CBER-CDER Data Standards Program 2024 Annual Assessment

March 2025

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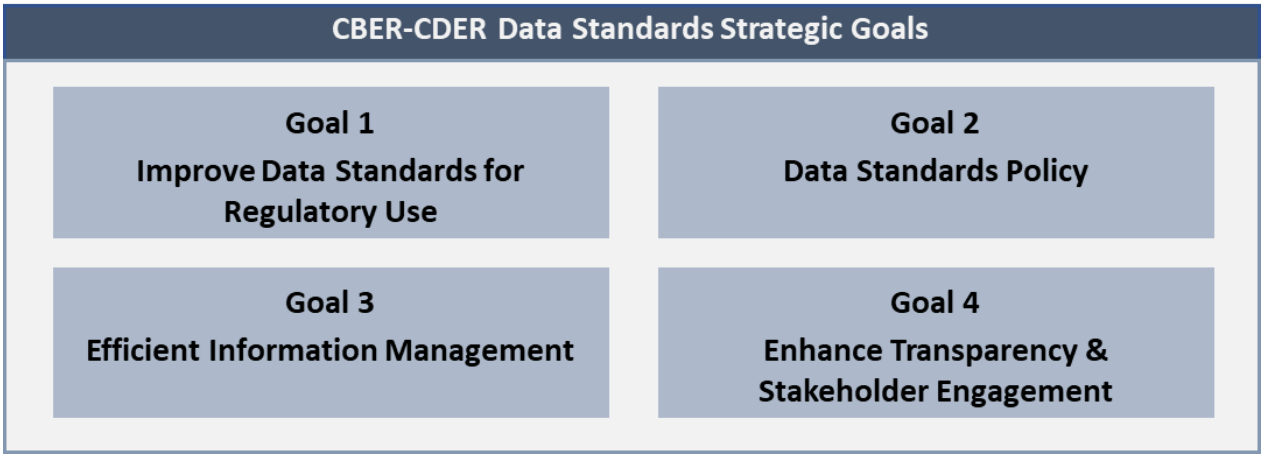
1 Introduction

FDA publishes an Annual Assessment for CBER-CDER's Data Standards Program (DSP) to provide a progress update to stakeholders reflecting the last calendar year. The previous year's assessment is available on the CDER DSP website. Further information for most projects referenced throughout this Annual Assessment is available in the Action Plan.

2 CBER-CDER Data Standards Program at a Glance

This assessment highlights the projects and ongoing efforts that cover the identification of need, development, testing, adoption, implementation, and maintenance of study data standards required for the efficient and effective review of regulatory submissions. The Annual Assessment is organized to align with the Data Standards Strategy and as of FY23, is now mapped to the four major areas of regulatory business activity of the CBER-CDER Strategic Plan (pictured below). The following sections highlight the program's accomplishments.



2024 Summary of Accomplishments

- Completed the first pilot testing of the SPL-FHIR Implementation Guide with 8 industry organizations, gathering valuable feedback to enhance its quality and accuracy.
- Published the first release of the HL7 FHIR Implementation Guide (STU 1.0.0) for PQ/CMC Stage 1 concepts and successfully tested it at the HL7 Connectathon.
- Global Identification of Medicinal Products (IDMP) Working Group (GIDWG) conducted end-to-end test cases on pharmacovigilance, product shortages, and cross-border healthcare, successfully assigning Global Substance IDs to 96% of selected substances and Global PhPIDs to 90% of medicinal products.
- FDA Adverse Event Reporting System (FAERS II) accepted ICH E2B(R3) XML submissions for pre- and post-market safety reports enhancing its capabilities for unified safety surveillance. The Safety Reporting Portal was updated to support ICH E2B(R3) data standards, with 25 companies adopting the new submission format by February 2025.

- Biologics Effectiveness and Safety (BEST) Innovative Methods (IM) initiative advanced its capabilities by integrating its HL7 FHIR Implementation Guide into the Interoperability Standards Advisory Reference Edition, strengthening its role in adverse event (AE) reporting and launched studies to scale AE detection in EHRs.
- Completed over 30 assessments of study data models, technical documents, and terminologies to evaluate suitability for regulatory review.
- Published 2 updates to the FDA Data Standards Catalog.

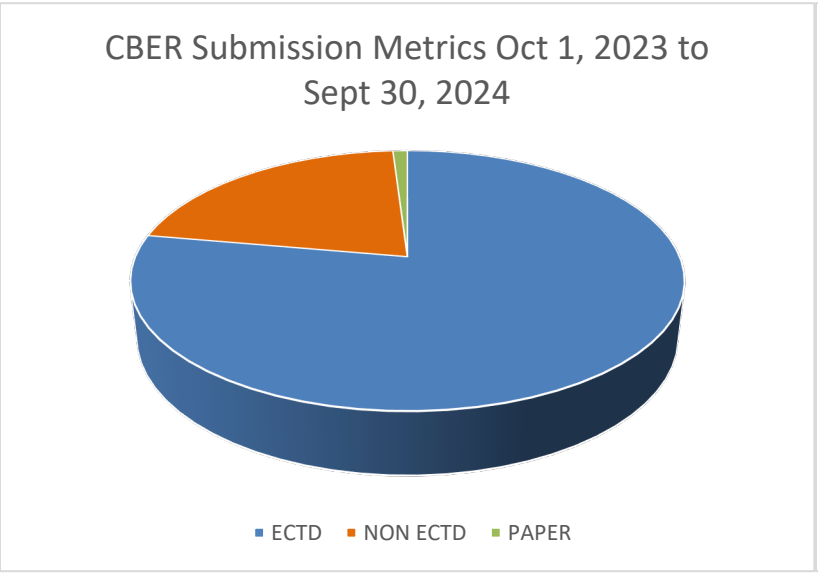
3 Impact of Requiring Standards

FDA continues to evaluate and implement data standards for study data and submissions and requires applications to use these standards as defined in the FDA Data Standards Catalog. The Data Standards Program's strategic goal areas and objectives were identified as part of an Agency assessment to evaluate the degree of implementation of electronic submissions and data standards, the readiness of data standards, effectiveness of electronic review tools and training, and impact of standards and electronic submission on the review environment.

4 2024 Electronic Submission Metrics

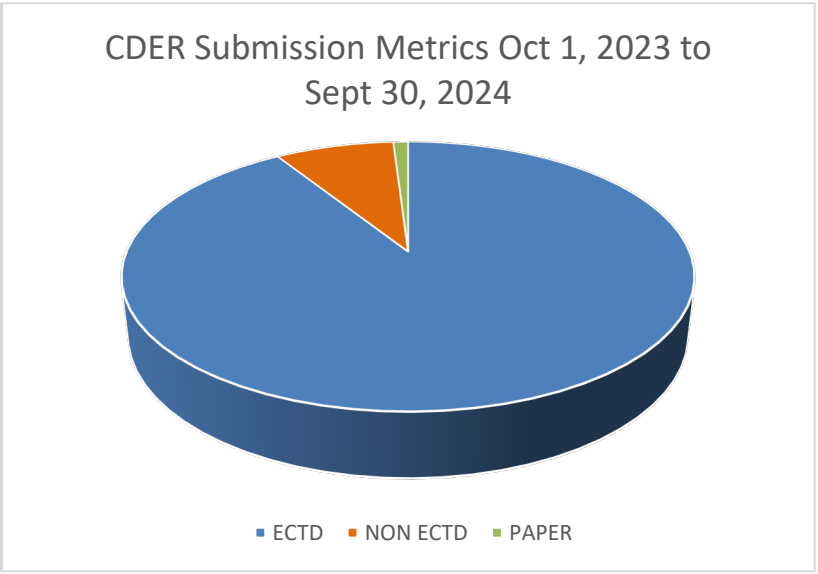
Analysis of FY 2024 data indicated that 91% of all submissions to CDER were in eCTD format, 8% in other electronic formats, and 1% paper. There was near 100% compliance with application types required in eCTD. For CBER, more than 77% of all submission were in eCTD format, with 22% in non-eCTD formats and approximately 1% paper submissions. CBER's metrics include submissions that are not subject to the eCTD requirement.

Figure 1. Percent of All Submissions to CBER and CDER by Electronic Format (FY 2024)



44,039 Total Submissions

ECTD	77%
NON ECTD	21%
PAPER	1%



383,004 Total Submissions

ECTD	91%
NON ECTD	8%
PAPER	1%

In 2020, FDA expanded electronic options for transmitting non-eCTD submissions. CDER's NextGen Portal began accepting non-eCTD submissions to Research IND and DMF Type III applications. Utilizing NextGen or ESG provides an easier and faster way to transmit a non-eCTD submission compared to paper or physical media (i.e., CD/USB Drive).

In April 2024, the FAERS system began accepting IND safety reports. Previously, commercial IND safety reports were only submitted to FDA in eCTD format. Utilizing the FAERS system requires the E2B(R3) format. Some industry partners have started utilizing the new E2B(R3) submission format. In 2024, FAERS received 900+ E2B(R3) IND safety reports.

5 2024 Data Standards Program (DSP) Year in Review

In 2024, CBER and CDER's DSP continued to make significant progress in multiple fronts including, but not limited to, updates to the FDA Data Standards Catalog and the Study Data Technical Conformance Guide, and conducted over 30 assessments of study data models, technical documents, and terminologies to evaluate suitability for regulatory review. The PQ/CMC project published its first release of the HL7 FHIR IG (STU 1.0.0) for PQ/CMC Stage 1 concepts and successfully tested it at the HL7 Connectathon. The Agency also contributed to the completion of the Global PhPID End-to-End test. The Global IDMP Implementation Working Group (GIDWG) with collaboration from EMA, ANVISA, Health Canada, and SwissMedic finalized the first draft of global PhPID business rules and submitted to ISO TC215 WG6 in 2024 for ISO IDMP revision consideration. The Agency continued development of its SPL-FHIR POC system

in 2024 and completed the project's first pilot testing of the SPL-FHIR Implementation Guide with 8 industry organizations, gathering valuable feedback to enhance its quality and accuracy.

5.1 Goal 1: Improve Data Standards for Regulatory Use

5.1.1 Objective 1: Enhancement of Submission Formatting and Review

Data Standards and Real-World Data

Consistent with the 21st Century Cures Act and the Food and Drug Omnibus Reform Act of 2022 FDA has issued guidance about the use of real-world data (RWD) to support regulatory decisions. RWD is data relating to patient health status and/or the delivery of health care routinely collected from a variety of sources. Some of the most prominent sources of RWD are Electronic Health Record (EHR) systems used by the vast majority of hospitals and primary care clinics in the United States and insurance claims databases used to document billing for medical care events. Many other sources of RWD also exist and continue to emerge. FDA's robust Real-World Evidence Program, in addition to facilitating the publication of a suite of guidances, includes continuing engagement and collaboration with interested parties to explore continuing advances in this space. FDA efforts to support submission of study data from RWD sources include evaluations by FDA comparing RWD to currently accepted data standards at FDA, clarifying the pertinent supported data standards for study data based on RWD sources, and supporting various projects addressing the needs of RWD use for research and regulatory submissions such as the OneSource project (see below). In 2024 FDA internally considered data standard elements optimized for study data derived from RWD, which the Agency plans to include in a discussion document for public release. If those standards are adopted by an SDO, it may make industry submission of RWD to FDA more efficient.

Dataset-JSON Standard

In 2023, FDA initiated participation in a new PHUSE project aimed at evaluating Dataset-JSON as an alternative transport format for the Agency to receive electronic submissions. In addition, FDA received several pilot submissions from industry containing the current XPT format and converted JSON file formats to capture data integrity. CBER and CDER representatives provided technical feedback to stakeholders on the ability to receive and accept these submissions. The FDA will continue evaluating Dataset-JSON as an alternative transport format.

During 2024, CBER and CDER continued internal testing of datasets in a Dataset-JSON format using all FDA process, systems, and tools. FDA is continuing to assess the impact to clinical and nonclinical reviewers and industry. There will be ongoing testing of our systems and tools that use SAS Transport v5 (XPT).

eCTD V4.0 Project – Phase 1

Phase 1 of CBER and CDER's implementation of eCTD v4.0 is the acceptance of new applications and subsequent submissions (e.g., amendments, supplements).

In 2023, an eCTD v4.0 Technical Pilot was completed. Eleven companies participated in the pilot and six companies submitted test submissions. The scope of the pilot was the submission of

original eCTD v4.0 applications and subsequent submission with a focus on the eCTD v4.0 enhancements (e.g., document reuse, one-to-many and many-to-one lifecycle).

CBER and CDER performed testing on our eCTD software, upgraded the electronic submissions process, and started the systems integration enhancements to incorporate the eCTD v4.0 functionality.

CDER and CBER published a Federal Register Notice announcing that support begins on September 16, 2024, for original eCTD v4.0 applications and subsequent submissions.

Engagement with HL7

CBER and CDER continues to actively participate in the HL7 Biomedical Research and Regulation (BR&R) workgroup. The BR&R areas of interest encompass clinical and translational research, both regulated and non-regulated, and the subsequent regulatory submissions and information exchanges to bring new products to market and to ensure safe use throughout the product lifecycle. The BR&R facilitates the development of common standards and the maintenance and enhancement of the research-focused domain analysis model for clinical research information management across a variety of organizations, including national and international government agencies and regulatory bodies, private researchers, research organizations, sponsored research, CROs and other interested entities. A shared semantic view is essential if the clinical research community is to achieve computable semantic interoperability, both for itself and as part of the larger healthcare and life sciences communities. The BR&R will seek to assure that related or supportive standards produced by other HL7 groups are robust enough to accommodate their use in regulated clinical research through participation as appropriate. The group also monitors information interchange standards developed outside of HL7 and attempts harmonization of information content and representation of such standards with the HL7 standards.

As part of FDA's participation with HL7, the HL7 FHIR Accelerator program for clinical research was jointly created by academia, sponsors, regulatory and translational research organizations, including TransCelerate Biopharma, FDA, NIH, JHU, HL7, CDISC, as well as several large professional societies. CBER and CDER are actively involved in Vulcan, participating in its Steering Committee, Advisory Board, and Technical Expert group to ensure that the solution is aligned with our regulatory review needs. Since 2021, the Agency also participated in multiple Vulcan FHIR Connectathon tracks including those focusing on RWD (which FDA also co-leads) and adverse events.

ICH eCTD File Tag Controlled Terminology

In 2022, FDA submitted a proposal to ICH M8 to expand the controlled terminology list for their eCTD file tags. The proposed file tags would be specific to the business needs around regulatory review and would serve reviewers by helping them to efficiently locate materials within a submission package that have been properly tagged. ICH has accepted the proposal and published the updated controlled terminology list in May 2024.

IDMP Project

As FDA focuses on the challenges of the global supply chain and foreign sourcing of medicinal products, the Agency continues to participate and promote conformance to international harmonized IDMP to foster the safety of medications throughout the world. FDA conducted a Global PhPID pilot with UMC to assess alternative solutions for ISO IDMP standards. The findings and recommendations are included in the 2023 revision of ISO 11239 and TS20440. The Agency also collaborated with EMA and UMC to establish the GIDWG (Global IDMP Working Group) to assess and promote global implementation of ISO IDMP standards based on the success of Global PhPID project and Global Vaccine Initiative. GIDWG conducted five projects to further investigate solutions and processes to address identified gaps of ISO IDMP standards and is working with ISO TC215 Working Group 6 to improve IDMP standards for global implementation. In Q4 2023, GIDWG completed a successful cross-border healthcare test case during HL7 Connectathon which demonstrated the benefit of using GIDWG global PhPID to identify like medicinal products in multiple jurisdictions. GIDWG conducted end-to-end test cases on pharmacovigilance, product shortage, and cross-border healthcare care, and PhPID operating model in 2024 to further fine tune the global IDMP implementation framework. The end-to-end test successfully assigned Global Substance IDs to 96% of the 150 selected substances and Global PhPIDs to 90% of the 2,947 medicinal products provided by nine regulators, based on the established framework and business rules. Test results and findings were shared with ISO TC215/WG6 and during September 2024 GIDWG annual meetings and public meetings.

Pharmaceutical Quality/Chemistry, Manufacturing, and Controls Data Standardization

The PQ/CMC Data Elements and Terminologies Data Standardization Project continued work related to characterizing data elements and terminologies for information used in support of Module 3 of eCTD-based drug applications. An overall goal of this initiative is the development of standardized, structured and computable data standards for PQ/CMC submissions, ensuring consistent representation of concepts. In 2022, the project finalized PQ/CMC Phase 1 requirements of FHIR resources and development of FHIR exchange standards, and all requirements for PQ/CMC Phase 1 data domains were included in the FHIR R5 ballot. In March 2022, FDA published an FRN [https://www.regulations.gov/document/FDA-2022-N-0297-0001] that provides the updated PQ/CMC Phase 1 Data elements and controlled terminology as well as Draft mappings [Data Elements & Terminologies Document] to HL7 FHIR. The focus of the 2022 FRN was to seek industry input on the FHIR mappings. In May 2023, FDA released another FRN requesting comments on further additions to data elements and terminology for PQ/CMC to support multi-layer products and manufacturing processes for solid oral products. The May 2023 FRN also established an open docket for further notices for comment about the PQ/CMC initiative. Additionally, work commenced in assessing the range of considerations to be addressed regarding regulation and guidance in order to ensure a successful implementation of PQ/CMC for submissions to FDA. At the January 2024 HL7 Connectathon, FDA tested the first iteration FHIR Implementation Guide, encompassing “Stage 1” of PQ/CMC (product composition, general substance information, and multiple quality specification reports). The Implementation Guide was then balloted for HL7 review and comment and ultimately published as Release 1, Standard for Trial Use (STU) 1.0.0. The next iteration Implementation Guide added support for “Stage 2”

concepts (additional substance information, batch formula, and characterization of product impurities), and was tested at the September 2024 Connectathon and submitted in December for the January 2025 HL7 ballot cycle.

Questionnaires, Ratings and Scales (QRS) Assessment

It is a common practice for sponsors to collect data in support of a clinical trial using specific data collection instruments (i.e., questionnaires, ratings or scales (QRS)). Codifying data structures for study data that has been collected using an instrument is an effort undertaken by SDOs. Under the Data Standards Testing contract, CDER has been evaluating these codified data structures for suitability. These dataset structures can come from instruments qualified by the COA Project, existing standards, or therapeutic area extensions to supported data models. Well-defined dataset structures ensure that data submitted to the Agency is fit-for-purpose. The Agency collaborates with industry to develop these dataset structures through the QRS effort.

In 2024, the QRS effort has evaluated 7 QRS data structures or related artifacts. FDA has sent substantive comments back to the SDO during development which has resulted in data structures that are more fit for purpose.

Source Data Capture from EHRs: Using Standardized Clinical Research Data (OneSource)

Electronic Source (eSource) data refers to the use of electronically recorded information as a source of data directly transferred to data systems used for clinical trials. The device or system that records the original data can include many items such as wearable devices and mobile apps. One of the larger potential sources of eSource data are EHR systems. A large amount of clinical trial participant data, which needs to be entered in research electronic case report forms (eCRFs), already exists in healthcare provider's EHR systems. However, EHR and eCRF data are generally collected in separate, non-compatible formats and exist in separate systems. This results in patient information being manually re-entered into the eCRF system, significantly slowing down workflow and increasing the risk of inaccuracies due to duplicate entry. This is a major barrier to research on real-world use of drug and biological products.

A number of initiatives exist to help mitigate these challenges, including CDER's supported projects that aim to demonstrate approaches for collecting eCRF data, stored on research Electronic Data Collection (EDC) systems, directly from an EHR system in an FDA-compliant way. These automated approaches demonstrate relevant improvements in efficiencies and potential returns on investment versus the current manual methodology. The OneSource project is a CDER-led project in collaboration with the University of California San Francisco. This project uses EHRs as the electronic Source (eSource) in I-SPY 2.2 Breast Cancer Trial, conforming to open, consensus-based standards. Phase III of this project aims to accomplish the following: 1) enhance the adverse event detection and reporting process by implementing standards-based electronic Patient-Reported Outcome (PROs); and 2) identify key data elements from electronic case report forms for breast cancer trials, focusing primarily on the I-SPY 2 family of trials and provide the data elements to the HHS/Assistant Secretary for Policy Technology United States Core Data for Interoperability (USCDI), aimed at for broader sharing of electronic health information to support patient care.

OneSource platform originally developed for I-SPY Breast Cancer Trial, is being reused in I-SPY COVID Trial and has been implemented for eSource data capture of laboratory results and concomitant medications at 15 sites for the I-SPY COVID Trial.

SPL FHIR

FDA maintains and updates its data standards to ensure continuous support of critical regulatory functions in light of exchange standards technology enhancements and upgrades. For example, FDA has been proactively reviewing the technology behind the Structured Product Labeling (SPL) standard used to support a wide range of regulatory uses including labeling. SPL is the current standard behind a range of information processed by FDA and public information systems and is implemented using the HL7 Version 3 standard. As HL7 is transitioning to the more advanced FHIR standard, FDA is conducting an assessment of the FHIR capability to support the full range of current functions and, potentially, new use cases in a more efficient, robust, and sustainable way. The FDA is creating a proof-of-concept intake system that would allow for the submission of the new FHIR standard as well as the SPL standard and will be working with industry on a pilot program to test the system. In 2024, FDA held its first pilot testing of the SPL-FHIR Implementation Guide with eight industry organizations. This was successful and also provided significant feedback to help improve the quality and accuracy of the SPL-FHIR Implementation Guide.

Study Data Standards Testing and Evaluation

This project involves testing an external organization's study data models, technical documents, terminologies, or exchange formats, for their ability to meet FDA's regulatory review needs and identify potential areas of concern. Findings and results of this effort contributes to the Agency's decisions on standards adoption. Below is a list of assessments in 2024.

1. CDISC SDTM Supplement for the Columbia-Suicide Severity Rating Scale Already Enrolled Subjects (C-SSRS) v2.0 questionnaire
2. CDISC SDTM Supplement for the Columbia-Suicide Severity Rating Scale Baseline (C-SSRS) v2.0 questionnaire
3. CDISC SDTM Supplement for the Columbia-Suicide Severity Rating Scale Since Last Visit (C-SSRS) v2.0 questionnaire
4. CDISC SDTM Supplement for the Short Form 36 Health Survey Standard, US Version 2.0 (SF36 V2.0 STANDARD) Supp V1.0 questionnaire.
5. CDISC SDTM Supplement for Functional Assessment of Anorexia/Cachexia Treatment v4 questionnaire
6. CDISC SDTM Supplement for Functional Assessment of Cancer Therapy-Hepatobiliary v4 questionnaire
7. CDISC SDTM Supplement for Functional Assessment of Cancer Therapy-General v4 questionnaire
8. SDTMIG v3.4 information package for PMDA
9. SDTMIG v4.0
10. ADAM v3.0
11. SENDIG v4.0

12. SDTM v3.0
13. PHUSE iADRG
14. Considerations for deprecating PP from SDTM
15. Considerations for Industry to split CC data out of RS Domain
16. SDTM for Observational Studies v1.0
17. Analysis Results Standard v1.0
18. Tobacco Implementation Guide v1.0
19. DDF Phase 3
20. CDISC Biomedical Concepts
21. SDTM Dataset Specializations
22. Define.xml v2.1.7
23. Dataset JSON v1.1
24. ADaM PopPK IG v1.0
25. SEND Tumor Combination v1.0
26. Rare Diseases TAUG v1.0
27. SDTMIG-MD v1.1
28. ADAMIG-MD v1.0
29. SDTM for Observational Studies v1.0
30. RECIST v1.1

5.1.2 Objective 2: Improve Pre and Post Market Safety Surveillance Data

Biologics Effectiveness and Safety (BEST) Innovative Methods (IM)

In support of CBER's mission for post-market safety surveillance, the Biologics Effectiveness and Safety (BEST) Innovative Methods (IM) initiative aims to utilize health information exchanges to streamline the detection of adverse events (AE) and improve the quality of AE reports submitted to CBER. The BEST IM platform uses the emerging HL7 FHIR standards to request and receive additional clinical data from healthcare providers to enrich the reported cases of adverse events. The BEST platform has the potential to improve the quality of information regarding adverse events reported to the FDA while minimizing the burden on providers and the public. BEST IM program progressed over the years, starting in 2021, developed and tested the BEST IM platform, a proof-of-concept adverse events validation and reporting system. In 2022, in collaboration with eHealth Exchange, the largest health information exchange network in the United States, the BEST IM platform launched a pilot to connect to production systems of 11 healthcare systems, as early adopters of the use of FHIR for public health reporting. Leveraging the emerging standards, BEST IM, in 2021, developed an HL7 FHIR Implementation Guide: Profiles for ICSR Transfusion and Vaccination Adverse Event Detection and Reporting was updated in August 2023 to address comments received and the changes were approved by HL7. In addition, the Implementation Guide has been included under AE Reporting within the 2024 Interoperability Standards Advisory Reference Edition published by the Office of the National Coordinator for Health Information Technology. Building on the past years' successes, in 2025, the BEST IM team is conducting two studies to explore solutions for scaling AE detection in EHRs. The studies are exploring the use of a multi-tier phenotyping approach (using simple code sets at the healthcare

systems' sites, and complex algorithms on the BEST IM Platform), to lower the burden on reporters. The BEST IM is exploring the use of enriching the FHIR bundles via advanced NLP techniques to convert unstructured data to structured form and leverage the interoperable clinical quality language (CQL) in the detection of AEs from FHIR bundles.

FDA Adverse Event Reporting System (FAERS) II

FAERS is a mission critical system for FDA. FAERS supports OC, CBER and CDER's safety surveillance program for investigational products, marketed drug, therapeutic biological products and cosmetics. The FAERS II program provides a modernized system for safety surveillance, including pre-market and post-market safety reports along with product quality defect reports making it a one-stop shop solution for intake, triage, case processing, reporting and analytics. The modernized FAERS Platform also allows for enhanced and unified data analytics and signal management lifecycle solution utilizing ICH E2B(R3) data standard.

To prepare for accepting ICH E2B(R3) standard, industry testing was conducted. Six companies participated in testing, where they prepared the XML files using the ICH E2B(R3) data standards along with the regional data elements. These files were then submitted to FAERS via the Electronic Submission Gateway (ESG). FAERS received 259 Pre- & Post-market safety reports. The scope of the testing was to send appropriate acknowledgements to the companies once the files are received and for FAERS to process the test files. After the conclusion of industry testing, further updates and internal testing was conducted, and the system was deemed ready to start accepting ICH E2B(R3) post market files.

To assist with testing, FDA provided the FDA E2B(R3) Validator tool to facilitate the validation of the E2B(R3) XML files generated from industry's safety database. This validator tool provides a web-based interface that enables submitters to submit an E2B(R3) XML file in a test environment and check the validity or correctness of the file. The validation status and results are displayed to the user in real-time.

In January 2024, FDA started accepting E2B(R3) XML submissions for post-market safety reports and in April 2024, FDA started accepting E2B(R3) XML submissions for premarket safety reports. Additionally, the Safety Reporting Portal (SRP) used by companies to submit safety reports via a web-based interface was also updated to transmit safety reports to FAERS using ICH E2B(R3) data standard. Details are posted on the FAERS Electronic Submission web page (https://www.fda.gov/drugs/fdas-adverse-event-reporting-system-faers/fda-adverse-event-reporting-system-faers-electronic-submissions). As of February 2025, there are 25 companies that have started submitting safety reports to FAERS using ICH E2B(R3) data standard.

5.2 Goal 2: Data Standards Policy

The Data Standards Policy goal supports the clear and consistent communication of policies that facilitate the effective adoption and implementation of regulatory submission data standards. The Data Standards Program (DSP) collaborates closely with Subject Matter Experts and review offices across CBER and CDER to accurately capture their data requirements and recommendations. This collaborative approach helps ensure that the Agency's data standards

policies remain aligned with regulatory decision-making needs, enhance data quality, promote interoperability across systems, and support harmonization with other regulators.

Guidance Documents

No guidance documents issued in 2024.

FDA Data Standards Catalog

The FDA Data Standards Catalog lists the study data models, associated technical documents, terminologies, and exchange formats FDA supports and requires for use in regulatory submissions for eCTD modules 4 and 5. In 2024, the Agency published 2 updates to the FDA Data Standards Catalog.

IDMP

No guidance documents issued in 2024.

Real-World Data

No guidance documents issues in 2024.

Study Data Technical Conformance Guide (sdTCG)

To ensure that current information continues to be available, new versions of the technical specifications associated with Providing Regulatory Submissions in Electronic Format — Standardized Study Data guidance, specifically the Data Standards Catalog and sdTCG, were updated throughout 2024. The sdTCG provides specifications, recommendations, and general considerations on how to submit standardized study data using the FDA Data Standards Catalog. In 2024, the Agency published 3 versions of the sdTCG (as opposed to the usual 2).

5.3 Goal 3: Efficient Information Management

The CDER Enterprise Data Governance project aims to improve data trust, discoverability, and clarify context to enable efficient data-driven decision-making and the use of Artificial Intelligence (AI) at the center.

The CDER Enterprise Data Governance project was initiated in 2022 with the goal of developing and implementing a data governance framework across CDER data domains such as Facilities Data and Products Data.

In 2023, the project continued to refine the Data Governance operating model and collaborate with other enterprise level data governance efforts within CDER to improve data management processes. Specifically, the CDER Enterprise Data Governance project began to enhance the maturity of data governance across people, process, and technology. The team introduced new key data governance roles and responsibilities and focused on operationalizing these roles at the office level. In 2022 and 2023, the project also created standard templates for key data governance artifacts that defined the Center’s internal data expectations in a more consistent way and also introduced common data quality dimensions such as data integrity, data consistency, and data accuracy from which data quality rules could be defined.

In 2023, the project conducted a Current State Assessment to identify data maturity gaps across CDER offices and inform a CDER-wide data strategy to address these gaps. The assessment identified inefficiencies reported by staff, including data access, data context, data discoverability, data trust and data tools challenges. In 2024, the project designed a data strategy and 5-year roadmap that details goals, activities, and data governance-specific roles and responsibilities to mature data capabilities (including AI, data architecture, data management, data analytics and data literacy).

The project continued to catalog a complete view of all CDER data in 2024 to improve data discoverability (allowing data analysts to efficiently find data they need to make decisions or build new AI models) and data context (needed to quickly understand what data can be used for decision-making and AI analysis). In 2024, the project distributed a data call across CDER to compile a comprehensive CDER Dataset Inventory containing metadata on datasets stored outside of CDER systems, shared datasets (from other centers, agencies, and organizations) and purchased datasets (from vendors). The project published the inventory to CDER staff to help data users efficiently locate, access and use needed data. The project continues to implement an enterprise data catalog tool to catalog data within CDER systems and act as a centralized metadata repository where trusted data knowledge can be built collaboratively to reduce redundancies. The project connected the tool to core CDER systems and onboarded users (giving them access to metadata and lineage across these systems) to enable efficient discovery of needed data and its context. The project created data lineage in the enterprise data catalog tool that shows upstream and downstream impacts of architectural changes which can be used to efficiently implement AI and retire legacy systems. Cataloging CDER data expedites regulatory processes by improving the accuracy and efficiency of data analysis through increased data discoverability and context.

The project continues to manage data elements that are core to CDER's mission to increase data quality, trust and context for efficient data-driven decision-making. In 2023, the project introduced common data quality dimensions such as data integrity, consistency and accuracy from which data quality rules could be defined. In 2024, the project trained data stewards, captured business glossary entries, and drafted and configured data quality rules in the center's enterprise data quality tool. Improving CDER's data quality practices enables data analysts to efficiently identify data that can be trusted for decision-making and AI through increased data trust and context. Also, these efforts reduce upfront data analysis, limit redundant cleansing, and support better regulatory decisions as data quality improves the accuracy of data analysis.

In 2024, the project continued to focus on data management optimization at CDER by designing a scalable solution to data management to improve the way in which teams acquire, build, and use data for their use cases. The project identified common CDER data management inefficiencies, which include manual extraction of data and redundant data storage leading to actions taken to use AI to reduce time and cost spent. The project established processes to reduce the need for redundant data entry and curation, resulting in more efficient use and management of data. This work provides industry with more consistent data to ultimately generate faster insights to create new generic drug products reducing costs for the public. In addition, these efforts will help facilitate more efficient drug application review as structuring data will prepare it

for AI use and help reviewers spend less time extracting and entering data, thus accelerating downstream team analyses.

The project continues to prepare CDER staff to enable efficient data-driven decision-making by upskilling them on topics such as metadata, business glossary, data quality. In 2024, the project developed a data governance resources SharePoint to explain foundational data governance topics. The project provided 250+ staff members training to make them more efficient at data management and lay a data management foundation to prepare the center for wider use of AI. In addition, the project established common data practices providing a consistent avenue for reusability of data materials and defining data expectations in a more consistent way through templates such as a data dictionary, business glossary, data quality rules, etc.

For 2025, both Facility Data and Product Data control boards will continue to integrate CDER Enterprise Data Governance (eDG) practices and literacy, which in turn promotes consistency in the way artifacts are defined and further standardize CDER's data-related operations. The PDCB (Product Data Control Board) will manage a new initiative in 2025: Common Product Dictionary --- the projects goal is to enable CDER systems to share product and substance information through a common set of data elements, which in turn enhances clarity on the data lineage, including the data source and ensuing series of changes. The board will provide oversight and manage CDER office collaborations with eDG to ensure accuracy and consensus of terminologies that are being implemented.

5.4 Goal 4: Enhance Transparency and Stakeholder Engagement

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

Program Operations	Updates
Outreach Opportunities, Public Meetings & Educational Activities	Clinical Trials Transformation Initiative GIDWG Annual and Public Meetings HL7 Weekly Calls, Work Group Meetings and Connectathon ICH E2B Expert Working Group Meetings ICH M2 Expert Working Group Meetings ICH M8 Implementation Work Group Meetings ICH M11 Expert Working Group Meetings Monthly FDA/CDISC Technical Meetings PHUSE CSS

Program Operations	Updates
	SBIA Presentations on IDMP, PQ/CMC, and the Data Standards Program Vulcan FHIR Accelerator, co-leads and/or participants in multiple track

FDA actively engages with Clinical Data Interchange Standards Consortium (CDISC) to adopt and implement standards that enable the exchange of study data, further enhance data quality, and improve the consistency and reliability of clinical trial data submitted for regulatory review. The Agency ensures alignment with CDISC standards through collaborative discussions, and participations in workshops, public meetings, and other ongoing communications.

FDA maintains a collaborative relationship with the International Organization for Standardization (ISO), particularly regarding the global implementation of ISO IDMP and ICSR standards. Through this collaboration the Agency aims to enhance global alignment of regulatory data management and medicinal product identification processes across international borders, which can ultimately improve global pharmacovigilance and may facilitate efforts to address drug shortages.

FDA participates in the International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use (ICH), a global initiative involving regulatory authorities and industry representatives from multiple member countries. Through participation in ICH working groups and conferences, the Agency contributes expertise and insights to the development of guidelines that support harmonization of international regulatory practices with an aim to reduce duplication of efforts.

6 Moving Forward - 2025 CBER-CDER Data Standards Program Direction

With required electronic study data standards and electronic submissions in effect or coming into effect, respectively, CBER and CDER continues to focus on ensuring that the review environment is capable of supporting receipt, processing and review of all electronic data. Continued collaboration with SDOs and stakeholders to ensure long-term sustainability of supported data standards as well as the testing of new standards and terminologies, will be a key focus of the Data Standards Program.

To support communication of new technical specifications, conformance guides, and relevant standards information, the sdTCG will be updated in March and October of 2025 and posted on the CDER Data Standards Program webpage. FDA webpages (e.g., PDUFA VII Informatics page, Study Data Standards Resources, PQ/CMC, IDMP Webpage) will be updated throughout 2025. These updates will ensure a consistent external web presence, revised materials, and interactive tools for both internal and external stakeholders.

In addition to these project areas, FDA is committed to continuing support for demonstration efforts that highlight standards-based technology solutions for collection of related healthcare and clinical research information. Continuing the DSP's progress in 2025, CBER and CDER's focus remains on initiatives such as SPL on FHIR, PQCMC Standardization, IDMP, and evaluation of Dataset-JSON for study data submissions. For updates on a comprehensive list of ongoing projects in 2025, see the DSP Action Plan published quarterly on the CDER Data Standards Program webpage.

Appendix A: Glossary of Acronyms

ADAM	Analysis Data Model
AE	Adverse Event
ANVISA	Brazilian Health Regulatory Agency
BEST - IM	Biologics Effectiveness and Safety - Innovative Methods
BR	Business Rules
BR&R	HL7 Biomedical Research and Regulation Group
CBER	Center for Biologics Evaluation and Research
CCB	Change Control Board
CDER	Center for Drug Evaluation and Research
CDISC	Clinical Data Interchange Standards Consortium
CDM	Common Data Model
COA	Clinical Outcome Assessment
DDF	Digital Data Flow
DSP	Data Standards Program
DSDG	Data Standards and Data Governance Board
E2B	Electronic-to-Business
eCRF	Electronic Case Report Form
eCTD	Electronic Common Technical Document
EDC	Electronic Data Collection
EHR	Electronic Health Record
EMA	European Medicines Agency
EUA	Emergency Use Authorization
FAERS	FDA Adverse Event Reporting System
FD&C Act	Federal Food, Drug, and Cosmetic Act
FHIR	Fast Healthcare Interoperability Resources
FRN	Federal Register Notice
FY	Fiscal Year
GIDWG	Global IDMP Working Group
GSRS	Global Substance Registration System
HL7	Health Level Seven
ICH	International Council for Harmonization of Technical Requirements for Pharmaceuticals for Human Use
IDMP	Identification of Medicinal Product
IG	Implementation Guide
IND	Investigational New Drug
ISO	International Organization for Standardization
JHU	Johns Hopkins University
JSON	JavaScript Object Notation
NDA	New Drug Application
NIH	National Institutes of Health
PDUFA	Prescription Drug User Fee Act
PhUSE	Pharmaceutical Users Software Exchange
PhPID	Pharmaceutical Product Identification
PQ/CMC	Pharmaceutical Quality/ Chemistry, Manufacturing, and Controls
QRS	Questionnaires, Ratings and Scales (QRS) Assessment
RECIST	Response Evaluation Criteria in Solid Tumors
RWD	Real World Data

SBIA	Small Business & Industry Assistance
SDO	Standards Development Organization
SEND	Standard for Exchange of Nonclinical Data
SENDIG	Standard for Exchange of Nonclinical Data Implementation Guide
SOP	Standard Operating Procedure
SPL	Structured Product Labeling
TA	Therapeutic Area
TCG	Technical Conformance Guide
TRC	Technical Rejection Criteria
UMC	Uppsala Monitoring Centre
XML	Extensible Markup Language