Fit-for-Purpose RWE is poised for regulatory decision given emerging guidances with need for continued learning

What can sponsors do?

Plan early for RWE use, pre-specify and meet with FDA

- Justify study design and RWD source choice: Retrospective/Prospective RWD and with/without intentional data capture
- Submit protocols and statistical analysis plans before conducting a study
- Meet with FDA to discuss protocol using RWD in the context of the clinical development plan
- Work with Data companies to define fit-for-purpose data for a protocol before and after meeting with FDA

What can Data vendor/analytics collaborators do?

Continuously improve data quality and transparency

- Increase transparency through robust Data Management & Monitoring Plans
- Create regulator-facing documentation with comprehensive protocol/SAP development that defines Fit-for-Purpose data
- Provide RWD that are traceable to patient-level source data and auditable

Areas for continued learning

Deepen understanding of validation and verification

- Determining what variables of interest require validation and to what extent
- Defining reference standards for critical real-world variable
- Defining the validation and verification needed for real-world endpoints that differ from clinical trial endpoints
- Advancing analytic methodologies to characterize (beyond sensitivity analyses) and correct for bias when RWE is substantial evidence

Global harmonization for defining Fit-for-Purpose RWE

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