



Center for Drug
Evaluation and Research

Data Standards Program Annual Assessment 2013

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Table of Contents

1	Introduction	1
2	2013 Year in Review	1
	2.1 Policy and Process	2
	2.2 Standards Development and/or Implementation	3
	2.3 Study Data Standards.....	4
	2.4 Research and Development.....	5
3	2014 Summary.....	5

1 Introduction

This is the third annual assessment of the Center for Drug Evaluation and Research's (CDER's) Data Standards program. The [2012 Assessment of CDER's Data Standards Program](#) is available on the CDER Data Standards Program page on the FDA website. This third assessment reflects progress made since the 2012 assessment and is organized to align with the CDER Data Standards Strategy – Action Plan initially published February 2013¹. The Action Plan presents accomplishments, current program status, and course adjustments anticipated based on work done to-date. The focus of this document is to provide stakeholders with an update on CDER's progress towards achieving the goals outlined in the Data Standards Strategy. The [Data Standards Strategy](#) is currently under review and an update is planned for publication in 2014.

2 2013 Year in Review

In its first year, CDER focused on operationalizing its Data Standards Program and assessing standards needs in the Center. In 2012, the CDER Data Standards Program Board refined its strategy and published a communication plan to establish direction and improve communications. Several key projects were begun in 2012, including a broad effort to define and implement terminology standards for clinical studies of distinct therapeutic areas.

The year 2013 saw significant progress in the Center's standards efforts, and expansion of the program's portfolio of standards development initiatives. Several key artifacts were published that provide visibility into the Program's efforts, including the Therapeutic Area (TA) Project Plan, Action Plan and position statements on study data standards and the use of SI units for lab test results. Draft guidance for electronic standardized submissions and components of submissions was developed during 2013 and recently published as draft for comment. Standard operating processes were expanded to support governance and consistency in project processes. Study data standard development projects were commenced for a number of therapeutic areas to define important data elements and relationships for efficacy analysis. And an assessment of standardization needs to support generic drug review was complete

¹ Data Standards Strategy – Action Plan is available from the CDER Data Standards Program webpage <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm249979.htm>

Figure 1 CDER's Data Standards Program Accomplishments and Planned Activities



2.1 Policy and Process

The 2012 Food and Drug Administration Safety and Innovation Act (FDASIA) authorized the electronic submission of information for certain Investigational New Drugs (INDs), New Drug Applications (NDAs), Biologics License Applications (BLAs) and Abbreviated New Drug Applications (ANDAs). CDER continued the development of key guidance and governance documents related to data standards implementation. In 2013, to implement the provisions of FDASIA, CDER and the Center for Biologics Evaluation and Research (CBER) developed and in February, 2014 published, for public comment, two guidances: 1. An interpretative draft guidance on “*Providing Regulatory Submissions in Electronic Format—Submissions Under Section 745A(a) of the Federal Food, Drug, and Cosmetic Act*”, 2 and 2. a revised draft of the guidance on “*Providing Regulatory Submissions In Electronic Format—Standardized Study Data*”. Additional guidances for other submission components are anticipated as the program and projects progress.

The *Data Standards Catalog* format has been revised to provide a listing of supported and/or required standards, their uses, the date FDA will begin (or has begun) to support a particular standard and the date support ends, the date the requirement to use a particular standard will begin and the date such requirement ends, as well as other pertinent information. The *Study Data Technical Conformance Guide* (Conformance Guide) was developed in 2013 and published for public comment in February, 2014. The Conformance Guide provides specifications, recommendations, and general considerations on how to submit standardized study data using FDA-supported data standards located in the **Data Standards Catalog** (Standards Catalog).

Following good practice for standards development and governance, the CDER Data Standards Program Board (DSPB) reviewed and updated its Charter in December 2013 to clarify quorum definition, voting procedures, and to outline member offices. The board, which serves as CDER's executive review and decision-making data standards governance body, oversees the planning and progress of center data standards projects, ensures collaboration and alignment of activities with other relevant groups within centers and across the agency (e.g., CDER Computational Science Center (CSC) Board, FDA Data Standards Council, CBER and the Center for Devices and Radiological Health (CDRH) standards programs).

The DSPB and its Operations Subcommittee drafted revisions to the Study Data Standards Resources web pages for ease-of-use and access to relevant information such as the guidance documents and Conformance Guide, which published in 2014. The website serves as a single location for standards information and deployed when the eStudy Guidance published in the In addition to the public website, CDER revisited overall information sharing needs. To provide process transparency, an effort commenced in 2013 to document CDER's data standard policy as part of a Manual of Policy and Procedures (MAPP). CDER identified this as an important step to clarify and set governance roles and responsibilities to audiences both internal and external to FDA. Process improvement activities began in 2013 to further expand processes outlined in the MAPP and Action Plan as part of the standards development framework. These include development and implementation of a testing methodology for acceptance and readiness testing of standards, and an initiative to evaluate and develop or refine standard operating procedures (SOPs) for operational activities.

2.2 Standards Development and/or Implementation

In October 2013, CDER and CBER published a position statement on the use of Système International (SI) units for lab tests. The centers recognize that SI units are the worldwide standards and are currently evaluating common and therapeutic area-specific lab tests to determine which pose significant interpretation risks during the review of new drug applications, since normal ranges expressed in SI units may differ greatly from those expressed in US conventional units. The [CDER/CBER Position on Use of SI Units for Lab Tests](#) is available in its entirety on the Study Data Standards webpage.

The Center recognized the need for a comprehensive and consistent testing approach to ensure data standards meet FDA's requirements as well as its readiness to receive and utilize data in standardized form. CDER started a *Testing and Acceptance Process for Study Data Standards* project in October 2013, and expects to define and implement a standard procedure that supports the Center in this effort. The project will exercise the process in 2014 on at least two standards.

With the authorization of the Generic Drug User Fee Act (GDUFA), CDER performed an internal assessment of the generic drug review process with an emphasis on data usage and submission quality to determine areas where standardization could add benefit. As a result, a project to improve efficient use of annual reports data was initiated. Additional projects are anticipated in 2014 pending further review of the assessment results. CDER also initiated a review of product quality data to assess consistency and usage to identify areas that if addressed would have a positive impact to the Offices using the data. This project is currently

in the initial phase of assessment and the project team is working with the impacted offices to define the next steps.

Health and Human Services (HHS) recently announced the release of the final rules for Stage 2 of Meaningful Use (MU) and updated certification criteria and standards². FDA does receive and utilize electronic healthcare data for regulatory purposes and the objective is to leverage existing standards and harmonize with healthcare standards where it makes sense. This prompted the initiation of a MU standards (terminologies) assessment project in 2013. Since changes to data standards potentially affect the scientific disciplines conducting reviews of the regulatory information, it is important to assess the impact of any potential changes to standards in use and utilize those results to plan appropriate transitions or document changes that should not be made at the current time. At this time, the project has done a preliminary assessment of two of the MU standards. Early findings indicate that transitions to standards initially thought to be straight-forward will actually require further consideration. The Data Standards Team expects this assessment to provide a practical perspective on next steps.

2.3 Study Data Standards

The Prescription Drug User Fee Act (PDUFA) V Performance Goals indicate that FDA will develop standardized clinical data terminology for distinct therapeutic areas in collaboration with Standard Development Organizations (SDOs). Significant progress was made in 2013 in the planning and development of therapeutic area data standards which focus primarily on requirements for the efficacy review and evaluation of new medical products. The *Therapeutic Area Standards Initiative Project Plan* (Project Plan), published in September 2013, serves as the primary document for guiding all major aspects of FDA's multi-year initiative to develop and implement TA standards to support the regulatory review process for drugs and biologics. A list of the prioritized therapeutic areas and their development status may be found at: <http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm287408.htm>.

CDER expanded its TA requirements project which started with four (4) TAs in late 2012 to a follow-on project in 2013 to address an additional 12 areas. In 2014 FDA will continue these activities and its collaboration with the Coalition for the Advancement of Standards and Therapies (CFAST) to develop therapeutic area data standards for use in regulatory submissions. CDER is committed to the advancement of all data standards for clinical research and regulatory submissions by working with stakeholder groups such as Health Level Seven (HL7), Clinical Data Interchange Standards Consortium (CDISC), and Critical Path Institute, along with CFAST.

The Standard for the Exchange of Nonclinical Data (SEND) Cardiovascular and Respiratory Safety Pharmacology Pilot team successfully executed its proposed plan to receive sample data for review. CDER published center-defined validation rules for SEND formatted files available on the Study Data Standards Resources web page. This pilot will be completed in 2014.

² More information on the Meaningful Use standards can be found here: <http://www.healthit.gov/policy-researchers-implementers/meaningful-use>

2.4 Research and Development

Over the past few years, CDER has increased its support for standardized study data submissions using CDISC standards. Since 2007, FDA has collaborated with CDISC, HL7 and other stakeholders on potential alternatives for exchanging study data.

Building on concepts discussed at the November 2012 [Solutions for Study Data Exchange Standards Meeting](#), CDER and CBER announced a pilot project in 2013 to evaluate the CDISC Extensible Markup Language (XML) transport format for the submission of regulatory study data (Docket Number FDA-2013-N-1424). This project will be conducted in 2014. CDER is also assessing Semantic Web (SW) technology as a modern approach with promising potential to address the challenges in representing and maintaining the evolving information models and standards in use by multiple stakeholders.

CDER completed testing and posted test results for Study Participation and Patient Narrative HL7 standards. This proof-of-concept (POC) effort will be completed in 2014. For additional research and development activities see the CDER Study Data Standards Research and Development webpage at:

<http://www.fda.gov/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/ucm269946.htm> or the research and development information available from the Study Data Standards Resources webpage at:

<http://www.fda.gov/ForIndustry/DataStandards/StudyDataStandards/default.htm>.

3 2014 Summary

As the Data Standards Program moves forward, we will build on the momentum of current projects to further the program's mission. A key focus area for 2014 will include strengthening internal and external communication through regular updates and improved web access to information. The Providing Regulatory Submissions In Electronic Format—Standardized Study Data and Study Data Technical Conformance Guide documents published as draft in 2013 will be updated based on public comments received and begin final reviews in 2014. The Data Standards Program will establish testing procedures that serve to ready the program to accept specific standards as they undergo the testing. The program will expand to include projects relevant to generic drug review and product quality assurance. The definition of SOPs begun in 2013 will be finished and put into action this year. In short, this will be an active and productive year.