Data Standards Strategy
FY2023 – FY2027

Center for Biologics Evaluation and Research (CBER)
Center for Drug Evaluation and Research (CDER)

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1.0 INTRODUCTION

The Center for Biologics Evaluation and Research (CBER) and the Center for Drug Evaluation and Research (CDER) have developed this Data Standards Strategy. The goal of the Data Standards Strategy is to reinforce their ongoing commitment to the development, implementation, and maintenance of a comprehensive data standards program that will facilitate the efficient and effective review of regulatory submissions.

CBER's mission is to ensure the safety, purity, potency, and effectiveness of biological products including vaccines, allergenics, blood and blood products, and cells, tissues, and gene therapies for the prevention, diagnosis, and treatment of human diseases, conditions, or injury.[1] The mission of CDER is to protect and promote public health by helping to ensure human drugs are safe and effective for their intended use, meet established quality standards, and are available to patients.[2] Together the two centers will leverage their combined resources, talent and expertise to maximize stakeholder collaboration, policy development, and project implementation to develop and use data standards for the effective and efficient review of pre- and postmarket submissions of safety and efficacy data.

The framework of the CBER-CDER Data Standards Program (DSP) is aligned with FDA's strategic plans, guiding principles, and key regulations and statutes; together, they form the data standards program framework.

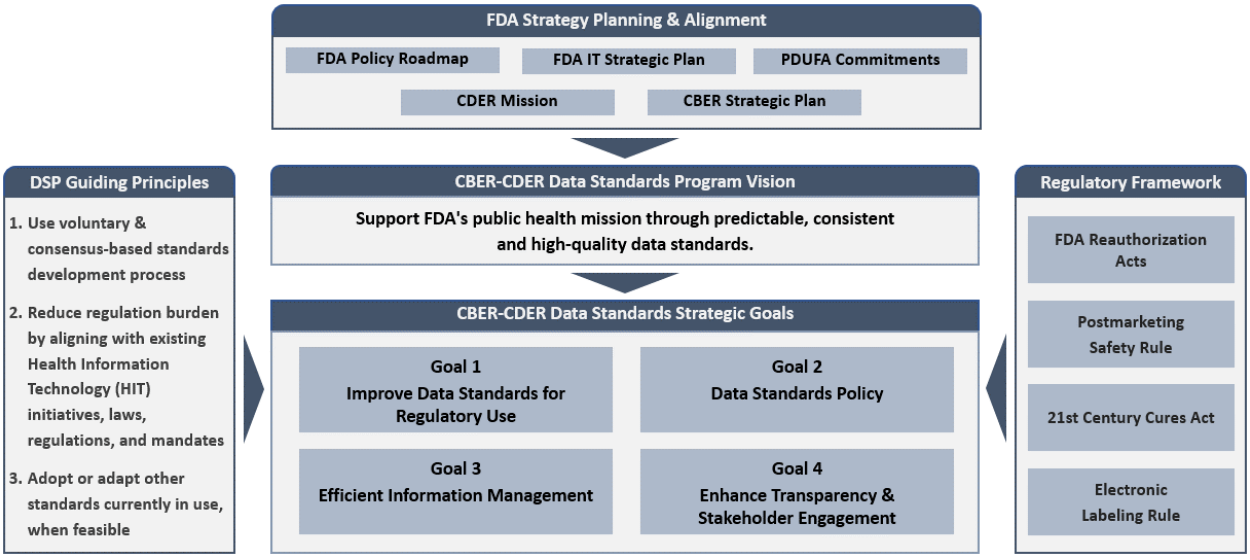

Figure 1: CBER-CDER Data Standards Program Framework

[1] https://www.fda.gov/about-fda/center-biologics-evaluation-and-research-cber/cber-vision-mission
[2] https://www.fda.gov/about-fda/fda-organization/center-drug-evaluation-and-research-cder

1.1 DATA STANDARDS PROGRAM VISION

Vision Statement:

Support FDA's public health mission through predictable, consistent and high-quality data standards.

CBER and CDER (FDA) continue to receive an increasing amount of submission data in non-standard formats requiring significant FDA staff time and effort to decipher and process. Although FDA requires certain data standards, which have improved the review and information exchange process, we intend to fully utilize the benefits offered by interoperable exchange mechanisms and direct system-to-system communication to seek further efficiencies.

1.2 GUIDING PRINCIPLES

Guiding principles are tenets followed in FDA's adoption of data standards, and aligned with the FDA Strategic Policy Roadmap,[3] FDA Data Modernization Action Plan and Technology Modernization Action Plan,[4] CBER Strategic Plan,[5] CDER Mission, 21st Century Cures Act and the commitments under user fee acts as appropriate.[6]

FDA's Data Standards Program is focused on three principles:

1. Use voluntary and consensus-based standards development processes.[7]
2. Reduce regulation burden by aligning with existing Health Information Technology (HIT) initiatives, laws, regulations, and mandates.
3. Adopt or adapt other standards currently in use, when feasible.

1.3 STAKEHOLDERS

We recognize that there are stakeholders, including regulated industry, health care professionals, patients, standards development organizations (SDOs), technology providers, as well as other government and non-government organizations (NGOs), and the public, who have a critical role in FDA's efforts to promote the use of open, consensus-based data standards. FDA collaborates

[3] https://www.fda.gov/about-fda/reports/healthy-innovation-safer-families-fdas-2018-strategic-policy-roadmap
[4] Data Modernization Action Plan | FDA, FDA's Technology Modernization Action Plan | FDA
[5] https://www.fda.gov/media/81152/download
[6] User Fee Amendments at the time of publication of this document:
https://www.fda.gov/industry/fda-user-fee-programs/prescription-drug-user-fee-amendments
https://www.fda.gov/industry/fda-user-fee-programs/generic-drug-user-fee-amendments
https://www.fda.gov/industry/fda-user-fee-programs/biosimilar-user-fee-amendments
https://www.fda.gov/industry/fda-user-fee-programs/over-counter-monograph-drug-user-fee-program-omufa
[7] Per Office of Management and Budget Circular No. A-119, and the National Technology Transfer and Advancement Act of 1995

with these stakeholders to develop new data standards, and to update existing ones. Examples of external stakeholders include, but are not limited to:

- Other Regulatory Authorities
- Other Federal Agencies
- Clinical Data Interchange Consortium (CDISC)
- Health Level 7 (HL7)
- International Council on Harmonisation (ICH)
- International Organization for Standards (ISO)
- World Health Organization (WHO)

2.0 REGULATORY FRAMEWORK

FDA's goal is to improve the predictability, consistency, transparency, and efficiency of the regulatory review process. Much of the improvement in the review process to date has hinged upon the submission of standardized electronic drug and biological product application data and the implementation of electronic review tools and systems. This strategy is supported by the regulations, statutes and guidances that promote or require the use of data standards in electronic regulatory submissions. The key areas of the regulatory framework include the following, with the applicability of each law and regulation to data standards outlined in appendix B:

- Final Rule, Requirements for Submission of Labeling for Human Prescription Drugs and Biologics in Electronic Format, 2003[8]
- Food and Drug Administration Amendments Act (FDAAA), 2007[9]
- Food and Drug Administration Safety and Innovation Act (FDASIA), 2012[10]
- Final Rule, Postmarketing Safety Reports for Human Drug and Biological Products; Electronic Submission Requirements, 2014[11]
- 21st Century Cures Act, 2016[12]
- User Fee Amendments[13]

[8] 21 CFR parts 314 and 601 (2003)
[9] https://www.gpo.gov/fdsys/pkg/PLAW-110publ85/pdf/PLAW-110publ85.pdf
[10] https://www.gpo.gov/fdsys/pkg/USCODE-2015-title21/pdf/USCODE-2015-title21-chap9-subchapVII-partD.pdf
[11] 21 CFR parts 310, 324 and 600 (2014)
[12] https://www.congress.gov/114/plaws/publ255/PLAW-114publ255.pdf
[13] User Fee Commitments at the time of publication of this document:
PDUFA: https://www.fda.gov/media/151712/download
GDUFA: https://www.fda.gov/media/153631/download
BSUFA: https://www.fda.gov/media/152279/download
OMUFA: https://www.fda.gov/media/106407/download

3.0 STRATEGIC GOALS

The CBER-CDER DSP 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. A quarterly progress report of CBER-CDER's Data Standards initiatives is documented in the Data Standards Action Plan.

The diagram below provides a high-level outline of the key activities within the current regulatory submission and review process. Each strategic goal is mapped onto this diagram aligning to the area that the goal was designed to support.

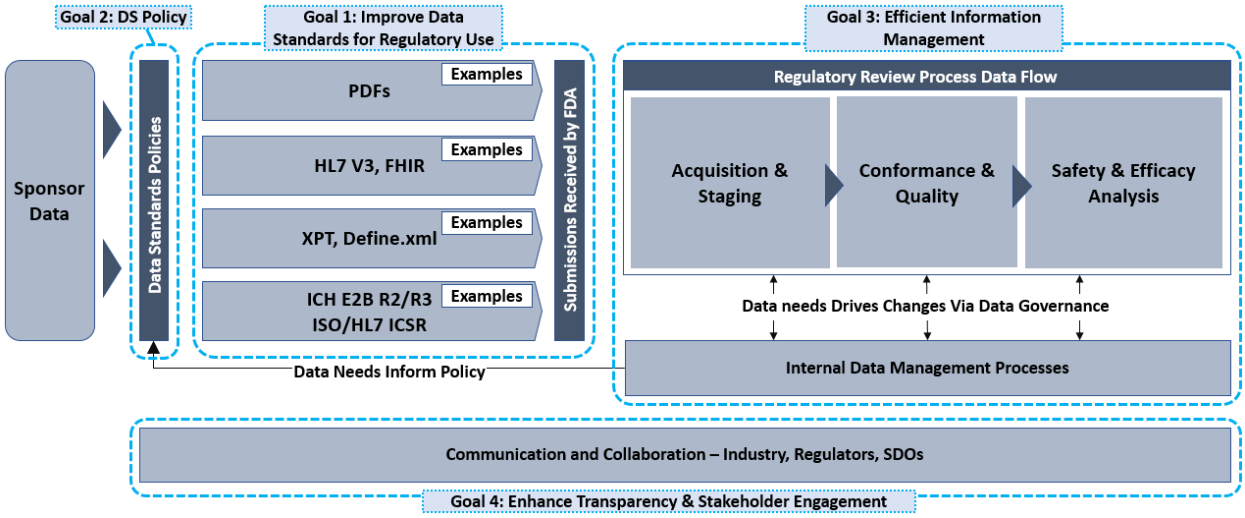

Figure 2: Data Standards Program Strategic Goals Alignment

The DSP serves a critical function within the lifecycle of regulatory submissions and reviews. As CBER and CDER continue to collaborate with SDOs to improve data standards and support initiatives for the adoption and adaptation of new standards (Goal 1), our data standards policy provides industry with guidance to facilitate conformance to the required or supported standards (Goal 2). Once submissions are received, data within these submissions are unpacked and processed by various systems and tools to support regulatory review activities. Oversight of review data and associated workflows are managed by CBER and CDER's internal data

governance processes (Goal 3). Improvement opportunities identified during regulatory reviews leading to new standardization initiatives are communicated to external stakeholders for progress reporting and public input. CBER and CDER are also committed to explore collaboration opportunities with industry, SDOs, and other regulators where feasible (Goal 4).

3.1 GOAL 1: IMPROVE DATA STANDARDS FOR REGULATORY USE

FDA will continue to improve data standards for the receipt and exchange of regulatory data to achieve predictable and consistent results. FDA will identify efficiencies to allow data to be systematically captured, processed, and analyzed, in alignment with user fee commitments.

OBJECTIVE 1: ENHANCEMENT OF SUBMISSION FORMATTING & REVIEW

To facilitate submission and review of bioinformatics and computational biology information, FDA will continue to develop data standards and revise or publish new guidance on how to format submissions and comply with technical validation criteria. FDA will work to advance global harmonization of these standards and methodologies and evaluate approaches for innovative use of healthcare data (e.g., Digital Health Technology Data, Real World Data). FDA will work globally to advance harmonization of these standards and methodologies.

OBJECTIVE 2: IMPROVE PRE AND POSTMARKET SAFETY SURVEILLANCE DATA

FDA will improve the ability to analyze premarket safety data by implementing standardized electronic premarket safety reports. FDA will improve data quality, accuracy, and integrity of post-market risk management strategies, pharmacovigilance and surveillance of drug and biologics product data by using data standards to communicate essential risk evaluation and mitigation strategy information.

3.2 GOAL 2: DATA STANDARDS POLICY

CBER and CDER's DSP provides governance and expertise for the development and revision of data standards policies related to the regulation of human drugs and biologic products. CBER and CDER will continue to implement and refine governance processes to 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.

FDA established a data standards governance framework of policies, processes, and organizational structures to manage and account for its data standards initiatives, including SDO collaboration. The Data Standards Advisory Board (DSAB) serves as a review and advisory body for data, exchange, and terminology standards initiatives relevant to FDA and to identify and sponsor cross-organizational data standardization needs of the Agency. DSAB, along with

representatives from each FDA Center and Office, focuses on Agency-level data standards priorities.

At the Center level, CDER's Data Standards and Data Governance Board (DSDG) and CBER's Data Standards Coordinating Committee (DSCC) ensure cross-center collaboration, communication, and alignment with respect to data standards strategy, development, implementation, and policy. DSDG and DSCC are responsible for the CBER-CDER Data Standards Strategy and the associated Action Plan, as well as the ongoing planning, coordination, and progress-tracking of data standards projects.

3.3 GOAL 3: EFFICIENT INFORMATION MANAGEMENT

Data standards contribute to an efficient review process because the data submitted is in a predictable and consistent format that can be more easily used by analytic systems.

OBJECTIVE 1: USE OF DATA STANDARDS TO MORE EFFECTIVELY POPULATE FDA SYSTEMS

FDA will work to enhance, recommend, and implement standards that reduce the handling necessary to make data analyzable. FDA will continue to identify opportunities where systems can be adapted and modernized to capture sponsor submitted and internally generated data, and build these solutions into IT planning.

OBJECTIVE 2: IMPROVE DATA QUALITY AND DATA GOVERNANCE

Standardization improves data quality, which is essential to develop large-scale analytics capabilities, including artificial intelligence, automation, forecasting, and other improvements that will increase reviewer efficiency. Data governance is essential to maintaining data integrity, quality, and usability in making regulatory decisions. FDA will continue to evaluate where data quality can be improved, and data governance can be developed or refined. In addition, FDA will work at the Center level along with the Office of Digital Transformation to establish a Data and Technology Modernization Strategy that reflects the vision in FDA's Technology and Data Modernization Action Plans.

3.4 GOAL 4: ENHANCE TRANSPARENCY AND PROMOTE STAKEHOLDER ENGAGEMENT

FDA will enhance transparency and promote stakeholder engagement in its decision-making regarding adoption of new standards, especially required standards.

OBJECTIVE 1: STAKEHOLDER COMMUNICATION AND INVOLVEMENT

Through professional meetings and webinars, as well as FDA-Industry meetings, FDA will communicate and collaborate with stakeholders to explore, develop, and update data standards. FDA will provide industry with clarification on supported and required data

standards and inform on opportunities for engagement, such as testing data standards where appropriate. FDA will update the Agency's resources webpage with current requirements and recommendations on the use of data standards in submissions and solicit stakeholder input on electronic submissions.

OBJECTIVE 2: DATA STANDARDS CATALOG AND ACTION PLAN

On the Agency's resources webpage, FDA will continue to maintain the FDA Data Standards Catalog, and provide a comprehensive list of on-going data standards projects by publishing quarterly updates to the CBER-CDER Data Standards Action Plan.

APPENDIX A: GLOSSARY OF ACRONYMS

BSUFA	Biosimilar User Fee Act
CBER	Center for Biologics Evaluation and Research
CDER	Center for Drug Evaluation and Research
DSAB	FDA Data Standards Advisory Board
DSCC	CBER Data Standards Coordinating Committee
DSDG	CDER Data Standards and Data Governance Board
FDAAA	Food and Drug Administration Amendments Act (2007)
FDASIA	FDA Safety and Innovation Act (2012)
FDA	Food and Drug Administration
GDUFA	Generic Drug User Fee Amendments
HIT	Health Information Technology
OMUFA	Over-The-Counter Monograph Drug User Fee Act
PDUFA	Prescription Drug User Fee Act
SDO	Standards Development Organization

APPENDIX B: DATA STANDARDS REGULATORY FRAMEWORK

- **Final Rule, Requirements for Submission of Labeling for Human Prescription Drugs and Biologics in Electronic Format, 2003**
    - Known as the Electronic Labeling Rule, effective in 2004, the regulation requires the submission of the content of labeling in electronic format for marketing applications. It also states that FDA will periodically issue guidance on how to provide the electronic submission.
- **Food and Drug Administration Amendments Act (FDAAA), 2007**
    - Effective in 2009, FDAAA amended the Federal Food, Drug, and Cosmetic Act (FD&C Act) to require the submission of electronic drug establishment registration and drug listing information in standardized format.
- **Food and Drug Administration Safety and Innovation Act (FDASIA), 2012**
    - FDASIA amended Section 745A(a) of the FD&C Act (21 U.S.C. § 379k–1) to require submissions to be submitted in an electronic format specified by FDA beginning no earlier than 24 months after FDA issues a final guidance specifying an electronic submission format.
    - In 2014, FDA issued the final guidance, Providing Regulatory Submissions in Electronic Format – Submissions Under Section 745A(a) of the Federal Food, Drug, and Cosmetic Act which describes how FDA will implement the requirements to specify the electronic formats in individual content-specific guidances.
    - Individual content-specific guidances have been published since 2014 (e.g., study data), and additional guidances are planned that describe the required standard formats to be used in electronic submissions.
- **Final Rule, Postmarketing Safety Reports for Human Drug and Biological Products; Electronic Submission Requirements, 2014**
    - The rule requires that persons subject to mandatory reporting requirements submit safety reports in an electronic format. It also requires the electronic submission of biological lot distribution reports. The rule went into effect on June 10, 2015.
- **21st Century Cures Act (Cures Act), 2016**
    - The Cures Act provisions are designed to help accelerate medical product development and bring new innovations and advances to patients who need them faster and more efficiently. The Cures Act includes FDA deliverables (Title III) related to clinical outcome assessment, biomarkers and real world evidence.
- **User Fee Commitments**

- User Fee programs support FDA's mission of protecting the public health and accelerating innovation in the industry. Over the course of the user fee reauthorization cycles, FDA has included information technology and electronic data standards commitments as appropriate.