

Abstract

PhUSE SDE

Industry Experiences Submitting Standardized Study Data to Regulatory Authorities

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FDA and PMDA require standardized study data for certain regulatory submissions. Industry approaches to meeting these requirements vary across companies and between regulatory review divisions. Within the PhUSE Optimizing the Use of Data Standards, the working group Industry Experiences Submitting Standardized Study Data to Regulatory Authorities established a project to recommend a best submission strategy and planning practices. This paper focuses on the communication methods within and between sponsors and regulatory authorities that can facilitate an effective exchange of the standardized data and the study related information.

Industry Experience in Submitting Standardized Study Data to Regulatory Authorities

PhUSE Single Day Event (SDE)

North Wales, PA

September 26, 2019



Agenda

01

**PhUSE Working
Group**

02

**Submission
Strategy &
Planning**

03

**Communication
Strategy**

04

**FDA and PMDA
Distinctions**

01 - PhUSE Working Group

PhUSE



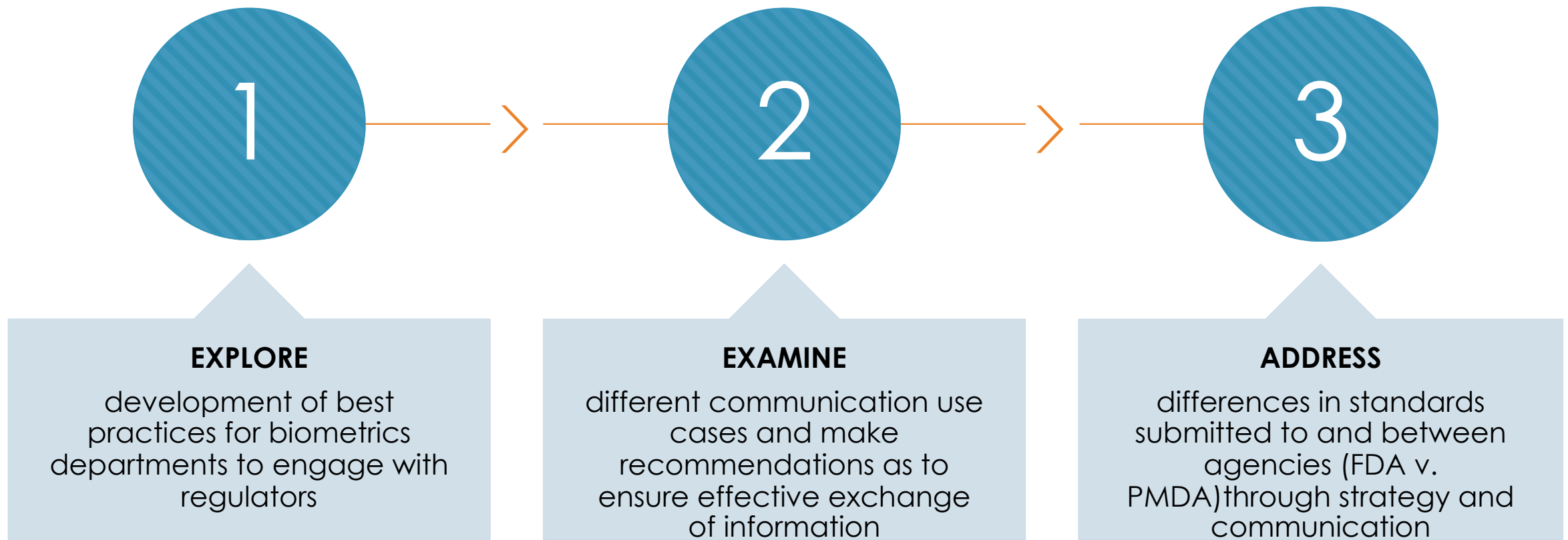
**Optimizing the Use of Data
Standards Working Group**



Industry Experience
Submitting Standardized Study Data
to Regulatory Authorities
(sub team)

- Investigate and recommend best strategy and planning practices for preparing data for a regulatory submission
- Suggest communication methods within and between sponsors and regulators that can facilitate these best practices

~60 global contributors from industry and FDA representing in areas of Statistical Programming, Statistics, Submission and Process



Project Objectives

02 - Submission Strategy & Planning Best Practices

Submission Strategy & Planning

Submitting standardized study data to regulatory agencies is a critical and complex process

Requires organized activities and discussions with cross functional teams

Steps

- Analyze your current state
- Identify what is important and a requirement
- Define what you need for future state implementation and who is responsible
- Review internally before sharing externally

Study Data Standardization Plan (SDSP)

Collection of study information and the supported data standards

Collection of non-clinical and clinical studies at the start of the IND

Retrospective implementation

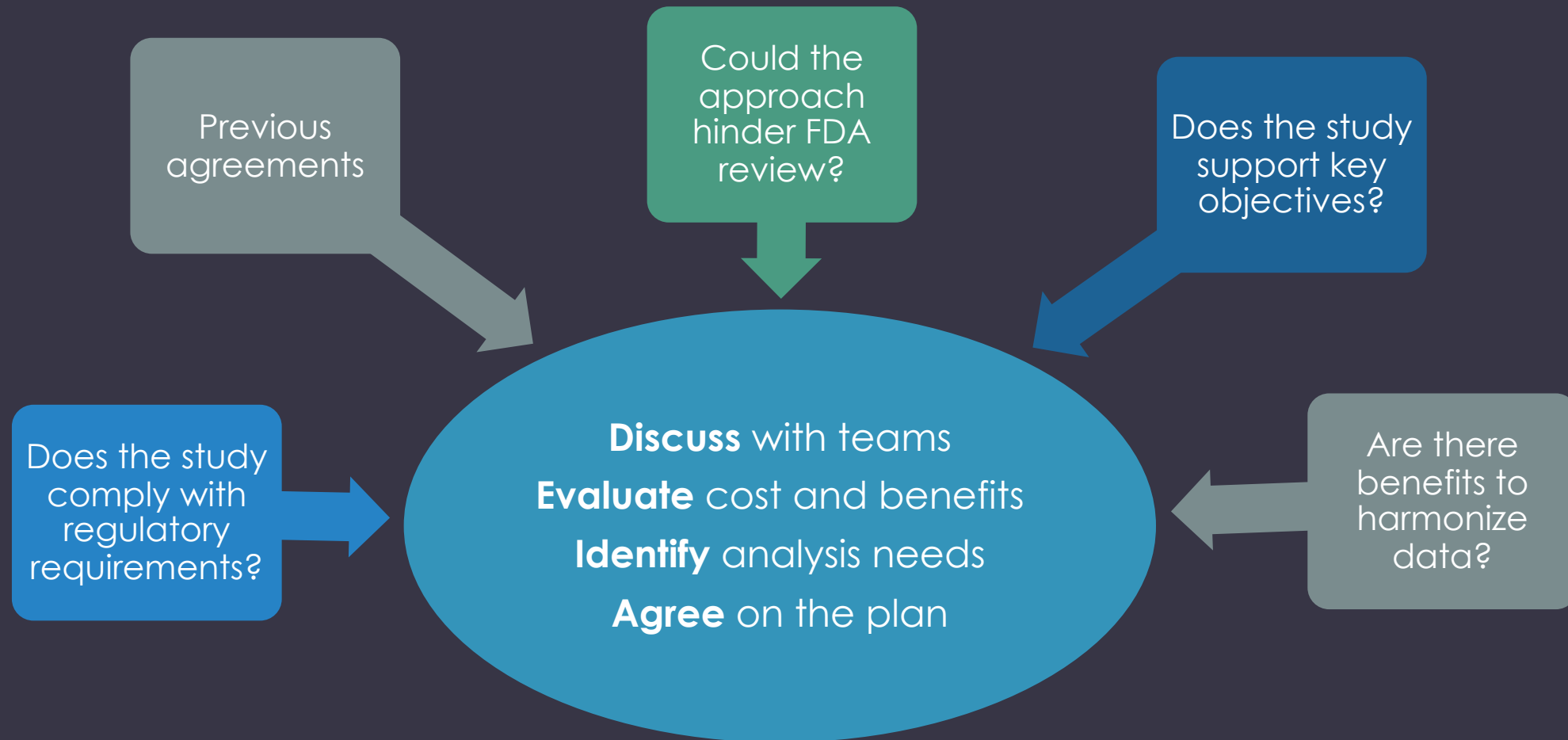
SDSP is a tool to develop your strategy and planning

- Review studies for compliance against the study data requirements
- Assess importance studies have in relation to the pooled analysis

Legacy Conversion & Up-Versioning Plan

- **Study Start Date BEFORE** Dec 17, 2016 for NDAs, BLAs, ANDAs can submit in legacy format. Conversion is optional.
- **Study Start Date AFTER** Dec 17, 2016 for NDAs, BLAs, ANDAs. Conversion of legacy data to comply with data standards is required unless a waiver is granted.
- Retired standard that is no longer supported in the FDA Data Standard Catalog is expected to be up-versioned unless a waiver is granted.
- PMDA study data requirements are dictated by the **submission date AFTER March 2020**

Factors to Developing Legacy Data Conversion Strategy and Planning



03 - Communication Methods Practices

Communication Strategy

Internal Communication

- Use the SDSP as a tool to help identify any gaps and facilitate team discussions
- Discuss, share, review future state plans and stakeholder involvement

External Communication

- Identify trials and concerns that require reviewer's agreement on the use of data standards to support the submission
- Gain concurrence on the plan at stage gate meetings, waivers, and written correspondences

04 – FDA and PMDA Distinctions

FDA vs. PMDA Study Data Submission

- In the end to end process of drug development best strategy and planning practices, it is essential to be aware of the current requirements of the regulatory authorities to plan, develop and submit the study data submission deliverables.
- The white paper also shares FDA and PMDA requirements to address differences in standards between agencies as both of these agencies require submission of study data. Additionally, includes questions to help industry plan for submitting to both agencies based on the industry experiences and feedback.

FDA vs. PMDA Study Data Submission

- Comprehensive comparison of recommendations and requirements of study data submission between FDA and PMDA.
- Industry experiences in submitting to both agencies

FDA and PMDA Distinctions

- Mandates across FDA and PMDA
 - Electronic Submission timelines and data standards dates
 - Waiver possibilities
- Drug development lifecycle across FDA and PMDA
 - Flow diagrams and Timelines
 - Types of meetings
- Regulatory Documents; Catalogs; eCTD folders
- Study data planning (SDSP/Appendix 8)
- Datasets packages (tabulations, analysis, ISS/ISE, OSI)
 - Reviewers Guide differences between PMDA and FDA
 - Analysis Results Metadata (ARM)
 - Validation rules, variations and resolution
 - Dictionaries; Controlled Terminology
- Software programs; accepted file formats
- Dataset and variable requirements
- Use of System International (SI) Units versus Conventional Units

Industry Experiences in Submitting to FDA and PMDA

- Can we create one single eSub Dataset Package and submit to both the FDA and the PMDA, either at the same time or later?
- Can we create one single CDISC eSub Dataset Packages that is acceptable to all HA agencies?
- What would you recommend if a study is filing to the FDA and PMDA?
- What if I do not know if we are submitting to the PMDA?

Summary

Submitting standardized study data to regulatory agencies is a critical and complex process and it requires **strategy**, **planning** and **communication**.

Steps:

- Analyze your current state
- Identify what is important and requirements from FDA and PMDA
- Define future state and stakeholder involvement
- Review the strategy and plan internally before sharing and communicating externally

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