The Journey Towards Standardization

Presented by Bess LeRoy;
Head of Standards Innovation,
CDISC

Meet the Speaker

Bess LeRoy

Title: Head of Standards Innovation

Organization: CDISC

Bess LeRoy is the Head of Standards Innovation at CDISC. Bess has been a CDISC team member since 2011. She has over 20 years of experience working in public health research and has held positions at the Framingham Heart Study, the Rotterdam Study, the Arizona Cancer Center, and the Critical Path Institute.

Bess has a BS from the University of Michigan, an MPH from Boston University School of Public Health, and is a doctoral candidate at Johns Hopkins Bloomberg School of Public Health.

Agenda

What Does It Mean To Be a Standard?

Stages and Attributes of Standards Development

TMF Standards Development Activities

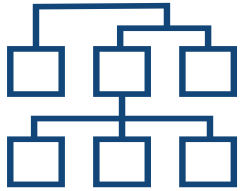
What Are Clinical Data Standards?

- Clinical data standards refer to a set of agreed-upon rules, formats, and guidelines for collecting, storing, and exchanging clinical information.

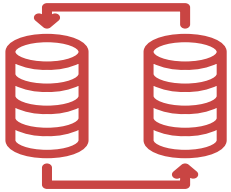
- These standards are crucial in healthcare and medical research because they ensure that data is consistent, reliable, and can be effectively shared and compared across different systems, studies, and organizations.

cdisc

What Can We Standardize?



Models



Data Exchange

Terminology

Process

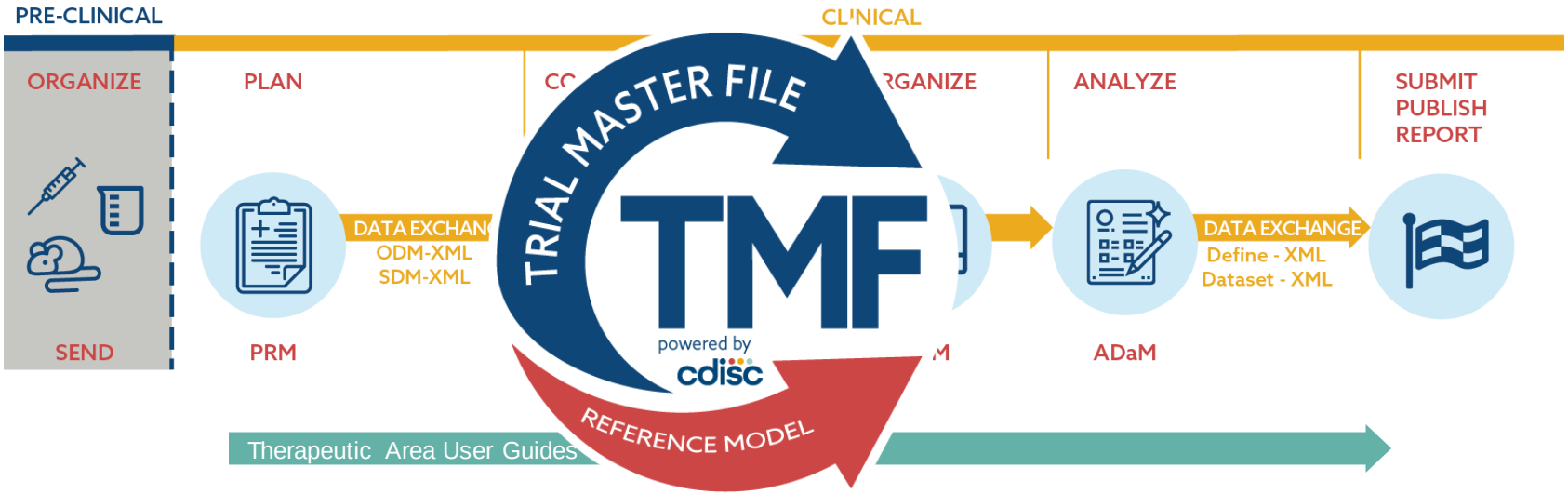
Identifiers

Data Types

Data Formats

Data Collection

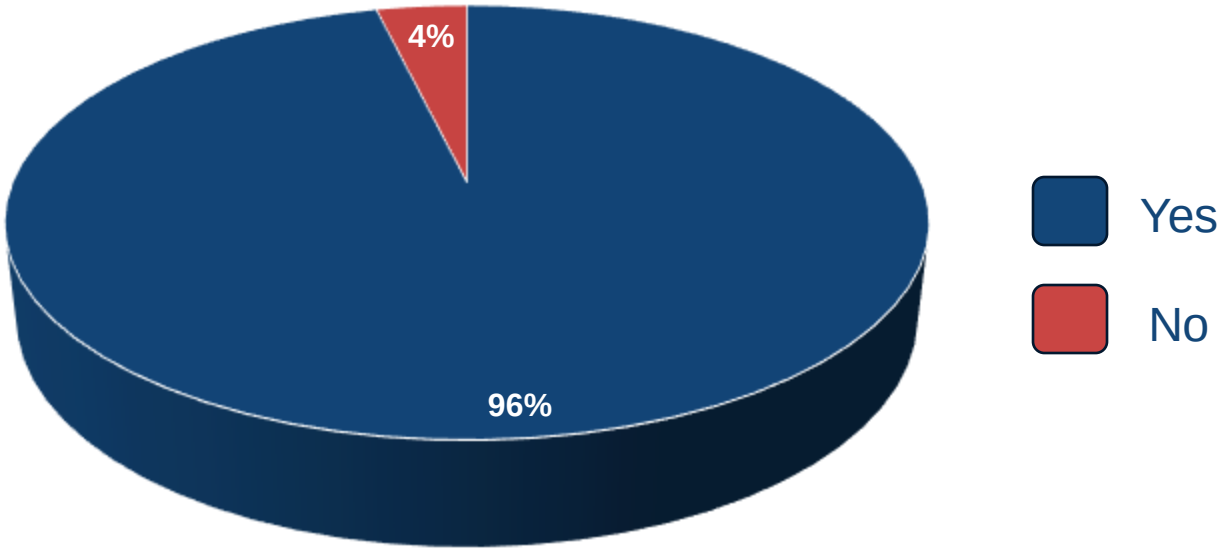
CDISC Standards



BRIDG, CONTROLLED TERMINOLOGY AND GLOSSARY

cdisc

TMF Reference Model as a De Facto Standard



*Based on a 2022 Survey

Importance of a Robust and Transparent Standards Development Process

CDISC Operating Procedure COP-001
Standards Development

US Federal Participation in the Development and Use of Voluntary Consensus Standards (Circular A-119)

- Standards developed by voluntary consensus standards bodies are often appropriate for use in achieving federal policy objectives and in conducting federal activities, including procurement and regulation

- The policies of Office of Management and Budget (OMB) Circular A-119 are intended to:
  - Encourage federal agencies to benefit from the expertise of the private sector
  - Promote federal agency participation in such bodies to ensure creation of standards that are useable by federal agencies
  - Reduce reliance on government-unique standards where an existing voluntary standard would suffice

cdisc

Source: OMB Circular A-119: Federal Participation in the Development and Use of Voluntary Consensus Standards and in Conformity Assessment Activities, January 2016

Attributes of Standards Development

Openness

- Processes are open and transparent
- Interested parties are provided meaningful opportunities to participate in standards development

Balance

- There should be meaningful involvement from a broad range of parties, with no single interest dominating the decision-making

Due Process

- Due process shall include documented and publicly available policies and procedures

Source: OMB Circular A-119: Federal Participation in the Development and Use of Voluntary Consensus Standards and in Conformity Assessment Activities, January 2016

Attributes of Standards Development

Appeals Process

- An appeals process shall be available for the impartial handling of procedural appeals

Consensus

- Consensus is defined as general agreement but not necessarily unanimity

Source: OMB Circular A-119: Federal Participation in the Development and Use of Voluntary Consensus Standards and in Conformity Assessment Activities, January 2016

CDISC Regulatory Collaborations

CDISC has a long history of regulatory collaboration.

FDA U.S. FOOD & DRUG ADMINISTRATION

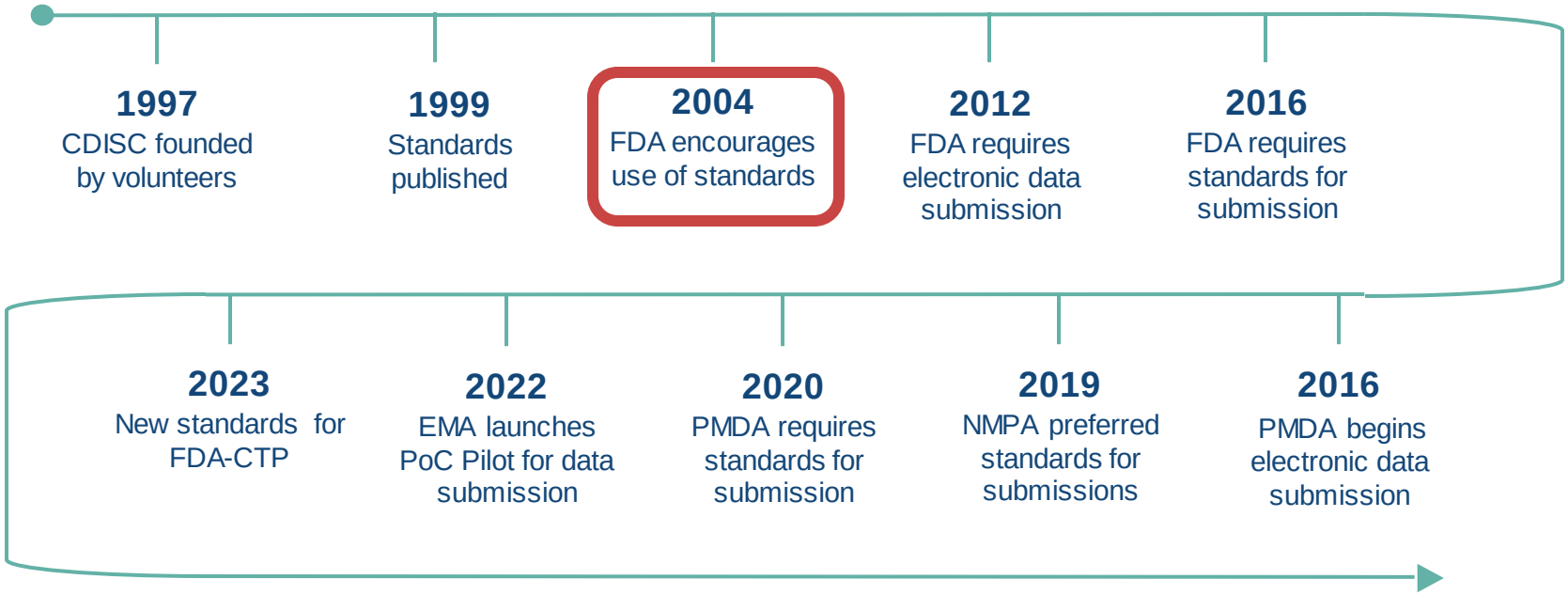
pmda 独立行政法人 医薬品医療機器総合機構 Pharmaceuticals and Medical Devices Agency

EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

NATIONAL MEDICAL PRODUCTS ADMINISTRATION
国家药品监督管理局

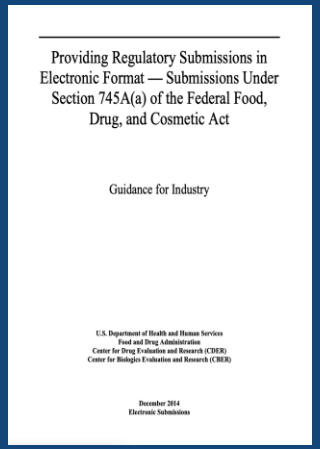
cdisc

CDISC Standards Adoption



CDISC Standards and Global Regulation

https://www.fda.gov/media/88120/download



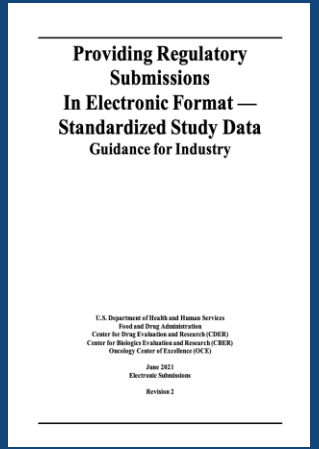
Providing Regulatory Submissions in Electronic Format — Submissions Under Section 745A(a) of the Federal Food, Drug, and Cosmetic Act

Guidance for Industry

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)

December 2014
Electronic Submissions

https://www.fda.gov/media/82716/download



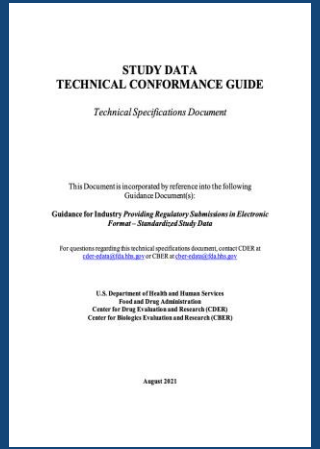
Providing Regulatory Submissions In Electronic Format — Standardized Study Data
Guidance for Industry

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)
Oncology Center of Excellence (OCE)

June 2021
Electronic Submissions

Revision 2

https://www.fda.gov/media/147233/download



STUDY DATA TECHNICAL CONFORMANCE GUIDE

Technical Specifications Document

This Document is incorporated by reference into the following Guidance Document(s):

Guidance for Industry *Providing Regulatory Submissions in Electronic Format – Standardized Study Data*

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

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)

August 2021

https://www.pmda.go.jp/english/review-services/reviews/0002.html

BINDING DOCUMENTS

- CDISC standards are required for submission to FDA and Japan PMDA
- CDISC standards are the only standards recognized for submissions by China NMPA
- CDISC standards can be used for patient-level data submission to EU EMA

Participating In Standards Development

Open Volunteer Process

Select the TMF volunteer group that you would like to join. (Please choose at least one)

TMF Groups

- TMF Subject Matter Experts
  Sub-group of experts who can be called upon for specific initiatives e.g. TMF Plan, e-Mail guidance, Inspection Readiness.
- Standards
  Sub-group for those who wish to get involved in the transition of the TMF Reference Model to being a Standard or in the further development of the Exchange Mechanism. A sub-group for Sponsors, CROs, Vendors and Consultants.
- Survey working group
  Sub-group for TMF Reference Model Annual Survey. The Group reviews and revises the survey questions, publishes the survey online, and then analyses and publishes the results.
- Website Content
  Sub-group for those who would like to meet regularly to discuss the format and content of the website. Output of this team will go to the Technical team.

Change Control Board Input

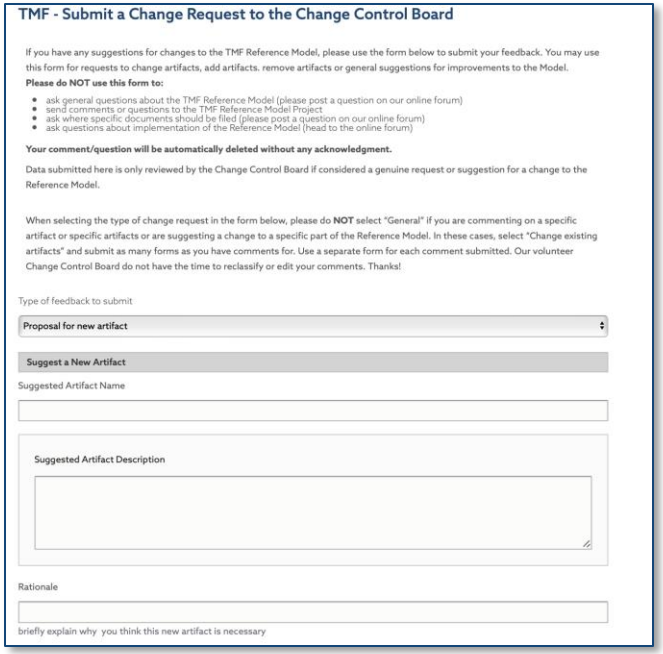
- Zone 01 Trial Management
- Zone 02 Central Trial Documents
- Zone 03 Regulatory
- Zone 04 IRB/IEC
- Zone 05 Site Management
- Zone 06 IP & Trial Supplies
- Zone 07 Safety Reporting
- Zone 08 Central & Local Testing
- Zone 09 Third Parties
- Zone 10 Data Management
- Zone 11 Statistics
- Devices
  Sub-team for trials using medical devices / diagnostics. Based on applicable guidance and best practice, this Group assesses artifacts and associated text that are specifically applicable to trials using medical devices and diagnostics.
- Non interventional studies
  Sub-group for Non-Interventional or Real World Studies. Based on relevant guidance and best practice, this Group assesses artifacts and associated text that would be expected for non-interventional studies (including observational/registry studies).

- Stay informed. Sign up for communications from CDISC.

cdisc

https://www.cdisc.org/volunteer/tmf

Submit a Request to the Change Control Board



TMF - Submit a Change Request to the Change Control Board

If you have any suggestions for changes to the TMF Reference Model, please use the form below to submit your feedback. You may use this form for requests to change artifacts, add artifacts. remove artifacts or general suggestions for improvements to the Model.

Please do NOT use this form to:

- ask general questions about the TMF Reference Model (please post a question on our online forum)
- send comments or questions to the TMF Reference Model Project
- ask where specific documents should be filed (please post a question on our online forum)
- ask questions about implementation of the Reference Model (head to the online forum)

Your comment/question will be automatically deleted without any acknowledgment.

Data submitted here is only reviewed by the Change Control Board if considered a genuine request or suggestion for a change to the Reference Model.

When selecting the type of change request in the form below, please do **NOT** select "General" if you are commenting on a specific artifact or specific artifacts or are suggesting a change to a specific part of the Reference Model. In these cases, select "Change existing artifacts" and submit as many forms as you have comments for. Use a separate form for each comment submitted. Our volunteer Change Control Board do not have the time to reclassify or edit your comments. Thanks!

Type of feedback to submit

Proposal for new artifact

Suggest a New Artifact

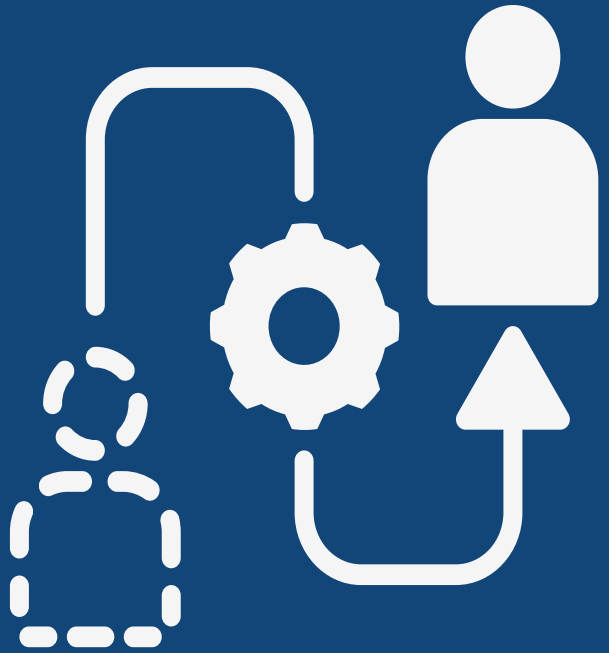
Suggested Artifact Name

Suggested Artifact Description

Rationale

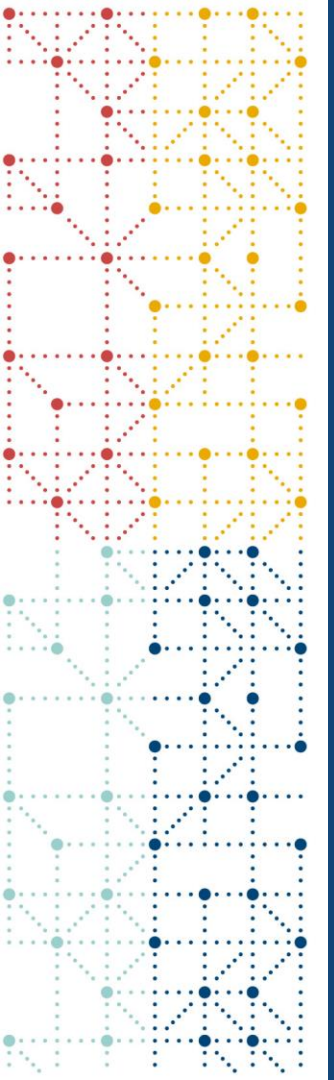
briefly explain why you think this new artifact is necessary

https://www.cdisc.org/tmf/change-request-form



Provide Feedback During Review Cycles

- ✓ TMF Community Review: 3 Weeks
- ✓ Public Review: 60 Days
- ✓ Terminology Review: 30 days
- ✓ Publication of all Public Review comments and dispositions



TMF Standards Development Activities

TMF Reference Model Standards Team

- **Migration to CDISC Library** - This initiative will focus on moving the TMF RM from the current spreadsheet format into the CDISC Library metadata repository to better align and expand the TMF RM and allow the management of the TMF RM as a standard.

- **Evolution of EMS/Interoperability** - The Exchange Mechanism Standard allows for the interchange of TMF content between systems and organizations. This initiative will continue to evolve the EMS and ensure alignment with other interoperability standards.

- **TMF RM Standard Alignment and Management** - This initiative will focus on implementing CDISC Standards Development processes within the TMF RM organization as the RM evolves into a formal standard. This initiative will also look at alignment of the TMF RM with other CDISC and industry standards as well as alignment with CDISC Controlled Terminology.

cdisc

Migration of TMF RM to CDISC Library

- The CDISC Library is a metadata repository where all CDISC standards are managed
  - Searchable through a web interface or via API
  - Ability to export the model in a spreadsheet format
- CDISC Library advantages
  - Ability to represent the reference model as a logical model vs. a tabular format
  - Robust way to control changes and version history
  - More easily map to other models and standards
  - Define sub-models for different types of trials
- Current Status: Developing formal use cases and data relationship diagram

Looking Toward the Future: Standardized Data Allows for the Use of Standard Tools

Conformance Rules:

- Can be used to ensure that a standard has been implemented correctly
- Component of all CDISC standards
- Vetted by the CDISC standards development teams
- Develop an open-source engine that serves as a Reference Implementations
- Publish the Rules in the CDISC Library and the engine under the CDISC Open Source Alliance (COSA)

Coming Up Next!

- ***An Overview of NCI EVS Services and Its Collaboration with CDISC;*** Jordan Li, NCI EVS

- ***A Deep Dive into the TMF Reference Model Change Control***; Kate Santoro, Intellia Therapeutics; Leila Ponce, Seagen

Relentless Collaboration

cdisc

Thank you!

bleroy@cdisc.org