

Introduction of the Guidance

This guidance outlines **technical specifications** for submitting patient-reported outcome (**PRO**) data collected in cancer clinical trials to **support marketing applications**. This includes information on:

- SDTM and ADaM
- Tables and figures
- Missing data representation
 - in SDTM and in ADaM
 - pertaining to PRO data usage, (i.e., assessing clinical benefit vs. safety and tolerability)
 - in the context of intercurrent events

New for SDTM

- QS domain: include records for missing data per planned assessment
- TS domain: add a parameter
TSPARAMCD = ‘FDATCHSP’, TSPARAM = ‘FDA Tech Spec’
TSVAL = ‘Oncology PROs Technical Specifications Guidance v1.0’

Highlights for ADaM

- Include records for **all planned visits** in the PRO assessment period, with both individual item scores and summary scores
- Missing data representation:
 - Create phantom records for missed visits, item and summary scores with DTYPE=“PHANTOM”, update DTYPE with missing data imputation method
 - Populate AREASND (Analysis reason not performed) with QS.QSREASND or other source data
- Introduce new flag variables such as
ONTRTFL and ONTRxxFL for on treatment record flag
PROEXPFL for PRO expected flag
PROSCMFL for PRO score completed flag
- Incorporate variables about intercurrent events such as
FPDDT (first progressive disease date)

1st Time! Additional Tables & Figures

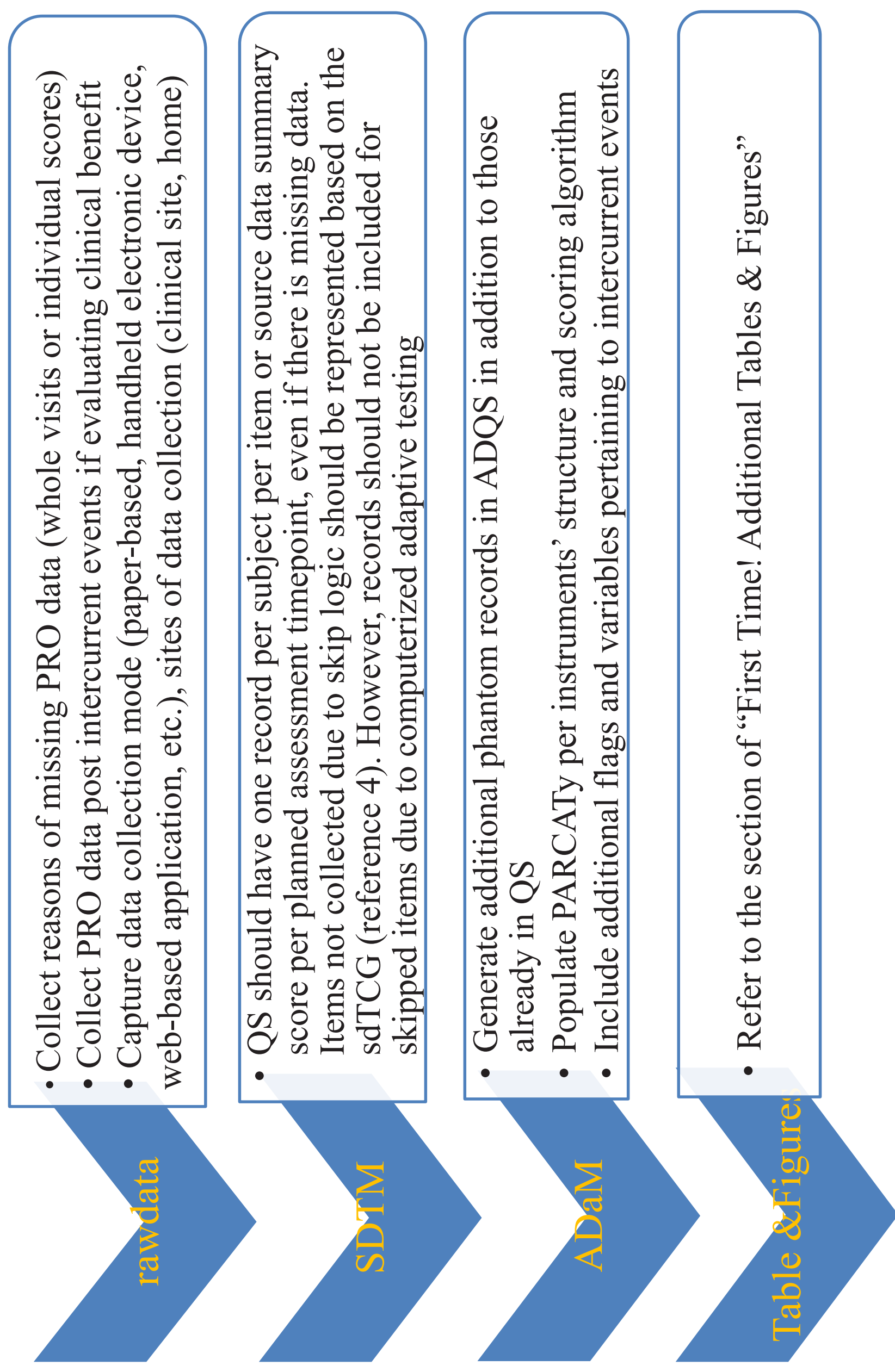
Include additional tables and figures in Clinical Study Report (CSR)

- Cumulative patient disposition of PRO data
- PRO data completeness
- Distribution of responses and change in responses from baseline
- Incidence of healthcare utilization

Provide an executive summary of clinical interpretation of the most critical PRO results informing risk(s) and benefit(s) within CSR

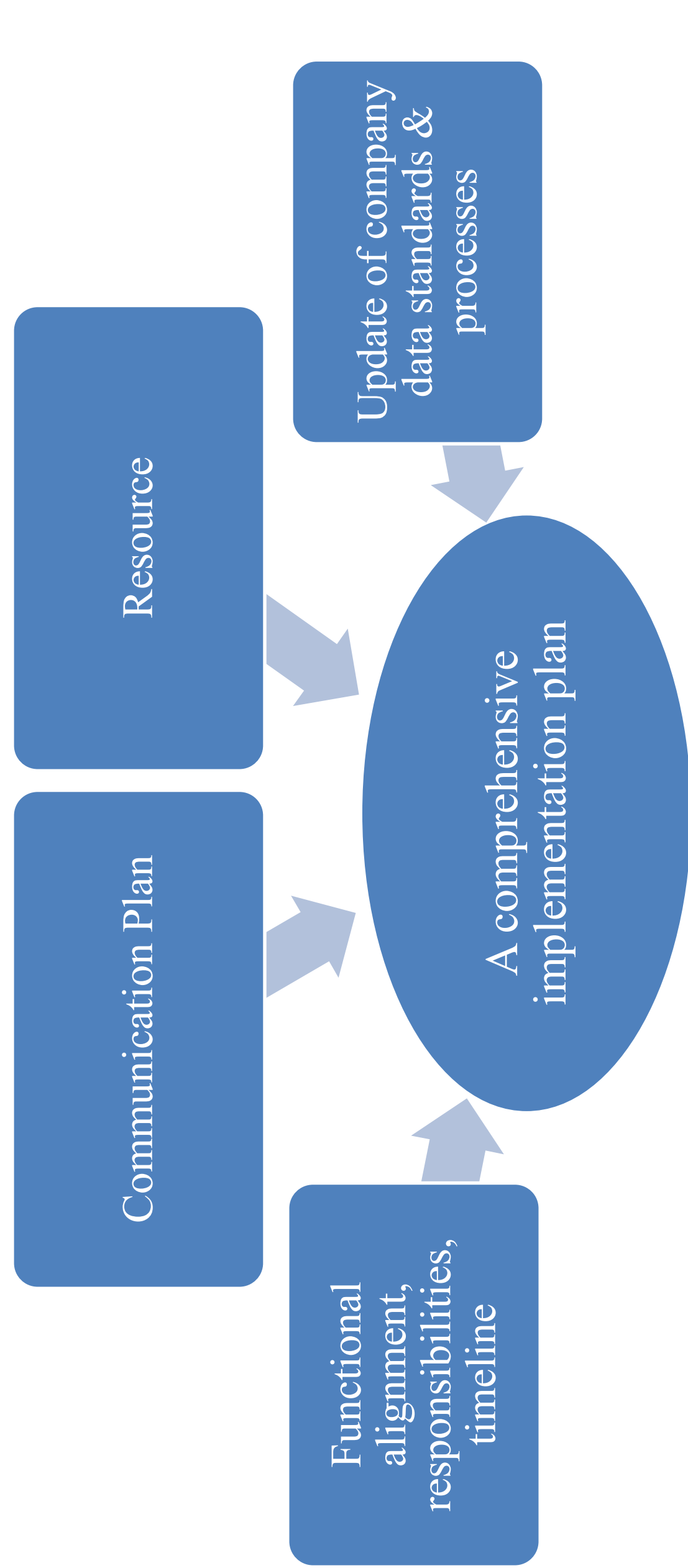
Impact Assessment

- Internal stakeholder identification, i.e., impacted functions
- Identification of specific groups/processes potentially affected
- Evaluation of current state
- Specification of extent of changes required



Implementation Strategies

- Establish a cross-functional team
- Create a comprehensive implementation plan



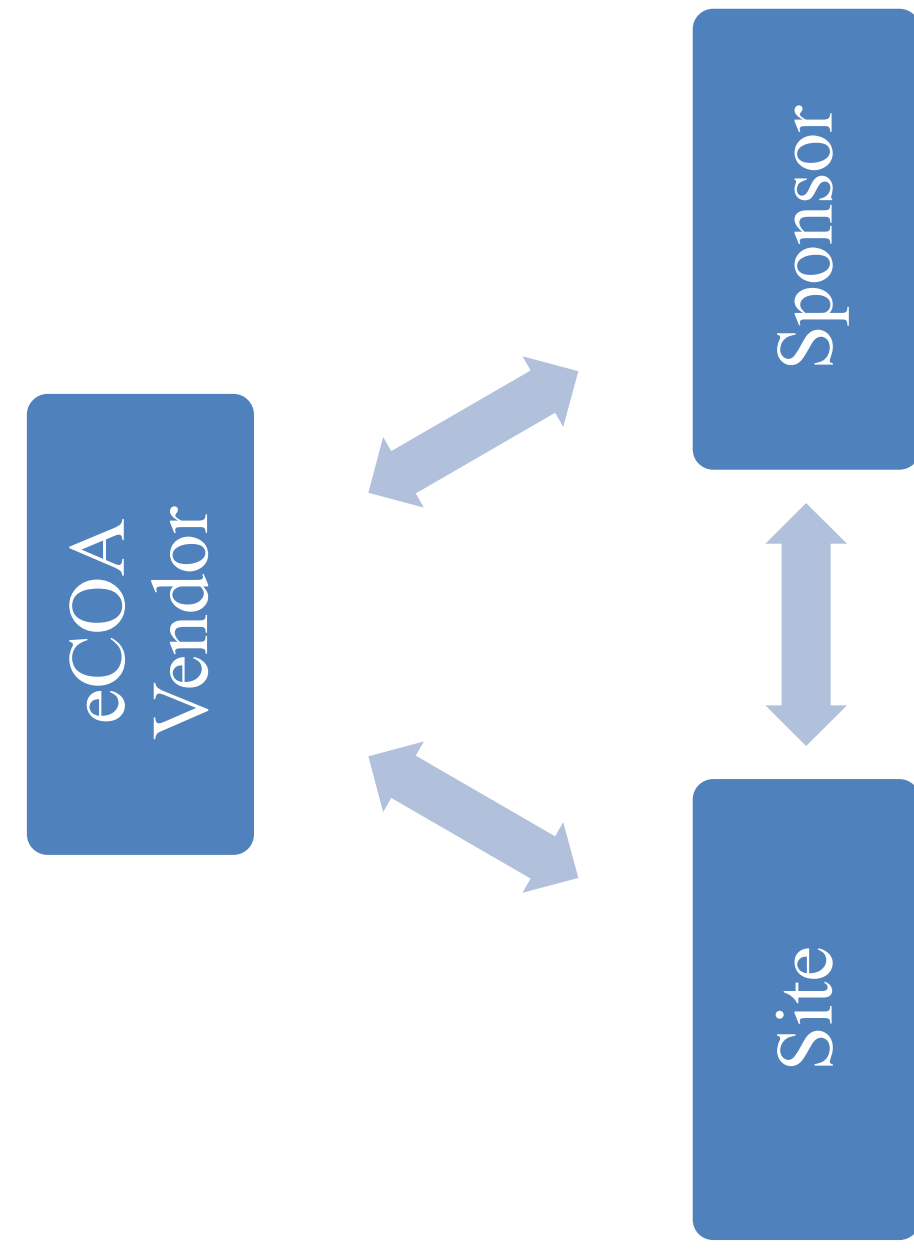
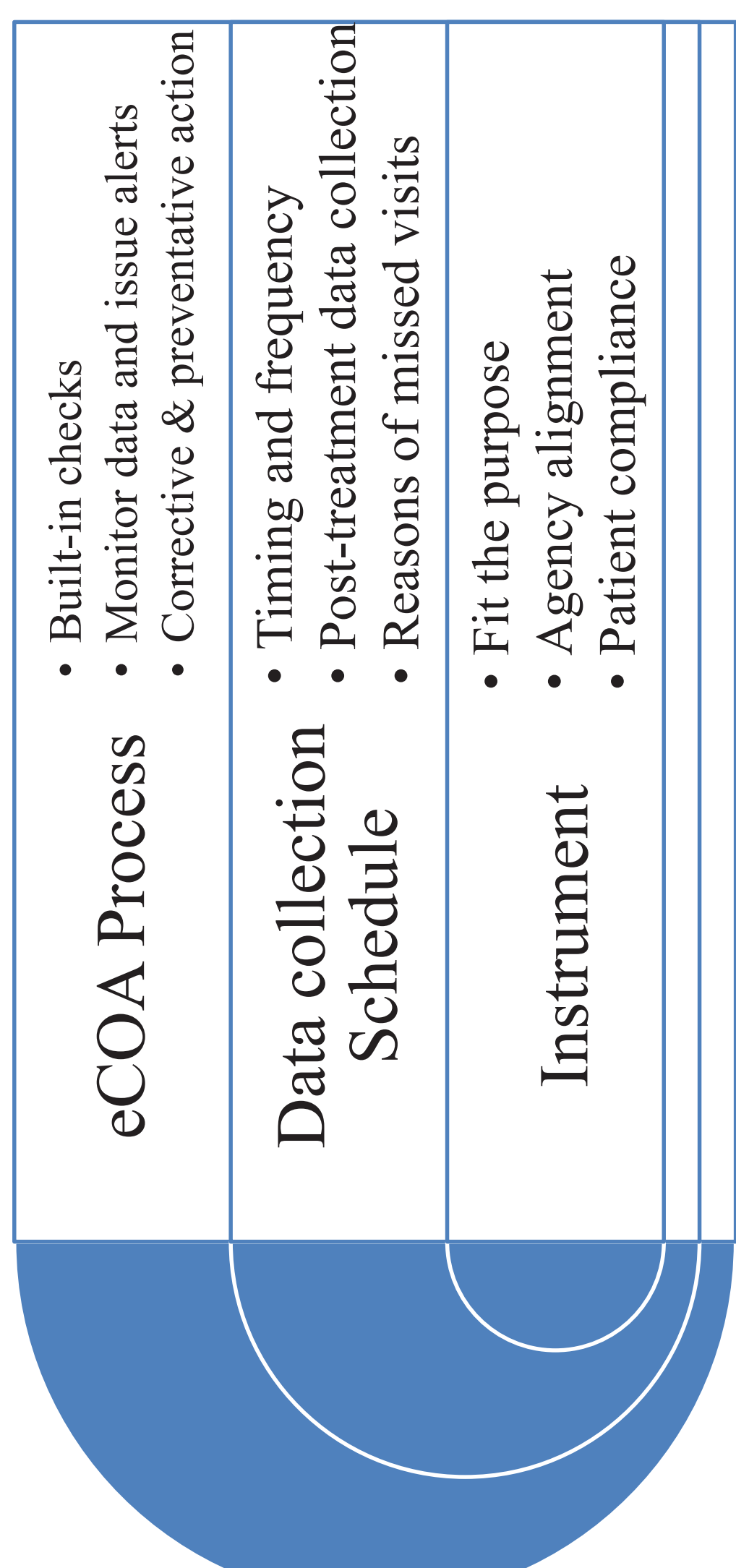
Breaking the Barriers of Implementation

- PRO data resides in a stand-alone database
- SDTM and ADaM is created by different parties
- PRO data analysis is not always pre-specified clearly in the protocol or statistical analysis plan
- Historically PRO data rarely made to the label

Beyond the Guidance

Ultimate goals:

- Making the most out of collected PRO data
- Minimizing missing data from root causes



PHUSE CSS Spirit (Connect, share and advance):

- eCOA vendor selection with predefined requirements
- Consistency check between eCOA and clinical database
- Early access of data and assessment of data quality, take needed actions

Feedback to the FDA

- Clarifications on the guidance (e.g., those may not be consistent with CDISC implementation guide)
- Suggestions for modifications to the guidance

References

- [Submitting Patient-Reported Outcome Data in Cancer Clinical Trials, November 2023](#)
- [Core Patient-Reported Outcomes in Cancer Clinical Trials, Draft Guidance for Industry, June 2021](#)
- [Patient-Focused Drug Development: Collecting Comprehensive and Representative Input, June 2018](#)
- [Submitting Clinical Trial Datasets and Documentation for Clinical Outcome Assessments Using Item Response Theory, November 2023](#)