



THE FORUM
For Collaborative ResearchSM
Berkeley's Hub for Regulatory Science

Data Overview

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**No one data source is perfect
Seek complementarity**

Data Sources

- Randomized clinical trials
 - Controlled/blinded
 - Open-label studies
 - Single-arm studies
- Observational studies
 - Registries
 - Cohorts
 - Retrospective/prospective
- RWD
 - Electronic health records
 - Claims data

STROBE Criteria for Obs Studies



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- Objectives
- Pre-specified hypothesis
- Study design
- Eligibility criteria
 - Methods for selection criteria
 - Methods for follow-up
- Clearly define outcome
 - Exposures, predictors, potential confounders, effect modifiers
- Sources of bias
- Study size
- Statistical methods
 - Confounding
 - Subgroups/interactions
 - Missing data
 - Loss to follow-up
 - Sensitivity analyses

Regulatory Guidance for RWD

Populations

- High prevalence
- Low prevalence
- Phenotypic/genotypic diversity
- Co-morbidities
- etc



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For MASH RLSE



- Phase 3 trials → → clinical outcome
 - Available “now” – REGENERATE
 - Available “in future” – MAESTRO; ESSENCE
- Long-term cohort studies
 - MASH CRN
 - LITMUS (+)
 - International collaborations

Liver Forum MASH PDB



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- Natural disease progression in untreated patients
- Biomarker - variation over time
- “Placebo response” predictors

Some Considerations

- RCT+RWD
- Statistical methods to address data “issues”
 - E.g. Missingness
 - Uncertainty
- Interpreting/learning from differences among cohorts