CBER-CDER Data Standards Program Action Plan

Version: 1.4

FY2024 Q1 update

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REVISION HISTORY

Version Number	Revision Date	Description of Change
1.0	January 25, 2023	Revision of document structure to align with FY23-FY27 CBER-CDER Data Standards Strategic Goals
1.1	May 9, 2023	• FDA Data Standards Catalog added to Goal 2 • IDMP Guidance added to Goal 2 • Technical Specifications and Conformance Guide Updates removed from Goal 4 • Appendix B updated
1.2	August 14, 2023	• IDMP Guidance removed from Goal 2
1.3	November 9, 2023	• Quarterly project updates
1.4	February 15, 2024	• Added 356H Modernization Project under Goal 1 Objective 1 • Removed Submission Data Standards Assessment Project from Goal 1 Objective 1 • Added Publication of Final Guidance for Industry "Data Standards for Drug and Biological Product Submissions Containing Real-World Data" Project under Goal 2 • Added "Dataset JSON" as its own project under Goal 1 Objective 1

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Introduction

The purpose of the *CBER-CDER Data Standards Strategy* is to reinforce the ongoing commitment to the development, implementation, and maintenance of a comprehensive data standards program that will facilitate the pre- and post-market regulatory review process so that safe and effective medical products are available to patients.

This action plan aligns to the CBER-CDER Data Standards Strategy and reflects progress in CBER and CDER towards the defined goals and objectives. Projects selected for this action plan have started, are resourced and funded, and have a scope that is primarily standards related.

Purpose

This action plan provides a quarterly update to internal and external stakeholders, with an overview and progress update of current data standards initiatives. The plan will continue to be updated quarterly to reflect progress of current projects, as well as initiation of new projects. For information on prior quarters, refer to previous versions of the Action Plan.

Program Goals and Initiatives

The program goals are derived from the major areas of regulatory business activities. A detailed description of these major areas can be found in the CBER-CDER Data Standards Strategy.

The CBER-CDER Data Standards Program goals focus on four areas:

- Goal 1: Improve Data Standards for Regulatory Use
- Goal 2: Data Standards Policy
- Goal 3: Efficient Information Management
- Goal 4: Enhance Transparency and Stakeholder Engagement

The successful accomplishment of these goals may be achieved given sufficient resources, regulatory/legislative factors, and collaboration with stakeholders.

For each project in this section, the project title, description, update, and project stage(s) are provided. The project update reflects work done in the previous quarter (e.g., the FY2023 Q1 report highlights work from October to December 2022).

The project stage lists the typical stages a project might address during work for the project and are generally conducted in sequence from left to right. The definitions of the project stage are defined in **Appendix A**.

Project Stage
Requirements (REQT)
Analyze Alternatives (ALT)
Development (DEV)
Testing (TEST)
Adoption (ADOPT)
Implementation (IMPL)
Policy (POLICY)

Project Stage Status
In Progress
Pending
Complete
Not Applicable

Goal 1: Improve Data Standards for Regulatory Use

Projects related to Goal 1 address our continued collaboration with Standards Development Organizations to improve data standards and support initiatives for the adoption and adaptation of new and existing standards.

OBJECTIVE 1: Enhancement of Submission Formatting & Review

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
356H Modernization This project will be assessing feasibility and working towards development of a machine-readable approach for receiving the data requested in Form FDA 356h.	**Q1:** Engaging internally to assess regulatory and IT requirements of project.	In Progress	Pending	Pending	Pending	Pending	Pending	Pending

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
Study Data Standards Testing and Evaluation This CBER-CDER project tests new and updated study data standards and standards adjacent properties to establish FDA support and requirements.	**Q1:** **Initiated Assessments:** 1. CDISC SDTM For Observational Studies v1.0 2. CDISC Analysis Results Standard v1.0 3. CDISC Tobacco Implementation Guide v1.0 4. CDISC ADaM PopPK IG v1.0 5. CDISC SEND Tumor Combination v1.0 **Closed Assessments:** 1. CDISC Analysis Results Standard v1.0 2. CDISC SDTM For Observational Studies v1.0 3. CDISC Tobacco Implementation Guide v1.0	**Not Applicable**						
Dataset-JSON Standard This CDER project is a collaboration with PHUSE and CDISC to test the use of Dataset-JSON as potential replacement for XPT.	**Q1:** Received test submissions from industry demonstrating that FDA can receive dataset packages in Dataset-JSON format with no impact to data integrity as compared to the XPT files.	**Complete**	**Complete**	**In Progress**	**In Progress**	**Pending**	**Pending**	**Pending**

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
Questionnaires, Ratings and Scales (QRS) Assessment This CDER project is focused on evaluations of proposed standardized data structures that capture the information from Questionnaires, Ratings, and Scales administered to subjects during a clinical study and prioritize the data collection instruments indicated in the Clinical Outcomes Assessment (COA) area.	**Q1:** **Ongoing Assessments:** 1. CDISC PASI Feldman v1.0 2. CDISC PASI Fredriksson v1.0 3. CDISC PRO-CTCAE v1.0 4. CDISC PASI Bozek v1.0 5. CDISC PASI EMA v1.0 **Initiated Assessments:** 1. CDISC Functional Assessment of Anorexia/Cachexia Treatment v4 2. CDISC Functional Assessment of Cancer Therapy-Hepatobiliary v4 3. CDISC Functional Assessment of Cancer Therapy-General v4 **Closed Assessments:** 1. CDISC Short Form 36 Health Survey Standard, US Version 2.0 (SF36 V2.0 STANDARD) Supp V1.0- Public Comment/QRS Instrument	**Not Applicable**						
eCTD v4.0 Project – Phase 1 This CBER-CDER project is focused on the development, testing, adoption, and implementation of the next major version of the electronic Common Technical Document (eCTD), version 4 for new applications. FDA currently uses eCTD version 3.2.2.	**Q1:** Continued to test updates provided in eCTD Software, update regional specifications as needed (IG, CV, Validations, CTOC, TCG). Continued work on FDA Implementation.	**Not Applicable**	**Not Applicable**	**Not Applicable**	**In Progress**	**In Progress**	**In Progress**	**In Progress**

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
Pharmaceutical Quality/ Chemistry, Manufacturing, and Controls Data Standardization This CDER project with participation from CBER and CVM will identify and standardize data elements, terminologies, and data structures to enable automation of key analyses of Pharmaceutical Quality (PQ)/Chemistry, Manufacturing, and Controls (CMC) data to support more efficient and effective regulatory decision-making.	**Q1:** Continued development of Phase 2. Preparation for PQ/CMC track at January 2024 HL7 Connectathon.	Complete	Complete	In Progress	In Progress	Pending	Pending	Pending

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
IDMP Project This project has multiple use cases focused on the adoption of ISO Identification of Medicinal Product (IDMP) standards: 1. Medicinal Product ID (MPID), 2. Substance ID (SubID), 3. Pharmaceutical Product ID (PhPID), 4. Route of Administration, Dosage Form, and 5. Units of Measure. These ISO standards define medicinal product information for regional and global data sharing. Generally, the use cases focus on safety (e.g., ICSRs) and can support quality (e.g., PQ/CMC). Additionally, the Global IDMP Working Group (GIDWG) has been engaged with ISO and other regulators to ensure the standards are fit for global implementation.	**Q1:** Continued working with EMA and WHO-UMC, via GIDWG to promote the harmonized global ISO IDMP implementation framework. GIDWG has initiated the conduct end-to-end testing focused on Cross-Border healthcare, Pharmacovigilance, and Product Shortage use cases in Q1 & Q2 2024. The plan is to have a full report of findings and next steps at the annual GIDWG stakeholder meeting in Fall 2024. Additionally, the FHIR exchange standard for global exchange of IDMP information continues to be developed.	Complete	Not Applicable	Complete	In Progress	Pending	Not Applicable	Not Applicable

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
Assessing Applicable Data Standards for Use in Submission of Real-World Data to FDA FDA is examining Real-World Data (RWD) and data standards to support submission of RWD to FDA. This assessment will help determine a roadmap for applying data standards for RWD submission to FDA.	**Q1:** Begin review of prioritized data elements with internal stakeholders to gather feedback.	In Progress	In Progress	Pending	Pending	Pending	Pending	Pending
Source Data Capture from EHRs: Using Standardized Clinical Research Data This CDER project is working to demonstrate an approach to collecting data for clinical trials that populates an electronic data capture (EDC) system directly from an electronic health record (EHR) system and document improvements to efficiency and accuracy compared to traditional methodologies.	**Q1**: Continued development.	Complete	Not Applicable	In Progress	Not Applicable	Not Applicable	Not Applicable	Not Applicable

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
SPL FHIR FDA is examining HL7 FHIR as an alternative to Structured Product Labeling (SPL). Currently the SPL data exchange standard is a modified version of HL7 version 3 data standard. Since HL7 is sunsetting HL7 in favor of HL7 FHIR, FDA is working to determine if an HL7 FHIR can support the same functionality and use cases as the current SPL standard.	**Q1:** Continued buildout of FHIR Implementation Guide including revisions reflecting FHIR version R4B. Planning upgrade of FHIR R5.	Complete	Complete	In Progress	In Progress	Pending	Pending	Pending
Grant: Investigating Support for 21 CFR 11 Compliance Using HL7 FHIR As a use case for enabling implementation of audit trailing and provenance capabilities in Real-World Data research, this grant is evaluating approaches to build out elements of the HL7 FHIR standard to support these capabilities. An initial use case is to add audit trail support to FHIR Resources used for recording Patient Reported Outcomes (PROs).	**Q1:** Grantee planning further engagement with HL7 workgroups.	**Not Applicable**						

OBJECTIVE 2: Improve Pre and Postmarket Safety Surveillance Data

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
FDA Adverse Event Reporting System (FAERS) II CDER and CBER project is receipt and processing of Investigational New Drug (IND) and post-market safety reports submission using E2B R3 standards.	**Q1:** FAERS II E2B R3 Industry Testing Phase II completed. No further update/enhancement was identified during testing.	Complete	Complete	InProgress	InProgress	In Progress	In Progress	In Progress

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
Biologics Effectiveness and Safety (BEST) Innovative Methods (IM) Leverages Artificial Intelligence, Machine Learning, FHIR standards and SMART-on-FHIR to develop a semi-automated adverse event (AE) reporting system from EHRs. The system uses such innovative methods to detect exposures/outcomes of biologics and facilitates validation and reporting of flagged cases to the FDA. Project goals include development of tools, methods and techniques needed to reduce the burden on providers to report AEs accurately and efficiently, which is critical to strengthen the post market active surveillance program of CBER regulated products.	**Q1:** Continued development..	Complete	Complete	Complete	In Progress	Pending	Pending	Pending

Goal 2: Data Standards Policy

Projects aligned under Goal 2 provide governance and expertise for the development and revision of data standards policies related to the regulation of human drugs and biologic products. The continued implementation and refining of governance processes ensure proper oversight during the development, publication, and maintenance of guidance documents detailing the use of data standards, terminologies, and exchange formats for regulatory submissions.

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
Post Approval Changes Rulemaking & Submission Standards This CBER-CDER project is focused on improving the usability of post approval submissions data.	**Q1:** Rulemaking proposal is currently undergoing internal agency review.	Complete	Not Applicable	In Progress	Not Applicable	Not Applicable	Not Applicable	Pending
Study Data Technical Conformance Guide (sdTCG)	**Q1:** Published the October 2023 sdTCG. Published a special edition of the sdTCG in December 2023.	**Ongoing Semi-Annual Publications**						

Project Title & Description	Project Status	Project Stages						
		REQT	ALT	DEV	TEST	ADOPT	IMPL	POLICY
FDA Data Standards Catalog	**Q1:** Published Version 10.2 in December 2023.	**Ongoing Updates**						
Publication of Final Guidance for Industry "Data Standards for Drug and Biological Product Submissions Containing Real-World Data" Published as part of a suite of FDA guidance documents under the FDA Real World Evidence Program. The document clarifies that submissions subject to section 745A(a) of the FD&C Act that contain study data derived from RWD sources must be in electronic format using the study data standards currently supported by FDA as specified in the Data Standards Catalog. This guidance provides recommendations to sponsors for complying with section 745A(a) of the FD&C Act when submitting study data derived from RWD sources in an applicable regulatory submission using standards specified in the Catalog.	**Q1:** Final Guidance published December 22, 2023.	**Complete**						

Goal 3: Efficient Information Management

Projects aligned under Goal 3 promote efficient review process because the data submitted is in a predictable and consistent format that can be more easily used by analytic systems.

As outlined in the CBER-CDER Data Standards Strategy document, technology is critically important and serves as an enabler for reviewers to access and use large amounts of data and information that is received and generated. Several data standards development projects are already underway, as highlighted earlier in this document, to promote access to high-quality, standardized data including the PQ/CMC Standardization and IDMP projects. CDER and CBER also continues to define and enhance ways to better capture information created internally to support continued knowledge management activities. Progress towards the Goal 3 objectives will be highlighted annually in the Data Standards Program Annual Assessment and not tracked quarterly.

Goal 4: Enhance Transparency and Promote Stakeholder Engagement

Efforts supported under Goal 4 enhance transparency and promote stakeholder engagement in its decision-making regarding adoption of new standards, especially required standards. In addition, these efforts are promoted through the following activities:

Program Operations	Updates
eCTD Submission Standards	File Format Specification updated and published December 2023.
Action Plan	FY2023 Q4 published November 13, 2023.
Annual Assessment	Published February 17, 2023.
Outreach Opportunities, Public Meetings & Educational Activities	FDA Webinars are planned to focus on various data standards topics CDISC Interchange, Falls Church, U.S.A. CDISC Interchange, Seoul, South Korea Clinical Trials Transformation Initiative DIA Annual Meeting, Japan GRx+Biosims Annual Meeting HL7 Weekly Calls, Work Group Meetings and Connectathon ICH General Assembly Meeting ICH M2, M8, and M4Q(R2) Implementation Work Group Meetings IDMP/GIDWG & UNICOM TransAtlantic Meetings ISO Meeting TC215 Work Group 6 Monthly FDA/CDISC Technical Meetings SBIA webinar - Toward Global IDMP Implementation: A Focus on Global Use Cases Vulcan FHIR Accelerator, co-leads and/or participants in multiple tracks

Appendix A: Project Stage Definitions

Stage Name	Stage Description
Requirements	A project with the objective of developing a standard, or utilizing an existing standard for the receipt, processing, review, and archive of data used in regulatory review is considered a data standards project.
Analyze Alternatives	A projects approach to the identification and analysis of alternatives to solve a data standards problem.
Development	The approach to address approved changes to data standards or data standards policy.
Test	A project may be required to test (CDER) study data standards that is adaptable based on the situation. Provides a process to determine if a standard meets the needs of the FDA and should be accepted by the FDA.
Determine Data Standard Adoption (Adoption)	The project is approved and proceeds towards the adoption.
Implement Standard (Implementation)	The advancement to implementing an approved data standard need or change.
Policy	FDA may publish an FRN or guidance, as well as relevant technical specifications or technical conformance guides, as needed.

Appendix B: Glossary of Acronyms

ADaM	Analysis Data Model
AE	Adverse Events
Catalog	FDA Data Standards Catalog
CBER	Center for Biologics Evaluation and Research
CDER	Center for Drug Evaluation and Research
CDISC	Clinical Data Interchange Standards Consortium
COA	Clinical Outcomes Assessment
eCTD	Electronic Common Technical Document
EHR	Electronic Health Record
FHIR	Fast Healthcare Interoperability Resource
FRN	Federal Register Notice
FY	Fiscal Year
GSRS	Global Substance Registration System
HL7	Health Level Seven
ICH	International Council for Harmonization
ICSR	Individual Case Safety Report
IDMP	Identification of Medicinal Product
IND	Investigational New Drug
ISO	International Organization for Standardization
PDUFA	Prescription Drug User Fee Act
PQ/CMC	Pharmaceutical Quality/Chemistry, Manufacturing, and Controls
QRS	Questionnaires, Ratings, and Scales
SDTM	Study Data Tabulation Model
SDTMIG	Study Data Tabulation Model Implementation Guide
SEND	Standard for Exchange of Nonclinical Data
SENDIG	Standard for Exchange of Nonclinical Data Implementation Guide
SENDIG-AR	Standard for Exchange of Nonclinical Data Implementation Guide: Animal Rule
SPL	Structured Product Labeling
sdTCG	Study Data Technical Conformance Guide
TAUG	CDISC Therapeutic Area User Guide

TCG	Technical Conformance Guide
UMC	Uppsala Monitoring Centre
UNII	Unique Ingredient Identifier