Berkeley School of Public Health

FORUM for Collaborative RESEARCH

Session V: New Working Group Discussion

Placebo Arm Database

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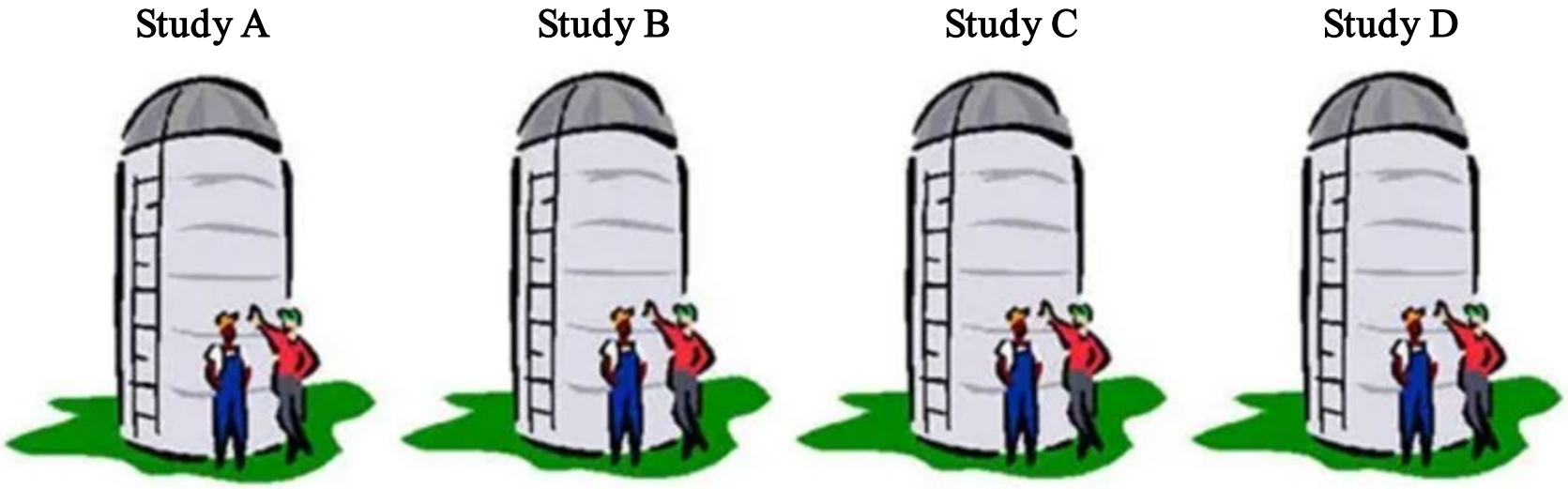
Mission of Placebo Arm Database

- Open dialog b/w all stakeholders and FDA/EMA
- Facilitate planning /powering of future trials
- Define natural history and/or placebo-response rates
- Decrease number of patients randomized to placebo vs active drug
- Develop better tools to stratify patient populations, assess treatment benefits and/or clinically meaningful outcomes
- Reduce time, size and cost of phase 3 trials through optimization

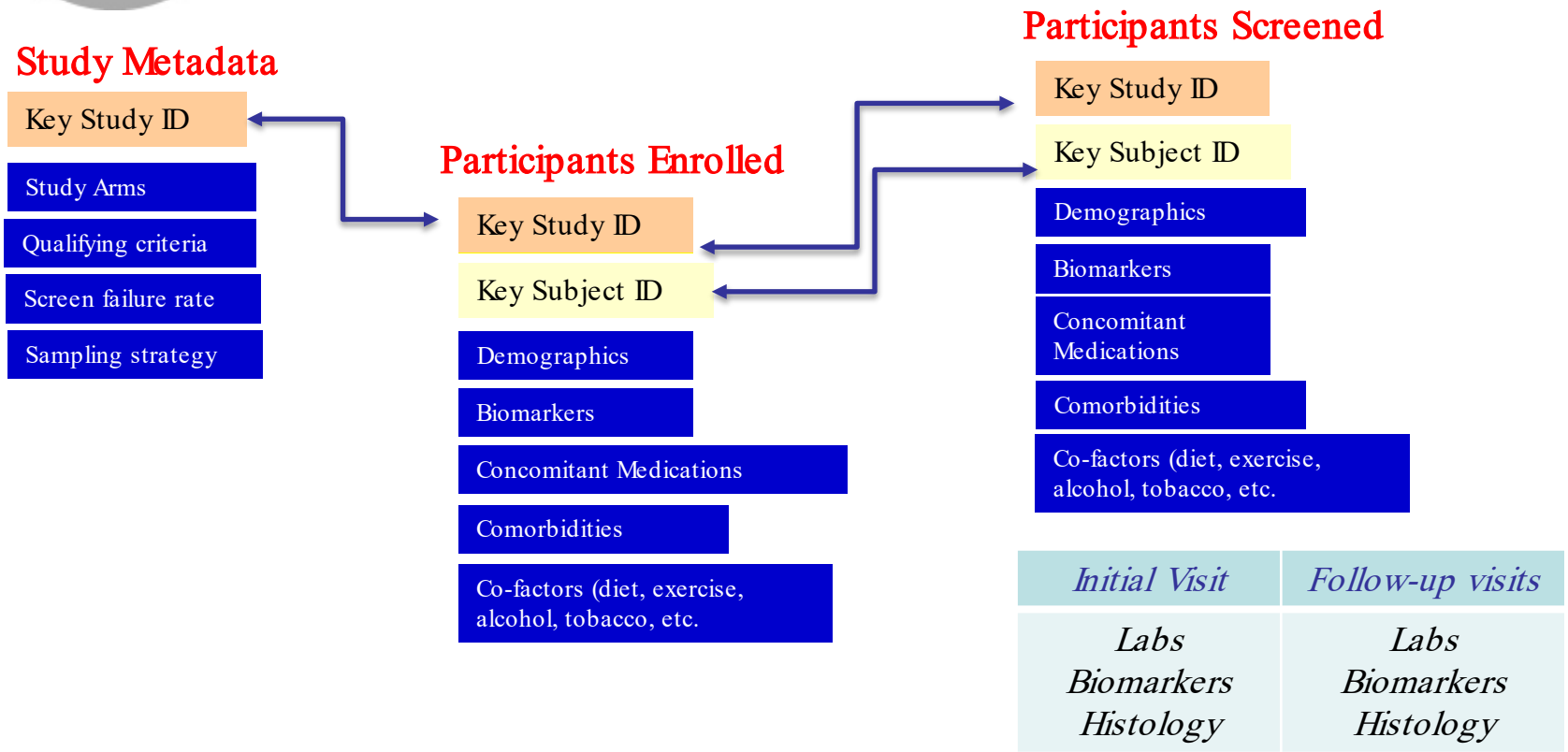
Liver Forum Placebo Arm DB Project

- Integrated patient-level DB from completed phase 2/3 NASH studies
- Natural history cohort representing patients eligible for RCT's
- Natural history across the spectrum of NASH stages in screened / enrolled in clinical trials
- Identify predictors of "placebo response" vs. disease progression
- Reduce placebo-arm burden in future phase 3 (phase 4) trials
- Increase specificity of patient selection
- Collaborative database serving the Liver Forum community, sponsors and regulators

Silos of Data



Integrated Construct—Placebo Arm DB



Highest Level of Security

Data will be housed in a secure research data and compute (SRDC) platform developed at Berkeley for researchers working with highly sensitive (P4 level) data.

PROTECTION LEVEL 4
• Protected Health Information (PHI) / patient records
• Research information classified as Protection Level 4 (P4) by an IRB or otherwise required to be stored or processed in a high-security environment.
• Sensitive Identifiable Human Subject Research data - including certain types of individually identifiable genetic information

- Direct access for regulatory agencies
- LF community (PDB WG) --Propose analysis to benefit field

Framework for Arm DB Project

PDB Working Group

LF members experts from all stakeholder groups

PDB Oversight/Advisory Committee

Key expert representative from each stakeholder group:

- Academic, industry, regulatory, patient
- Guide/advise overall process
- Review analysis proposals
- Guide publication policy and process

Discussion

Thoughts...

Questions...

or Ideas…