meaning is elusive. We believe it refers to information on health care that is derived from

shortage of researchers with adequate methodologic savvy could result in poorly conceived study and analytic designs that generate incorrect or unreliable conclusions. Accordingly, if we are to realize the full promise of such evidence, we



The NEW ENGLAND JOURNAL of MEDICINE

REVIEW ARTICLE

THE CHANGING FACE OF CLINICAL TRIALS

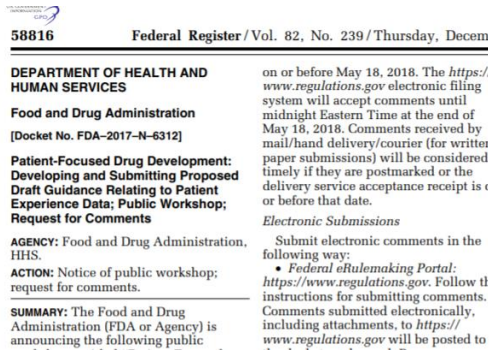
Drazen, M.D., David P. Harrington, Ph.D., John J.V. McMurray, M.D., James H. Ware, Ph.D., and Janet Woodcock, M.D., *Editors*

Master Protocols to Study Multiple ıerapies, Multiple Diseases, or Both

Janet Woodcock, M.D., and Lisa M. LaVange, Ph.D.

HAT WE USE TO GUIDE MEDICAL PRACTICE. ıerating this evidence — a series of clinical or two interventions in a single disease — hallenging to execute. As a result, important onduct of "precision medicine" trials to evalu- ges in recruiting patients with rare genetic creasing interest in performing mechanism- ed on criteria other than traditional disease is a need to answer more questions more ef-

ive to this need involves coordinated efforts ıents in more than one patient type or disease



58816 Federal Register / Vol. 82, No. 239 / Thursday, Decem

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA–2017–N–6312]

Patient-Focused Drug Development: Developing and Submitting Proposed Draft Guidance Relating to Patient Experience Data; Public Workshop; Request for Comments

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

SUMMARY: The Food and Drug Administration (FDA or Agency) is announcing the following public

on or before May 18, 2018. The *https://www.regulations.gov* electronic filing system will accept comments until midnight Eastern Time at the end of May 18, 2018. Comments received by mail/hand delivery/courier (for written paper submissions) will be considered timely if they are postmarked or the delivery service acceptance receipt is on or before that date.

Electronic Submissions

Submit electronic comments in the following way:

- *Federal eRulemaking Portal: https://www.regulations.gov.* Follow the instructions for submitting comments. Comments submitted electronically, including attachments, to *https://www.regulations.gov* will be posted to

second copy, which will have the claimed confidential information

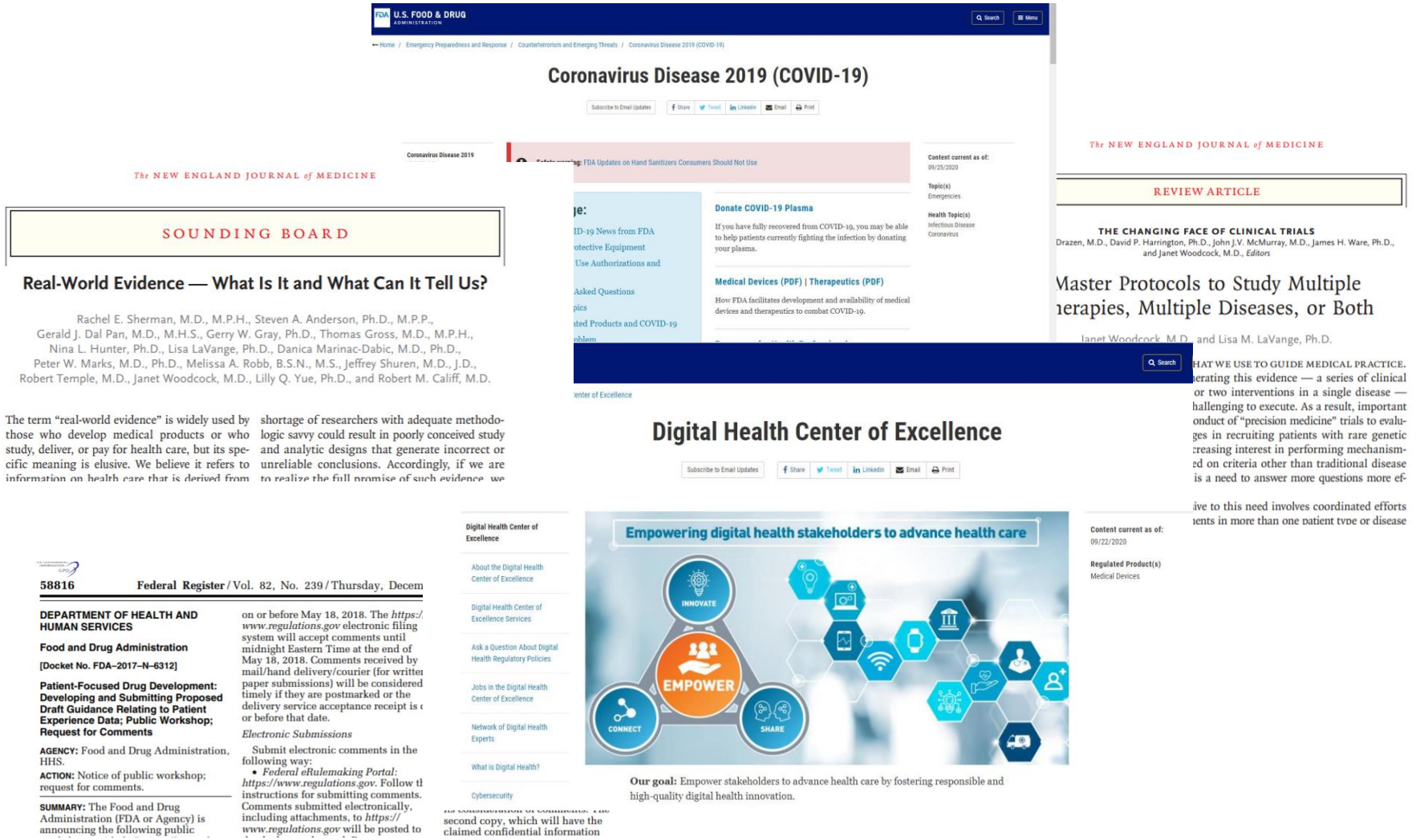
Our goal: Empower stakeholders to advance health care by fostering responsible and high-quality digital health innovation.

Content current as of:
09/22/2020

Regulated Product(s)
Medical Devices

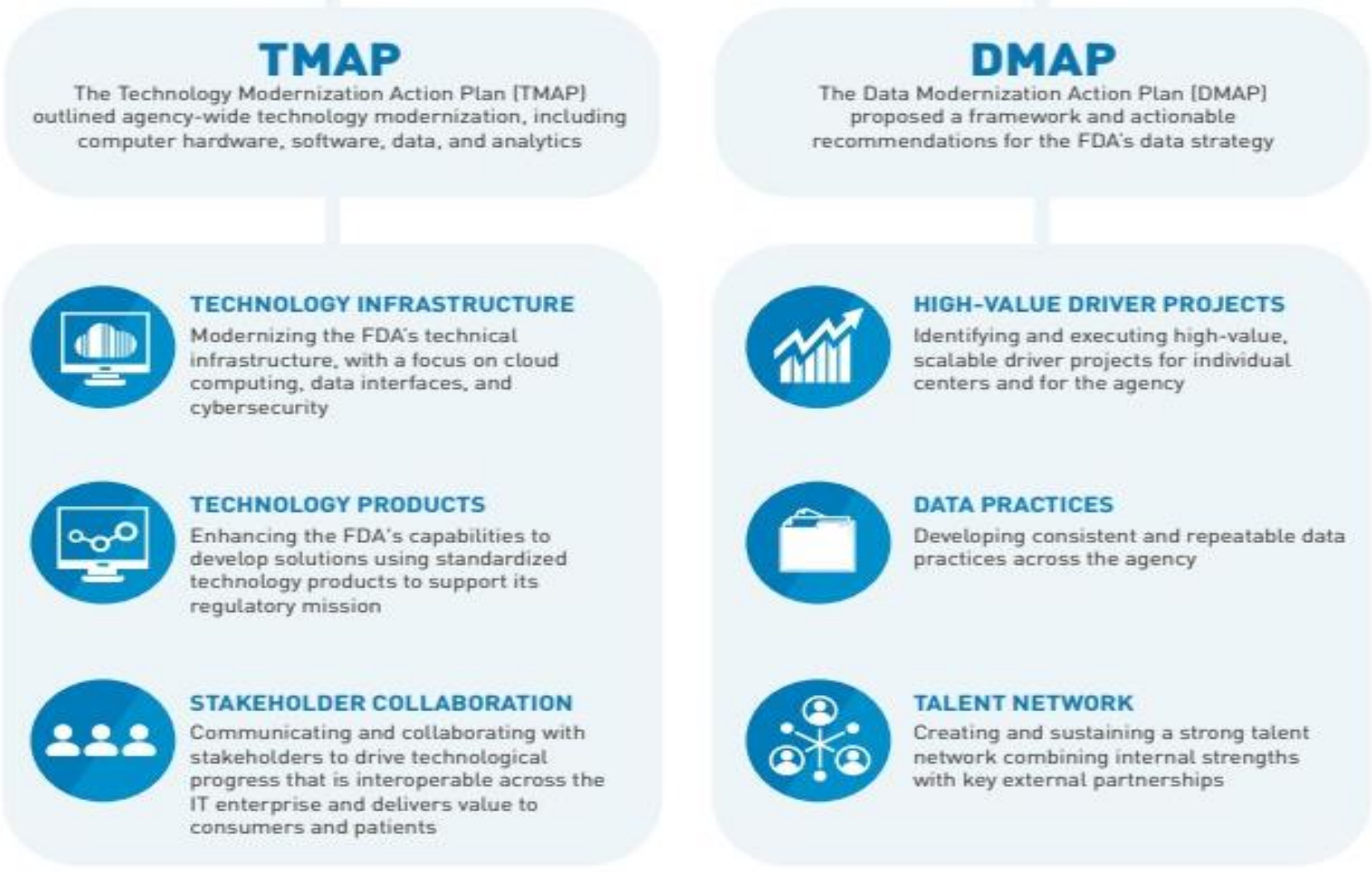
Kam, 2022

The Evolving Science/Data/Evidence/Decision Landscape



Adapted from Kam, 2022

The Technology Modernization Action Plan (TMAP) Data Modernization Action Plan (DMAP)

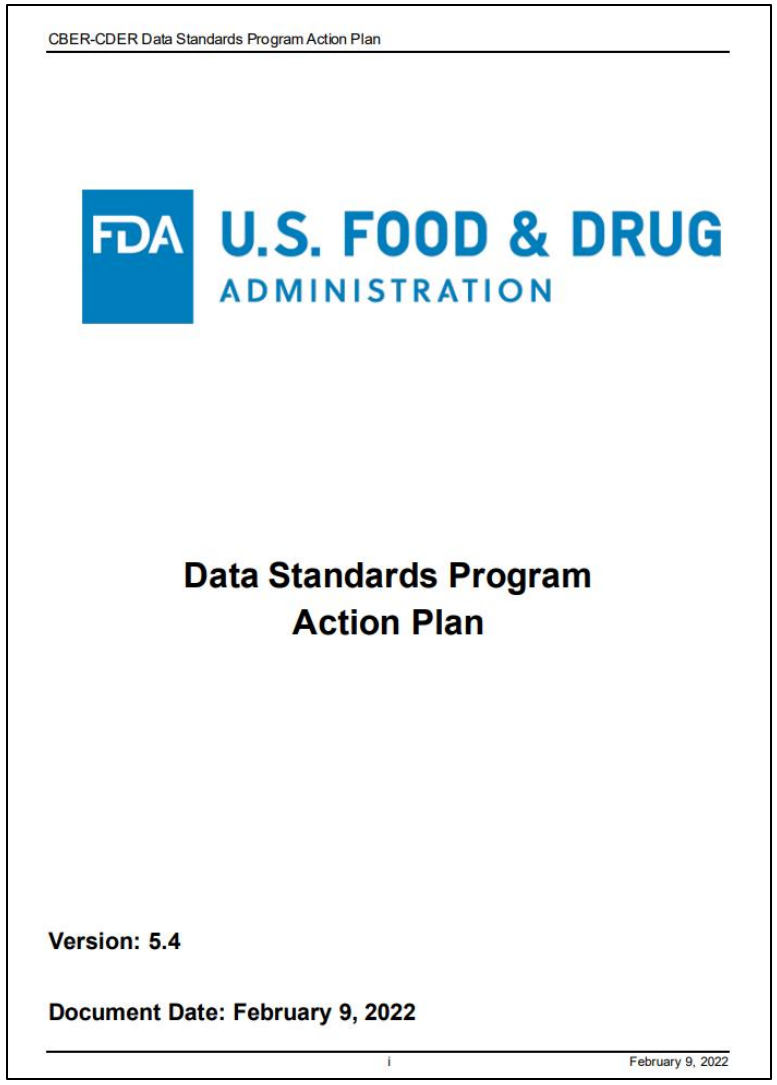


https://www.fda.gov/about-fda/reports/fdas-technology-modernization-action-plan

https://www.fda.gov/about-fda/reports/data-modernization-action-plan

Adapted from Kam, 2022

CDER/CBER Standards Program Action Plan (DSPAP)



CBER-CDER Data Standards Program Action Plan

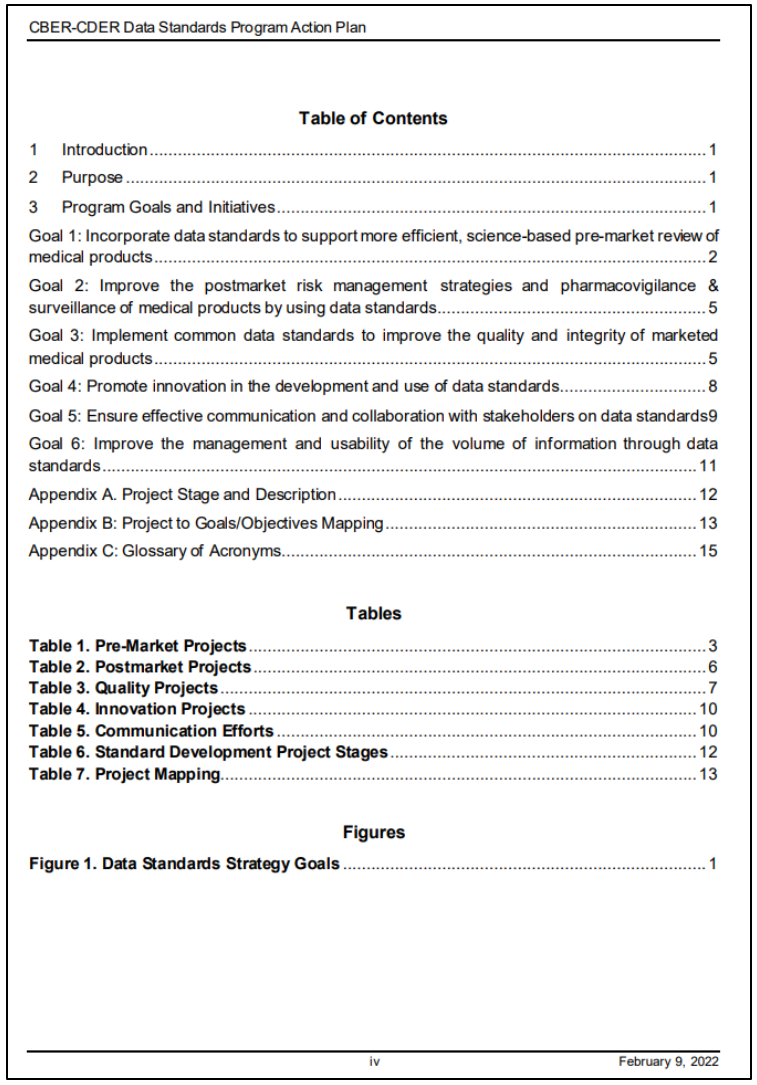
U.S. FOOD & DRUG ADMINISTRATION

Data Standards Program Action Plan

Version: 5.4

Document Date: February 9, 2022

i February 9, 2022



CBER-CDER Data Standards Program Action Plan

Table of Contents

Tables

Figures

iv February 9, 2022

https://fda.report/media/156156/Final_Data_Standards_Program_Joint_Action_Plan_v5.4.pdf

21st Century Cures Act

The 21st Century Cures Act (Cures Act), signed into law on December 13, 2016, is designed to help accelerate medical product development and bring new innovations and advances to patients who need them faster and more efficiently.

Section 3022

- Real World Evidence

Section 3021

- Novel clinical trial designs

Section 3002

- Patient-focused drug development

Section 3001

- Patient experience data

https://www.fda.gov/regulatory-information/selected-amendments-fdc-act/21st-century-cures-act

Adapted from Kam, 2022

Prescription Drug User Fee Act (PDUFA) VII

PDUFA REAUTHORIZATION PERFORMANCE GOALS AND PROCEDURES FISCAL YEARS 2023 THROUGH 2027

This document contains the performance goals and procedures for the Prescription Drug User Fee Act (PDUFA) reauthorization for fiscal years (FYs) 2023-2027, known as PDUFA VII. It is commonly referred to as the "goals letter" or "commitment letter." The goals letter represents the product of FDA's discussions with the regulated industry and public stakeholders, as mandated by Congress. The performance and procedural goals and other commitments specified in this letter apply to aspects of the human drug review program that are important for facilitating timely access to safe, effective, and innovative new medicines for patients. While much of FDA's work is associated with formal tracked performance goals, the Agency and industry mutually agree that it is appropriate to manage some areas of the human drug review program with internally tracked timeframes. This provides FDA the flexibility needed to respond to a highly diverse workload, including unanticipated public health needs. FDA is committed to meeting the performance goals specified in this letter and to continuous improvement of its performance regarding other important areas specified in relevant published documents[1] that relate to preapproval drug development and post-approval activities for marketed products. FDA and the regulated industry will periodically and regularly assess the progress of the human drug review program throughout PDUFA VII. This will allow FDA and the regulated industry to identify emerging challenges and develop strategies to address these challenges to ensure the efficiency and effectiveness of the human drug review program.

https://www.fda.gov/media/151712/download

- Split Real Time Application Review (STAR) Pilot Program
- Enhancing Capacity to Review Complex Innovative Designs
- Advancing RWE for Use in Regulatory Decision-Making
- Advancing Development of Drugs for Rare Diseases
- Enhancing Incorporation of Patient's Voice in Drug Development & Decision-Making
- Enhancing use of DHTs to Support Drug Development and Review

Kam, 2022

Advancing RWE: Goals

- Identify approaches for generating RWE that meet regulatory requirements in support of:
  - labeling for effectiveness (e.g., new indications, populations, dosing information) or
  - meeting post-approval study requirements
- Develop agency processes that promote consistent decision-making and shared learning regarding RWE
- Promote awareness of characteristics of RWE that can support regulatory decisions by allowing FDA to discuss study designs considered in the Advancing RWE Program in a public forum

Kam, 2022

Advancing RWE: Goals

Jacqueline Corrigan-Curay, "Framework for FDA's Real-World Evidence Program"

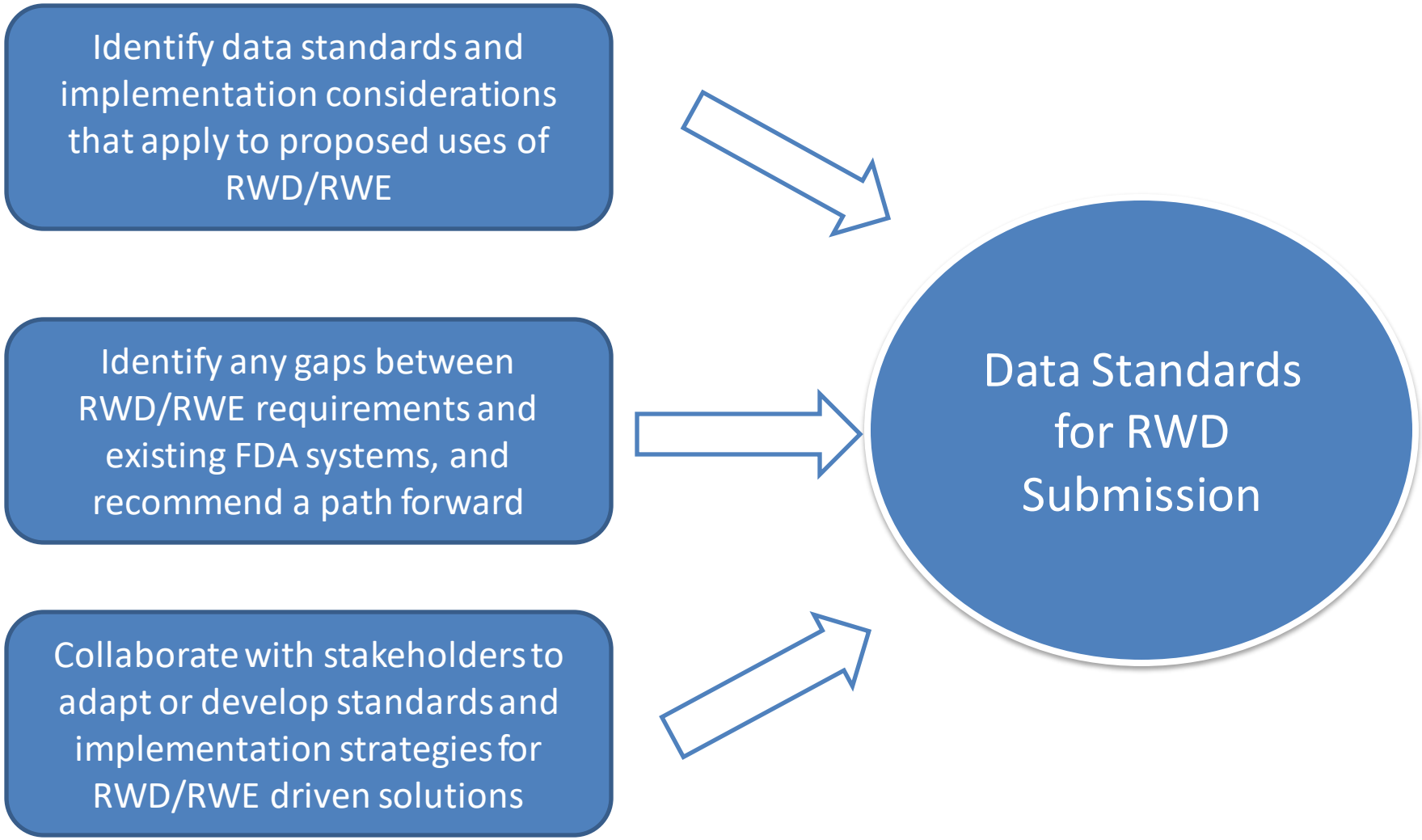
Considerations

- Whether the RWD are fit for use
- Whether the trial or study design used to generate RWE can provide adequate scientific evidence to answer or help answer the regulatory question
- Whether the study conduct meets FDA regulatory requirements

https://sbiaevents.com/files2/RWE-Webinar-Mar-2019.pdf

Adapted from Kam, 2022

Data Standards and RWD/RWE



https://www.fda.gov/media/120060/download

Kam, 2022

Data Standards for Drug and Biological Product Submissions Containing Real-World Data -- Guidance for Industry (Dec 2021)

"RWD submitted as study data to NDAs, ANDAs, certain BLAs, and certain INDs as further described in section II. A of the Study Data Guidance must be in an electronic format that the Agency can process, review, and archive. Currently, as stated in the Study Data Guidance, the Agency can process, review, and archive electronic submissions of clinical and nonclinical study data (including those derived from RWD sources) that use the standards specified in the Data Standards Catalog (Catalog)."

The guidance specifies that submissions based on RWD must utilize existing, supported standards in FDA Data Standards Catalog!

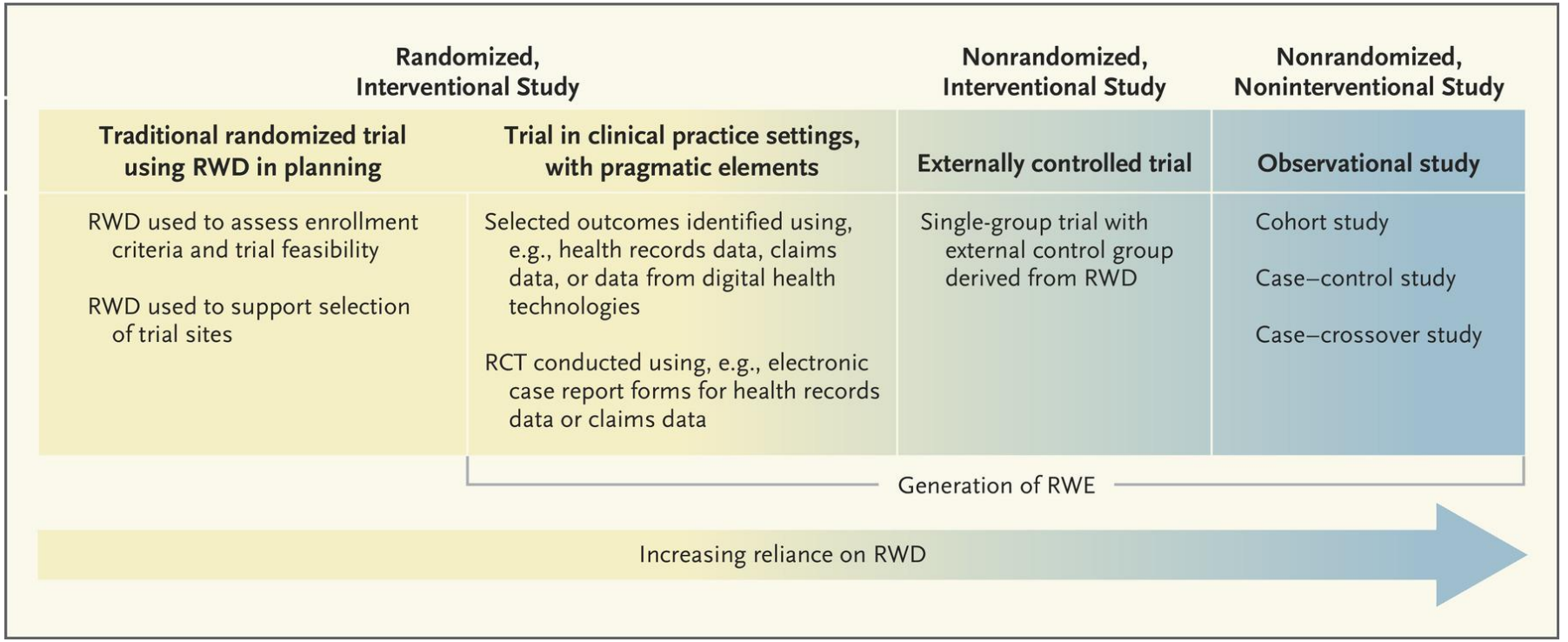
https://www.fda.gov/media/153341/download

RWD/RWE – Evidence and Experience

The NEW ENGLAND JOURNAL of MEDICINE

Real-World Evidence — Where Are We Now?

John Concato, M.D., M.P.H. and Jacqueline Corrigan-Curay, J.D., M.D .
FDA/CDER
May 5, 2022
VOL. 386 NO. 18



https://www.nejm.org/doi/full/10.1056/NEJMp2200089

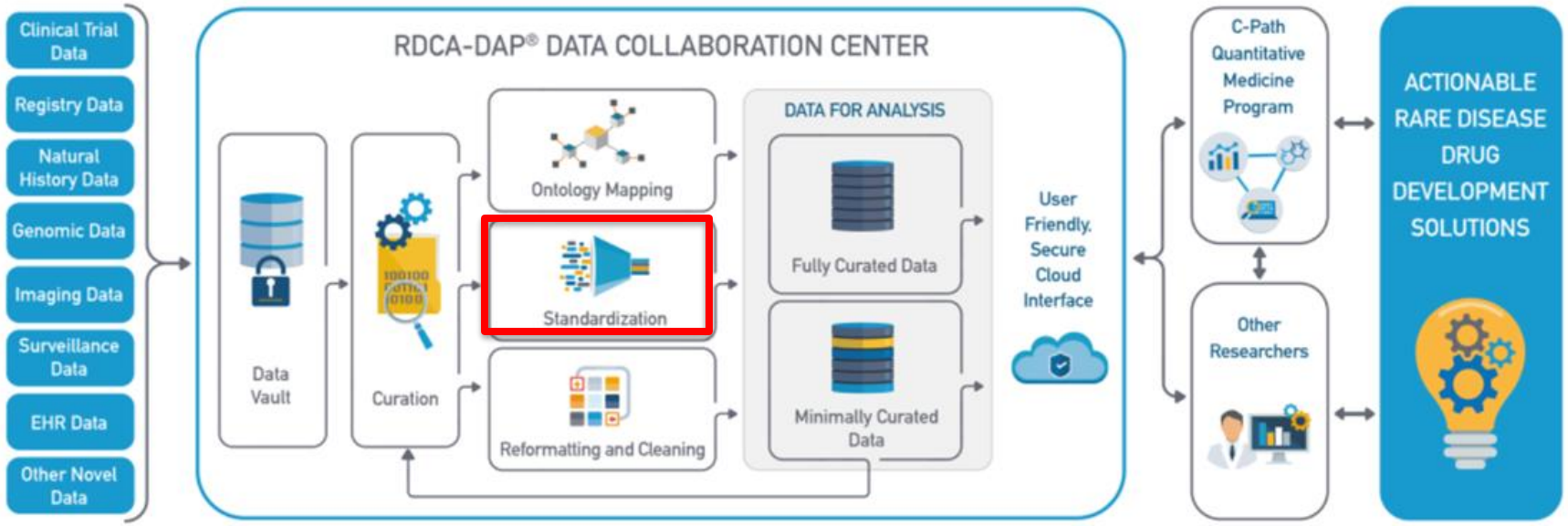
Advancing Development of Drugs for Rare Diseases

- CDER Accelerating Rare disease Cures (ARC) Program
  - Launched May 2022 to harnesses CDER's collective expertise and activities to provide strategic overview and coordination of CDER's rare disease activities
- FDA CDER & NIH NCATS Regulatory Fitness in Rare Disease Clinical Trials Workshop- May 16-17, 2022
- Rare Disease Endpoint Advancement Pilot Program (RDEA):
  - Aims to increase collaboration between industry and FDA to determine meaningful endpoints for rare diseases in clinical trials.
- Statisticians can help provide expertise on flexible and feasible approaches to
  - Innovative use of biomarkers
  - Non-traditional clinical development programs
  - Use of adaptive study designs
  - Evaluation of novel endpoints
  - New approaches to statistical analysis

Rare Disease Cures Accelerator (RDCA-DAP)

- The Rare Disease Cures Accelerator - Data Analytics Platform (RDCA-DAP) effort is being led by the Critical Path Institute through a cooperative grant agreement from the FDA. To learn more, please visit the Critical Path Institute.

  https://www.fda.gov/drugs/regulatory-science-research-and-education/rare-disease-cures-accelerator



For questions or additional information about participating in RDCA-DAP, please email rdcadap@c-path.org.

Patient-Focused Drug Development (PFDD)

- **PFDD Meetings**
  - Designed to engage patients and elicit their perspectives on
    - a) most significant symptoms of their condition & impact on daily life;
    - b) current approaches to treatment
- **Standard Core COA Grant Program**
  - Enable development of standard core sets of measures of disease burden and treatment burden for a given area – that would be made publicly available at nominal or no cost
- **Methodologic Guidance Documents**
  - PFDD Guidance 1: Collecting Comprehensive and Representative Input
  - PFDD Guidance 2: Methods to Identify What is Important to Patients
  - PFDD Guidance 3: Select, Develop or Modify Fit-for-Purpose COAs
  - PFDD Guidance 4: Incorporating COAs into Endpoints

Kam, 2022

CDER Pilot Grant Program: Standard Core Clinical Outcome Assessments (COAs) and their Related Endpoints

FDA has developed a Pilot Grant Program to support the development of publicly available core set(s) of COAs and their related endpoints.

Patient input can help inform the therapeutic context for regulatory review. Patient input also can inform the selection of clinical outcomes, ensure the appropriateness of instruments used to collect trial data, and help ensure that investigations of the effect of treatments are assessing outcomes that are meaningful to patients. If methodologically-sound data collection tools are developed and used within clinical trials of an investigational therapy, patient input can provide a direct source of evidence regarding the benefits and risks of a drug.

https://www.fda.gov/drugs/development-approval-process-drugs/cder-pilot-grant-program-standard-core-clinical-outcome-assessments-coas-and-their-related-endpoints

FDA/CDISC Collaboration

FDA/CDER OB/AIS QRS Review Team: QSuRT

Rachel Dlugash, Liping Sun, Steve Wilson & OND/OB Reviewers/SMEs

U.S. FOOD & DRUG ADMINISTRATION

Collaborative Review of CDISC QRS Instruments

Steve Wilson

Senior Staff Fellow

FDA/CDER/OTS/OB/AIS

Session 3: Track A – Questionnaires, Ratings and Scales

Wednesday, October 20, 2021

CDISC

2021 US Interchange

Live Stream | 20-21 October

FDA QRS Draft Supplement Review Process – FDA Review

- In the initial review of the QRS Draft Supplement Package, the OB QRS Review Team reviews the submitted documents, assesses completeness, and identifies any need for SME input.
- If required, internal/external SMEs are identified/notified and requested to provide input regarding specific review questions/comments.
- Following a process in which the Review Team collects/flags, coalesces and reconciles comments and issues the final review document is submitted to the OSP for posting to the COA Data Standards SharePoint Site and transmittal to the CDISC QRS Subteam.
- In subsequent review cycles, the CDISC QRS Subteam includes an Excel spreadsheet describing Jira issues and responses to FDA questions/comments in the review package submitted to the Agency.

Enhancing the Use of DHTs to Support Drug Development and Review

- Expand capacity and advance a DHT framework that will promote regulatory consistency and coordination across FDA on digital health
- Establish a committee to promote consistency across centers regarding DHT-based policy, procedure, and analytic tool development
- Convene public workshops on the use of DHTs in regulatory decision-making; identify demonstration projects to inform methodologies for DHT evaluation; and issue DHT-related guidances
- Enhance IT capabilities to support the review of DHT-generated data

Kam, 2022

Digital Health Technologies (DHT)

Digital Health Center of Excellence

Subscribe to Email Updates | Share | Tweet | LinkedIn | Email | Print

Our goal: Empower stakeholders to advance health care by fostering responsible and high-quality digital health innovation.

Our objectives: The Digital Health Center of Excellence aims to:

- **Connect and build partnerships** to accelerate digital health advancements.
- **Share knowledge** to increase awareness and understanding, drive synergy, and advance best practices.
- **Innovate regulatory approaches** to provide efficient and least burdensome oversight while meeting the FDA standards for safe and effective products.

https://www.fda.gov/medical-devices/digital-health-center-excellence

Digital Health Technologies for Remote Data Acquisition in Clinical Investigations

Guidance for Industry, Investigators, and Other Stakeholders

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 electronic comments to https://www.regulations.gov. Submit written comments to the Dockets Management Staff (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) Elizabeth Kunkoski, 301-796-6439; (CBER) Office of Communication, Outreach and Development, 800-835-4709 or 240-402-8010; or (CDRH) Program Operations Staff at 301-796-5640.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)
Center for Biologics Evaluation and Research (CBER)
Center for Devices and Radiological Health (CDRH)
Oncology Center of Excellence (OCE)

December 2021
Clinical/Medical

24217145dft.docx
21/20/2021

https://www.fda.gov/media/155022/download

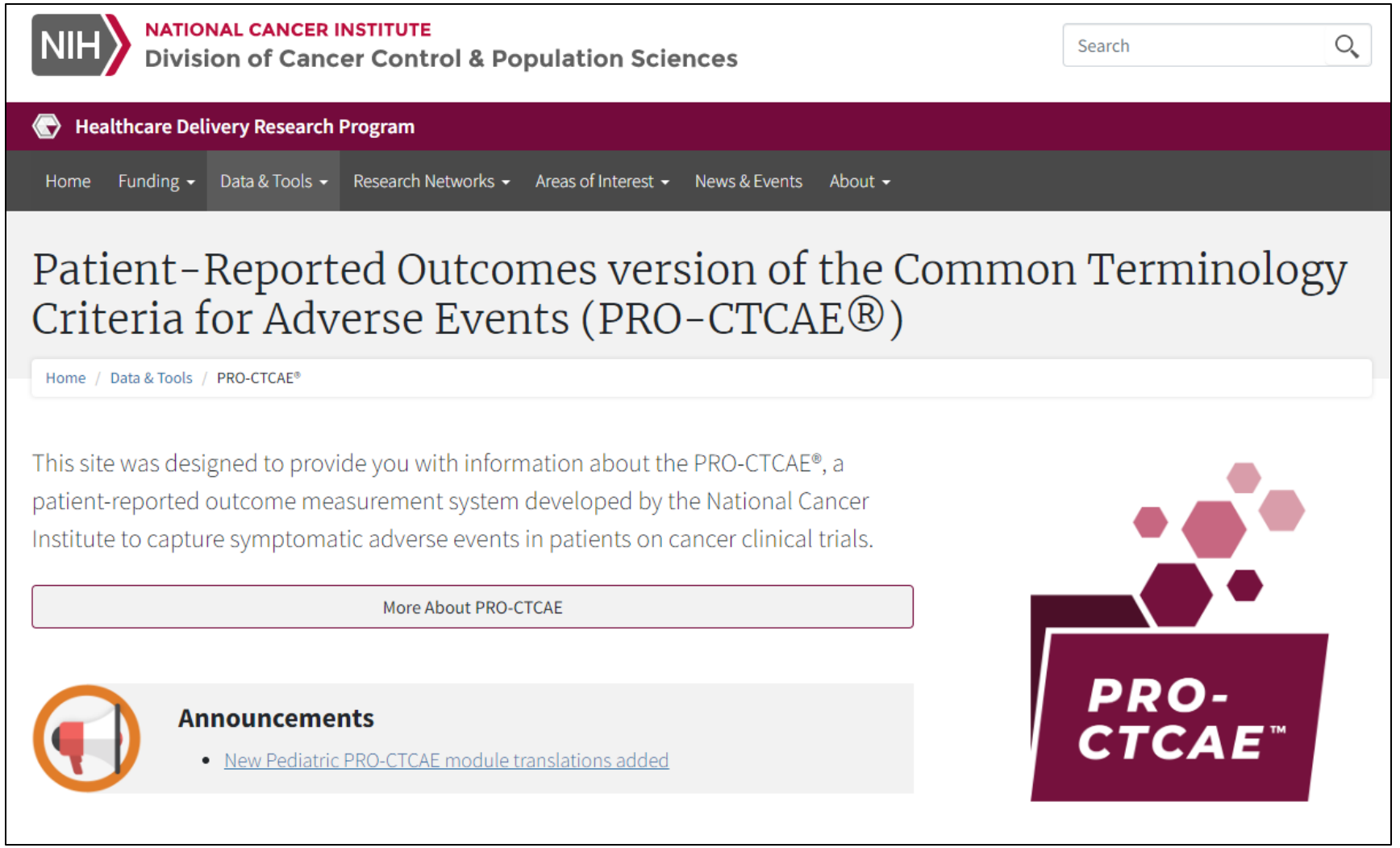
Kam, 2022

Collaboration: Opportunities & Experiences

- Patient-Reported Outcomes version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE®)
- FDA Technical Specifications
- Working with Japanese Colleagues on Data Standards: Some Personal Reflections and Thanks

Patient Focused Drug Development

Collaboration: COA/CDISC QRS, FDA & NCI



https://healthcaredelivery.cancer.gov/pro-ctcae/

PRO-CTCAE® -- Measurement System

- The NCI Patient Reported Outcomes version of the Common Terminology Criteria for Adverse Events (PRO-CTCAE®) Measurement System was developed to evaluate symptomatic toxicities by self-report in adults, adolescents and children participating in cancer clinical trials. It was designed to be used as a companion to the Common Terminology Criteria for Adverse Events (CTCAE), the standard lexicon for adverse event reporting in cancer trials.

- The PRO-CTCAE Measurement System characterizes the frequency, severity, interference, and presence/absence of symptomatic toxicities that include pain, fatigue, nausea, and cutaneous side effects such as rash and hand-foot syndrome, all toxicities that can be meaningfully reported from the patient perspective.

- The PRO-CTCAE Item Library includes 124 items representing 78 symptomatic toxicities drawn from the CTCAE. A pediatric module permits self-reporting by children and adolescents ages 7-17 years (Ped-PRO-CTCAE®). A version for caregiver reporting is available for use when children or adolescents ages 7-17 are unable to self-report (Ped-PRO-CTCAE®[Caregiver]). The pediatric module includes 130 items representing 62 symptomatic toxicities drawn from the CTCAE.

https://healthcaredelivery.cancer.gov/pro-ctcae/

NCI- PRO-CTCAE® ITEMS-JAPANESE

Item Library Version 1.0

As individuals go through treatment for their cancer they sometimes experience different symptoms and side effects. For each question, please select the one response that best describes your experiences over the past 7 days…

がんの治療を受けている方は、しばしば異なる症状や薬の副作用を経験いたします。それぞれの質問事項について、過去 7 日間にそれぞれの症状を経験されたかどうか、また経験された方は、その症状がどの程度だったか、もっとも自分の症状に適していると思われる回答を 1 つ選択してください。

1. **PRO-CTCAE® Symptom Term:** Dry mouth				
口の中の乾き				
a. この 7 日の間で、口の中の乾きは一番ひどい時でどの程度でしたか？				
O そういうことはなかった	O 軽度	O 中等度	O 高度	O 極めて高度

2. **PRO-CTCAE® Symptom Term:** Difficulty swallowing				
食べ物が飲み込みにくい				
a. この 7 日の間で、食べ物が飲み込みにくいことは一番ひどい時でどの程度でしたか？				
O そういうことはなかった	O 軽度	O 中等度	O 高度	O 極めて高度

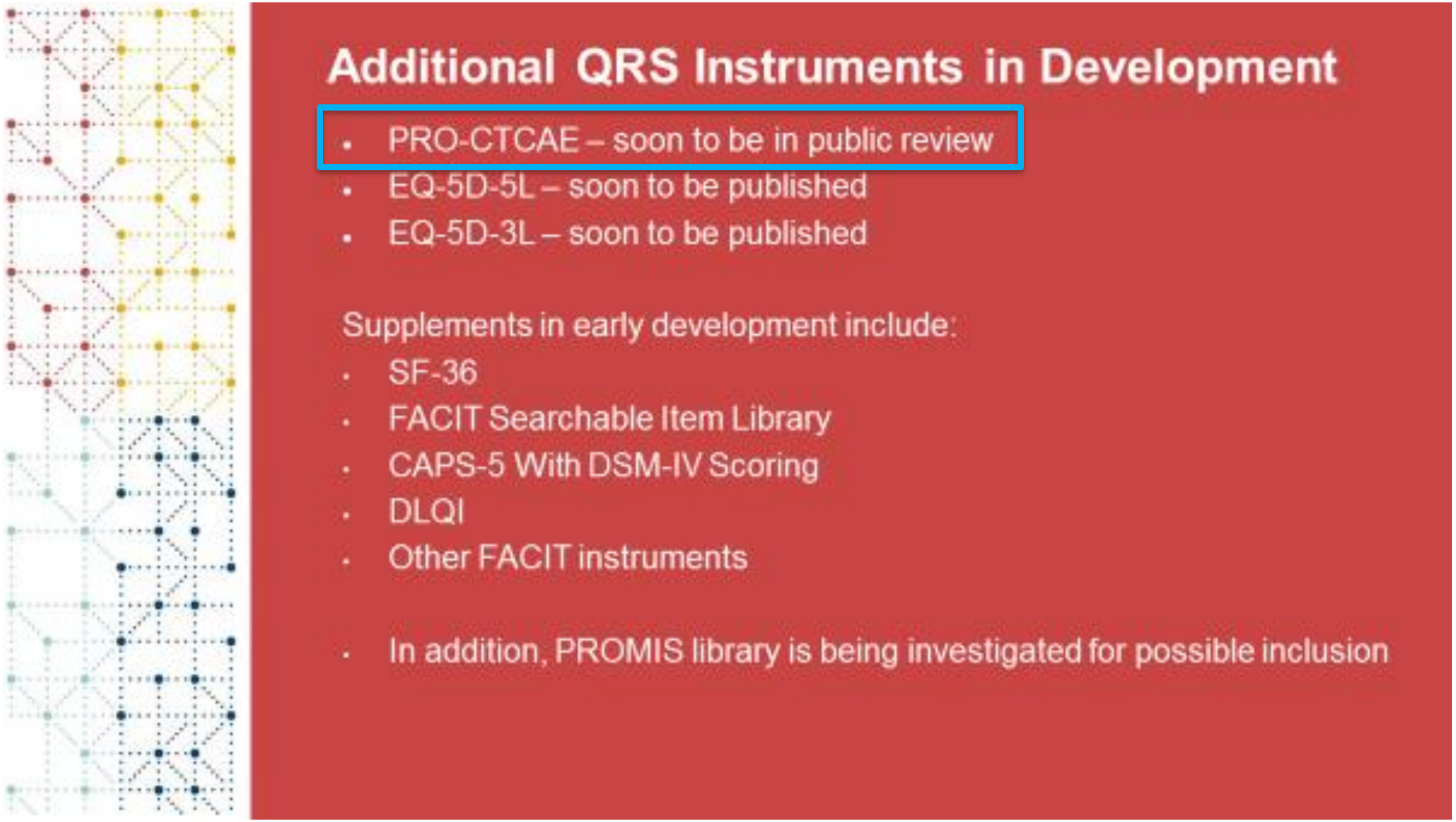
3. **PRO-CTCAE® Symptom Term:** Mouth/throat sores				
口の中や喉の痛み				
a. この 7 日の間で、口の中や喉の痛みは一番ひどい時でどの程度でしたか？				
O そういうことはなかった	O 軽度	O 中等度	O 高度	O 極めて高度
b. この 7 日の間に、口の中や喉の痛みはどの程度ふだんの生活の妨げになりましたか？				
O 全然ならなかった	O 少し	O ある程度	O かなり	O ものすごく

https://healthcaredelivery.cancer.gov/pro-ctcae/pro-ctcae_japanese.pdf

“CDISC Updates from the SDS QRS Subteam”

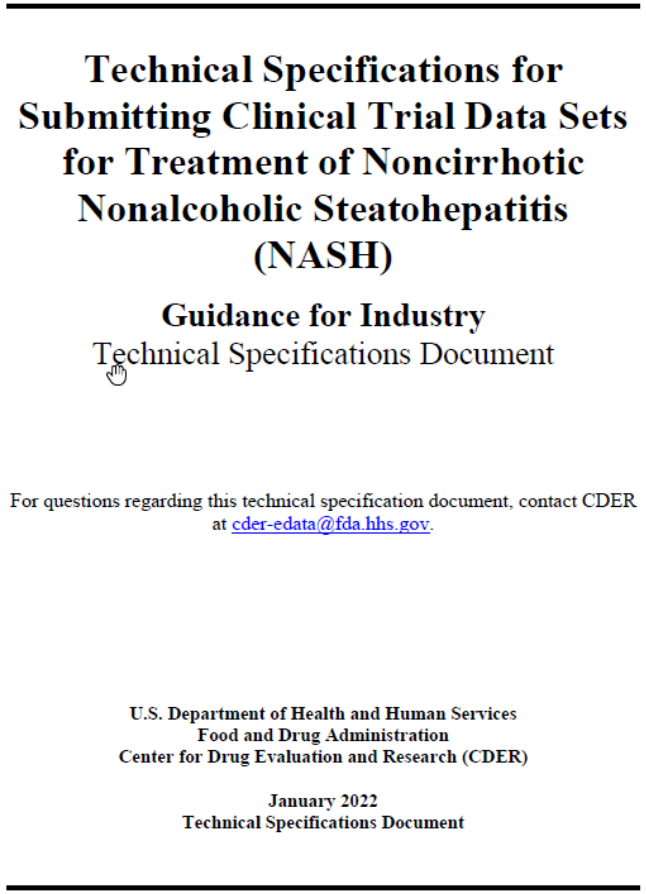
Presenter: Dana Booth, Senior Project Manager, CDISC
With additional contributions by: Steve Kopko, CDISC SME, External Consultant CDISC

25 May 2022, *PharmaSUG 2022*, Austin, Texas



Technical Specifications

Collaboration: FDA, Industry, Academia, Vendors, Patients, etc.



Technical Specifications for Submitting Clinical Trial Data Sets for Treatment of Noncirrhotic Nonalcoholic Steatohepatitis (NASH)

Guidance for Industry

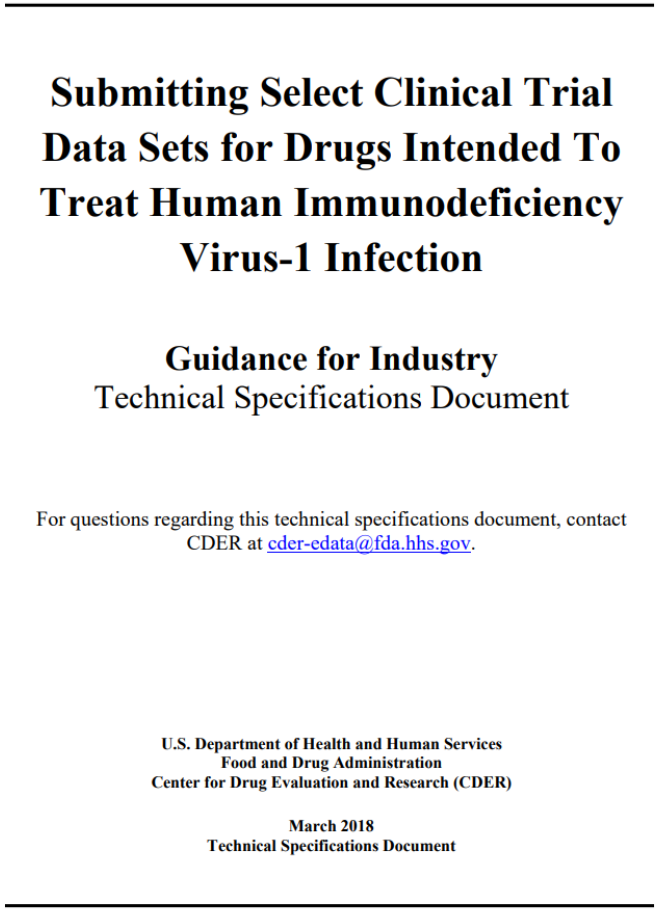
Technical Specifications Document

For questions regarding this technical specification document, contact CDER at cder-edata@fda.hhs.gov.

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

January 2022
Technical Specifications Document

https://www.fda.gov/media/151870/download



Submitting Select Clinical Trial Data Sets for Drugs Intended To Treat Human Immunodeficiency Virus-1 Infection

Guidance for Industry

Technical Specifications Document

For questions regarding this technical specifications document, contact CDER at cder-edata@fda.hhs.gov.

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

March 2018
Technical Specifications Document

https://www.fda.gov/media/112667/download

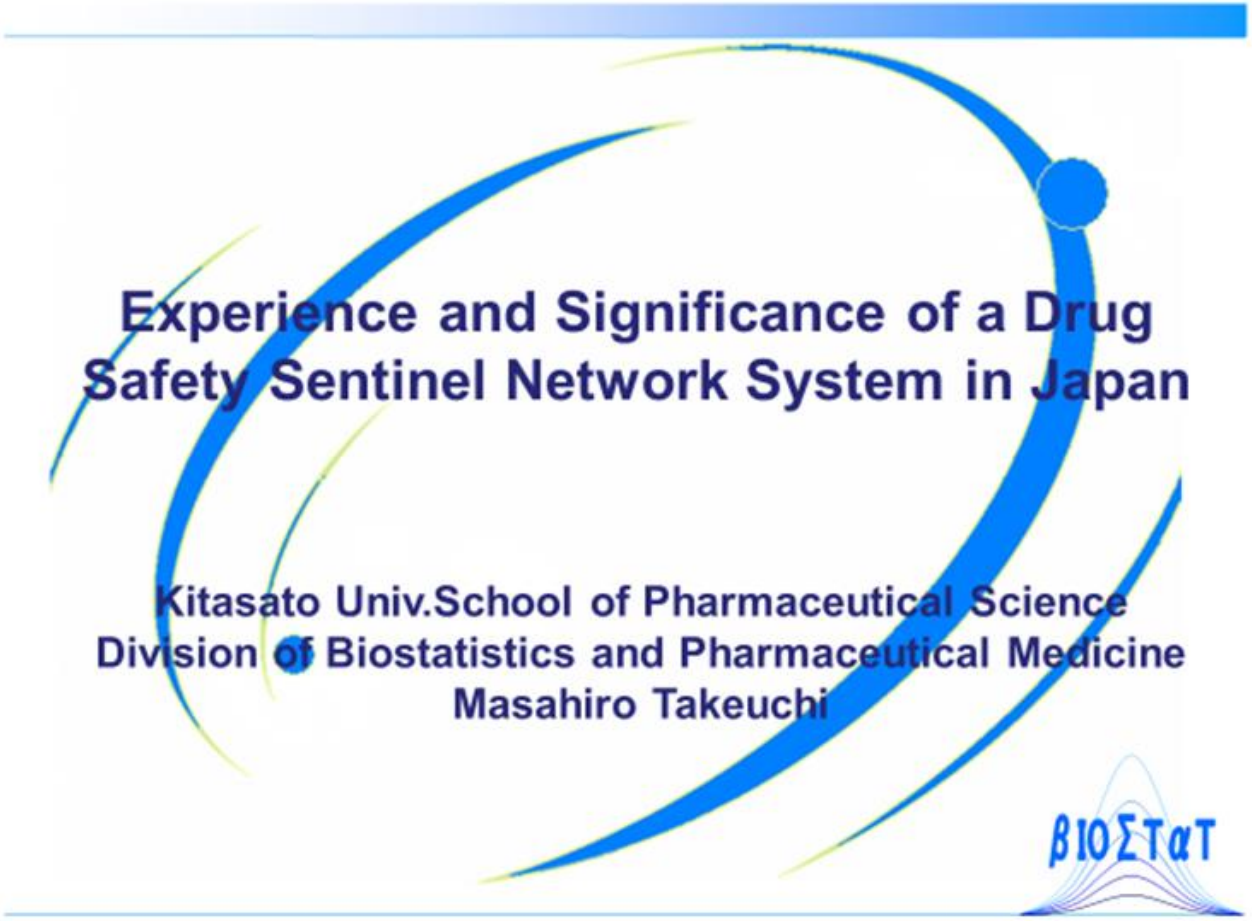
Technical Specifications Docket

Collaboration: FDA, Industry, Academia, Vendors, Patients, etc.

"This technical specifications document has been prepared by the Office of New Drugs and the Office of Translational Sciences in the Center for Drug Evaluation and Research at the Food and Drug Administration. You may submit comments on this guidance at any time. Submit comments to Docket No. FDA-2018-D-1216 (available at https://www.regulations.gov/docket?D=FDA-2018-D-1216) (see the instructions for submitting comments in the docket)"

“Our Experience and Significance in a Japanese Sentinel Network System”

SESSION CHAIRPERSON: Masahiro Takeuchi, DrSc, MPH Professor of Biostatistics and Pharmaceutical Medicine, School of Pharmaceutical Sciences, Kitasato University



“Our Experience and Significance in a Japanese Sentinel Network System”

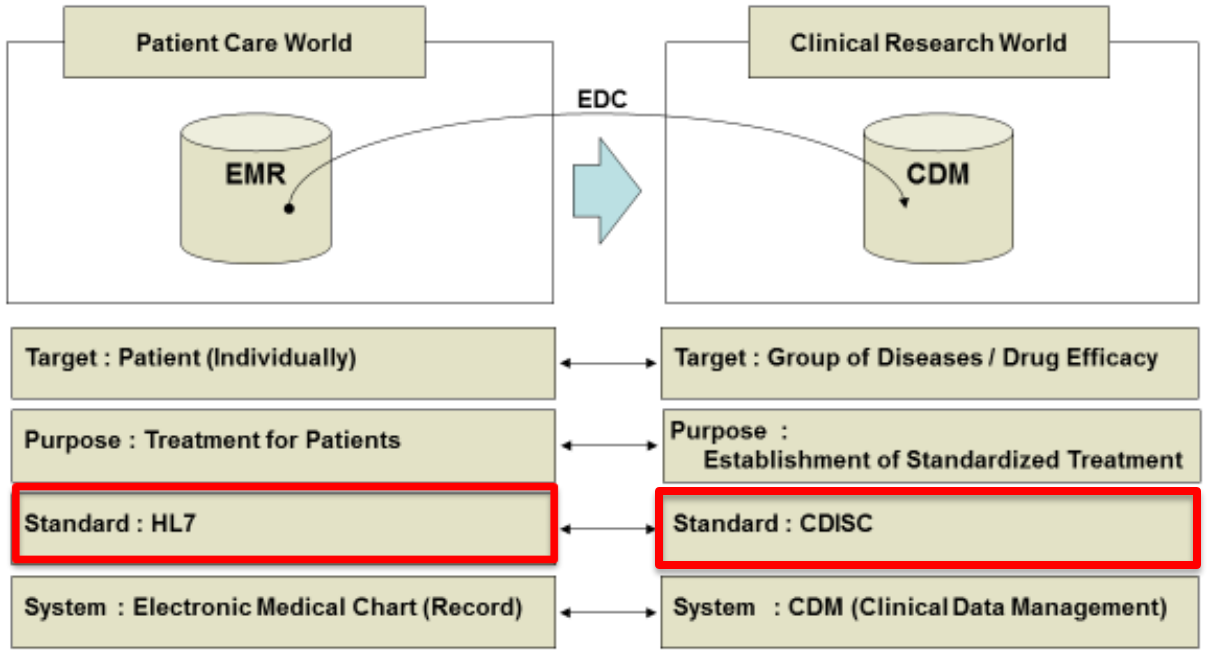
SESSION CHAIRPERSON: Masahiro Takeuchi, DrSc, MPH Professor of Biostatistics and Pharmaceutical Medicine, School of Pharmaceutical Sciences, Kitasato University

Safety Issues

Possible Solution: a Sentinel Network System

- Network system between hospitals in Japan
  - EDC Network system within hospitals to monitor patients
  - Detection of unexpected AEs
  - Build data base of pats` background for signal detection and pharmacoepidemiology

Treatment & Clinical Research



THANK YOU!

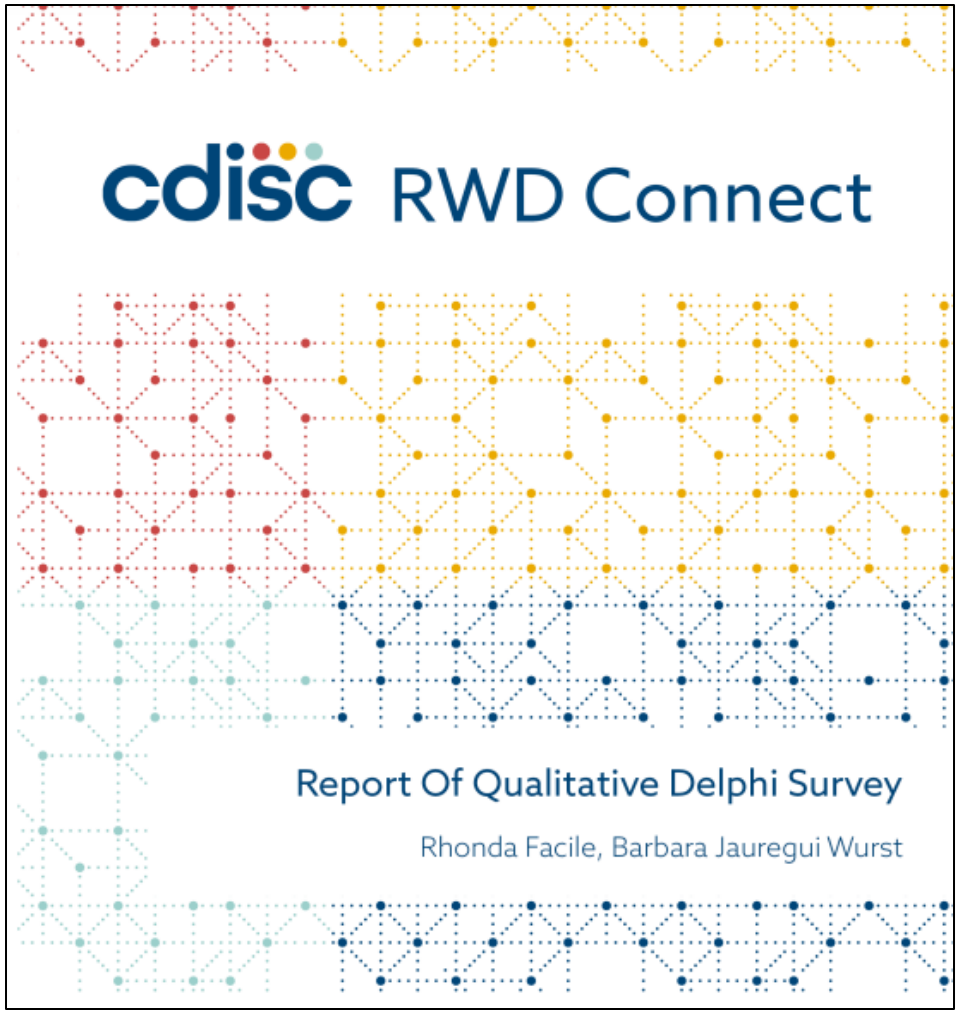
CDISC, PMDA & The DIA Clinical Data Management Meeting

THANK YOU
CJUG, UMIN, and Ciba-San

CDISC RWD Connect /Report of a Qualitative Delphi Survey

Rhonda Facile and Barbara Jauregui Wurst, 2021

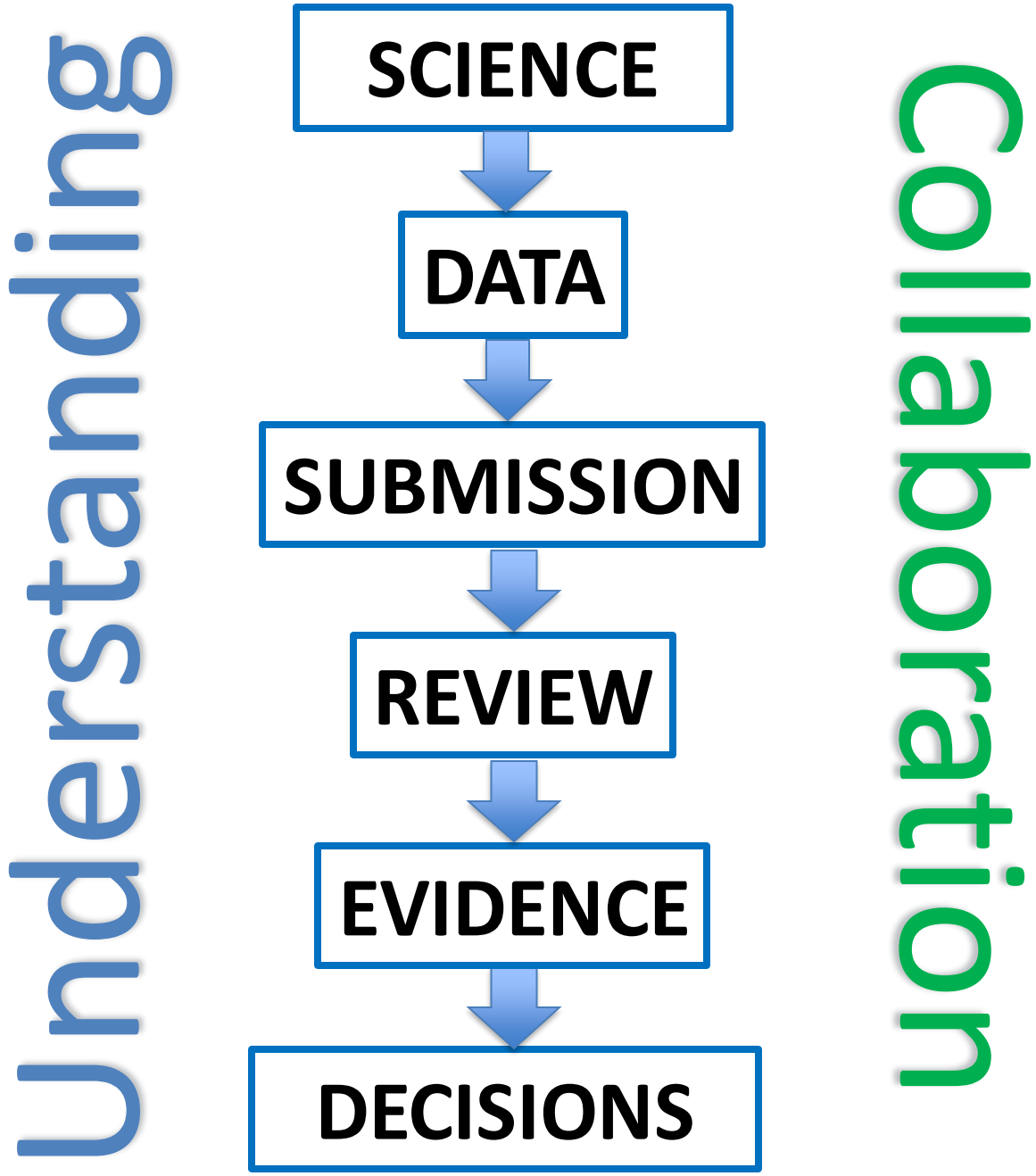
- Barriers to the use of CDISC Standards for RWD
- Steps Needed for Implementation of CDISC in Academia
- Making the Case for Using CDISC Standards for RWD
- Tools or Support Needed
- Benefits and Opportunities from Standardization of RWD
- How to Build Knowledge and Expertise on CDISC Implementation
- How to Reward and Promote the Use of CDISC Standards in Academia
- Standards for Devices and Wearables
- Patient's Perspective in RWD
- Collaborations with Other Standards and Initiatives
- The Future of RWD and CDISC Standards



https://www.cdisc.org/sites/default/files/2021-08/CDISC_RWD_Connect_Report_07-27-2021.pdf

What is Your Perspective?

What are the Next Steps that You Will Take to Help Make a Better Future Happen?



THANK YOU!!!

Please Stay Safe and Well …

Stephen.Wilson@fda.hhs.gov