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Therapeutic Area (TA) Specific Data Standard Requirements in FDA Filing

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Outlines

- Therapeutic Area Standards Initiative Project Plan
- Data Standards Program Action Plan
- FDA specific data standards requirements for HCV studies
- What Can We Do Next to FDA TA Data Standards Program

Therapeutic Area Standards Initiative Project Plan

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Electronic Submissions to CDER

- CDER Data Standards Program
- Electronic Common Technical Document (eCTD)
- Electronic Regulatory Submissions and Review Helpful Links
- Electronic Submissions Presentations
- Study Data Standards for Submission to CDER
- Source Data Capture from Electronic Health Records (EHRs)
- Data Standards Manual (monographs)

Priority Therapeutic Areas for Development

Standardizing key study data specific to therapeutic areas (TAs) will facilitate clinical research and the evaluation of medical products. In 2011, CDER identified a set of therapeutic areas that could benefit from further standardization, organizing them into three tiers of priority. Several factors were considered at the time: (1) areas of particular medical need, (2) areas with existing data standardization projects underway, and (3) areas with greater drug development pipeline activity. The number and ordering of these areas may change as stakeholder input and further analyses are considered.

The roadmap below displays the TAs in priority groupings and suggests a series of standardization projects over time to achieve significant results by December 2017. This roadmap assumes standardization projects for therapeutic areas are scoped narrowly enough to be accomplishable within a 12-month period, and that subsequent projects in those areas build on the results. Periodically, updates to the timeline will be provided as progress, opportunity and additional information is available. The tabular version, also listed below, provides the current status information for all identified TAs.

FDA, CDISC, and the Critical Path Institute are collaborating on efforts to support development of therapeutic area standards. FDA is also collaborating with HL7's Clinical Interoperability Council and other consortia to define related clinical concepts. We encourage stakeholders to engage in and, where possible, support these data standardization efforts.

Questions and comments can be forwarded to CDERDataStandards@fda.hhs.gov.

- **Roadmap for Development of Priority Therapeutic Area Standards** (PDF -89KB) (updated 4/22/2016)
- **Table of Priority Therapeutic Area Standards** (PDF - 420KB) (updated 4/22/2016)
- **Therapeutic Area Project Plan** (PDF -296KB)
  FDA announces the availability of the Project Plan for Developing Data Standards for Therapeutic Areas. Under Section XII of the PDUFA V performance goals, FDA agreed to create a plan for developing distinct data standards for therapeutic areas using a public process that allows for stakeholder input through open standards development organizations. These data standards should enable and enhance the ability to integrate, analyze, and report regulatory information about therapeutic areas.

Therapeutic Area Standards Initiative Project Plan

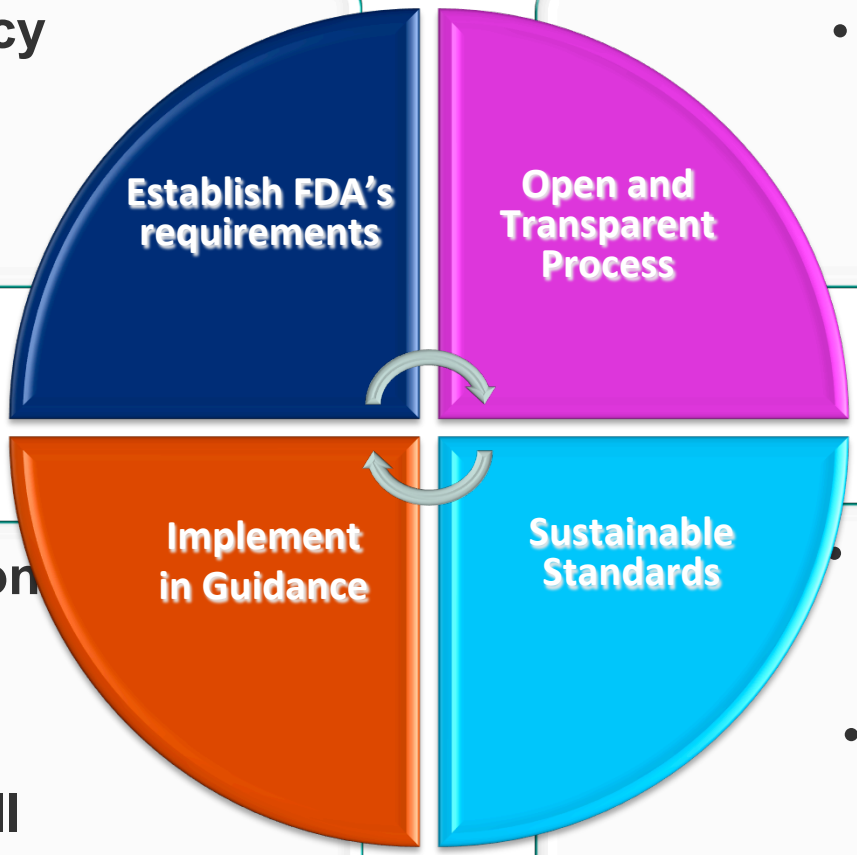
Therapeutic Area Standards Initiative
Project Plan

Version: 3.0
Document Date: October, 2015

http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/UCM371691.pdf

FDA TA Standards Development Objectives

- Identify efficacy endpoints
- Manageable increments

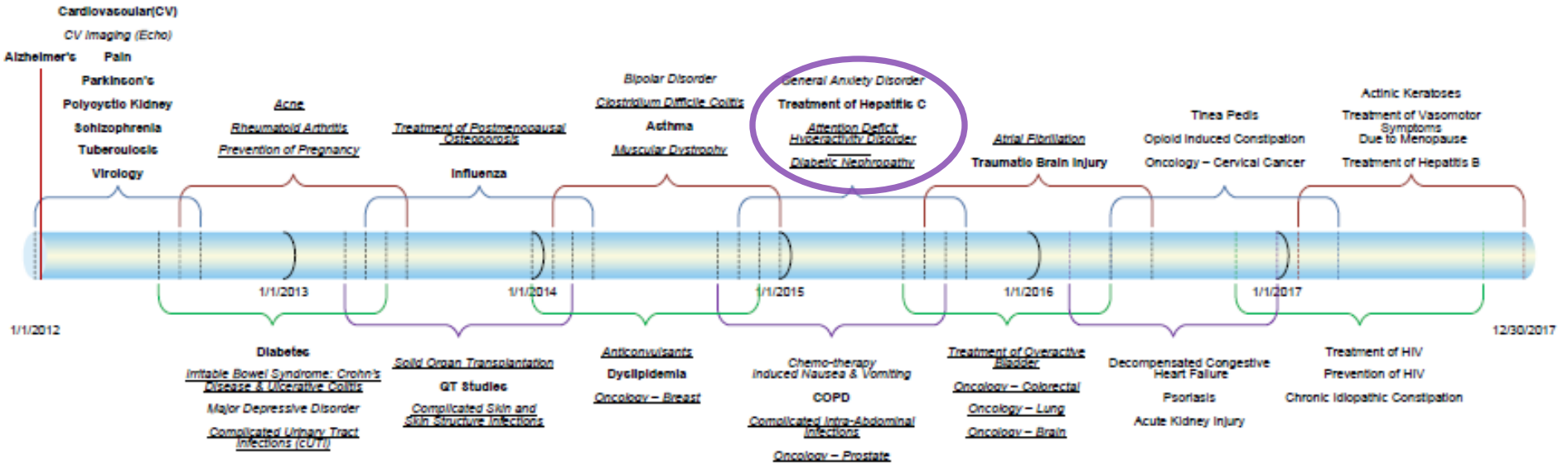


- Collaborate with SDOs & TA Experts
- Ensure Public Comment

- Implementation Guidance
- Standardized Study Data will become Binding

- Adopt or adapt existing TA data elements
- Promote interoperable and sustainable submission environment.

Therapeutic Area Data Standards Roadmap

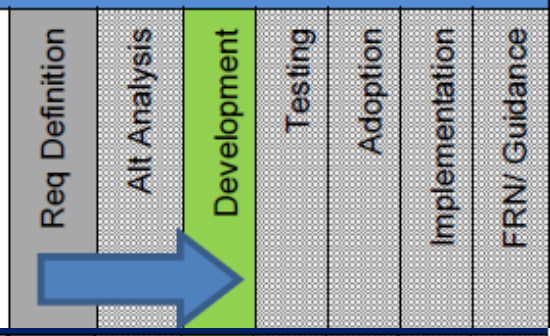
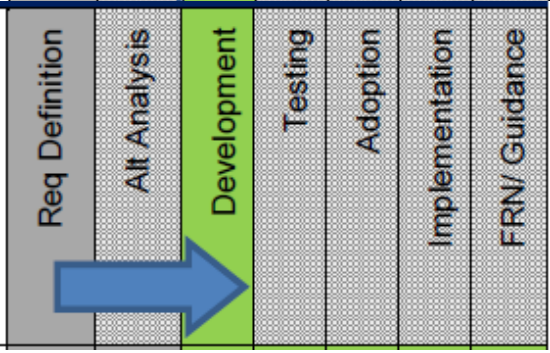


"The term "therapeutic area" also includes diagnostic and preventive areas. Some areas may represent a disease/domain area.

Page 1

Legend:
Project commenced
FDA requirements definition
TA User Guide Published

Data Standards Program Action Plan

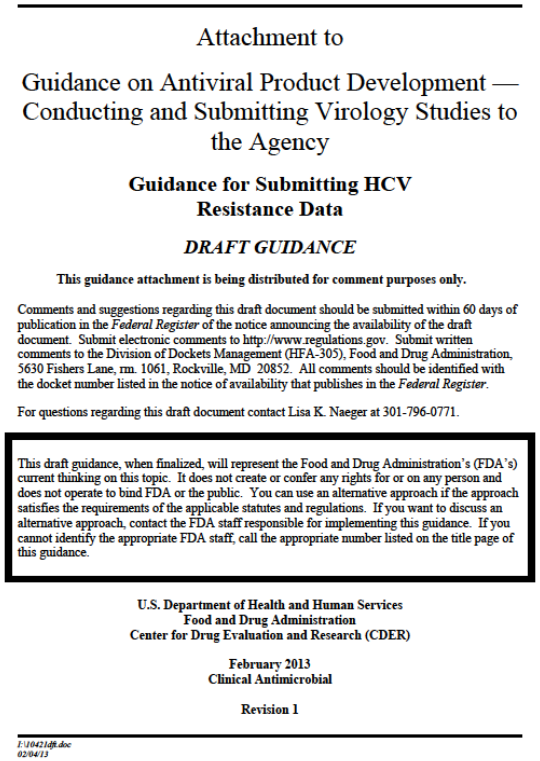
Project Title and Description	Project Update	Project Stage*
Projects that will impact the data received/reviewed during the pre-market review of submissions		
FDA Therapeutic Areas[1] (TAs) Data Standards Efficacy Requirements Capture CDER data requirements for review of clinical efficacy for the priority TAs.	Project completion estimated by end of FY 2016.	
FDA Therapeutic Areas[2] Data Standards Analysis Requirements Develop the approach for standardizing analysis data sets.	Project completion estimated by the end of FY2016.	

FDA Specific Data Standards Requirements for HCV Studies

- Efficacy Outcomes and Related Covariates (ADEFFOUT)
  - Efficacy outcomes and related covariates dataset shall have one record only per subject and need to include at least following information
    - Demographic variables
    - Baseline characteristics (including Baseline Genotypic Data, stratification factors, etc.)
    - Exposure variables (first and last dosing date, etc.)
    - Population flags (ITT, PP, etc.)
    - Efficacy outcomes (primary, secondary, etc.)
    - Covariates and subgroup variables
    - Subject disposition variables
- Raw HCV viral load dataset

FDA Specific Data Standards Requirements for HCV Studies

- Guidance for Submitting HCV Resistance Data



Attachment to

Guidance on Antiviral Product Development — Conducting and Submitting Virology Studies to the Agency

Guidance for Submitting HCV Resistance Data

DRAFT GUIDANCE

This guidance attachment is being distributed for comment purposes only.

Comments and suggestions regarding this draft document should be submitted within 60 days of publication in the *Federal Register* of the notice announcing the availability of the draft document. Submit electronic comments to http://www.regulations.gov. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852. All comments should be identified with the docket number listed in the notice of availability that publishes in the *Federal Register*.

For questions regarding this draft document contact Lisa K. Naeger at 301-796-0771.

This draft guidance, when finalized, will represent the Food and Drug Administration's (FDA's) current thinking on this topic. It does not create or confer any rights for or on any person and does not operate to bind FDA or the public. You can use an alternative approach if the approach satisfies the requirements of the applicable statutes and regulations. If you want to discuss an alternative approach, contact the FDA staff responsible for implementing this guidance. If you cannot identify the appropriate FDA staff, call the appropriate number listed on the title page of this guidance.

U.S. Department of Health and Human Services
Food and Drug Administration
Center for Drug Evaluation and Research (CDER)

February 2013
Clinical Antimicrobial

Revision 1

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02/04/13

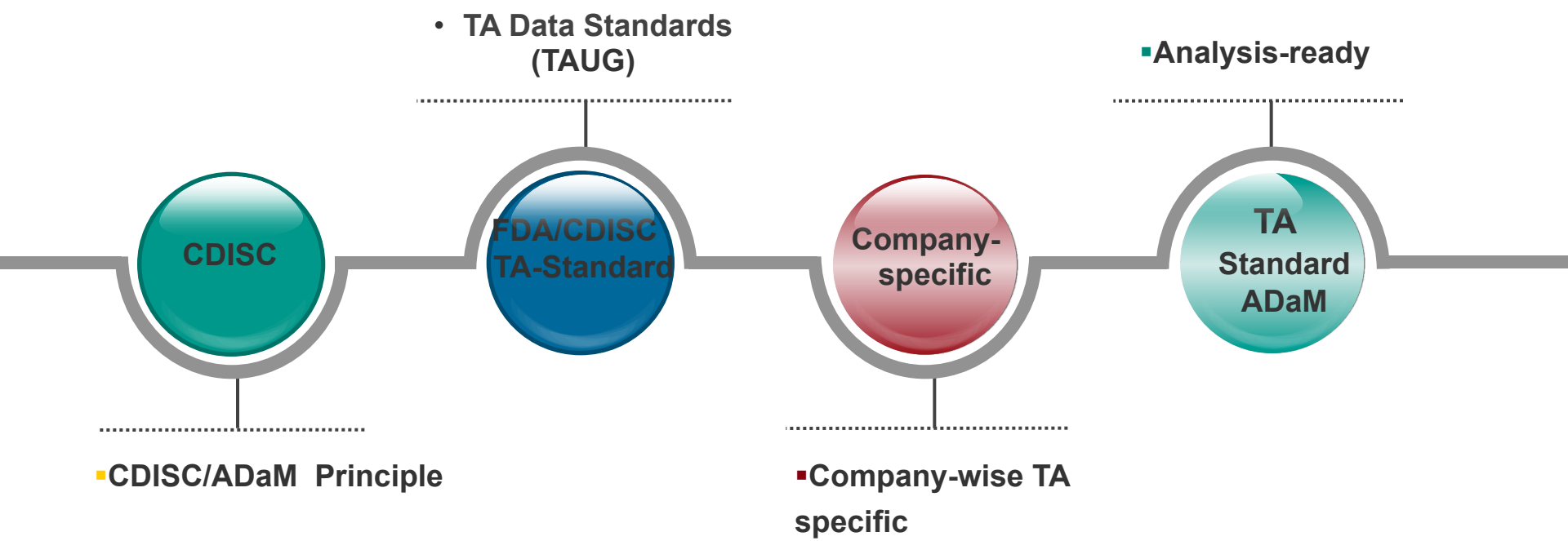
What Can We Do Next to FDA TA Data Standards Program?

Design and Construct TA Standard ADaM Based on FDA TAS Requirements

What Is Therapeutic Area Data Standards

Standardized data elements, terminologies, and data structures enable automation of important analyses of clinical study data to support more efficient and effective regulatory decision-making

How to Build TA Data Standards



- TA Data Standards (TAUG)

- Analysis-ready

- CDISC/ADaM Principle

- Company-wise TA specific

References

- Providing Regulatory Submissions in Electronic Format —Certain Human Pharmaceutical Product Applications and Related Submissions Using the eCTD Specifications (May 2015);

- Providing Regulatory Submissions In Electronic Format — Standardized Study Data http://www.fda.gov/downloads/Drugs/.../Guidances/UCM292334.pdf

- Study Data Technical Conformance Guide (Technical Specifications Document) Oct 2015
- http://www.fda.gov/downloads/ForIndustry/DataStandards/StudyDataStandards/UCM384744.pdf

- Therapeutic Area Standards Initiative Project Plan

  http://www.fda.gov/downloads/Drugs/DevelopmentApprovalProcess/FormsSubmissionRequirements/ElectronicSubmissions/UCM371691.pdf

Questions?

Thank You