Data Collection on Patients: Determining Purpose, Value, Risks and Burdens

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Disclaimer

The views expressed in this talk are my own. They do not represent the position or policy of the NIH, DHHS, or US government.

Overview

- Ethical Considerations
  - Clinical Care
  - Research
  - Quality Improvement/Assessment

Clinical Care

- Goal of clinical care is to act in the best interest of the patient

- Diagnosis of ASD
- Plan of Care
  - Evidence based interventions
  - Assessments to plot progress specific to individual patient
    - Range of abilities across diagnosis of ASD

Clinical Care

- Using clinical care data for research purposes
  - Chart abstraction of relevant variables
    - Comparison within and across cases
- Approach would be to:
  - Seek approval from an Institutional Review Board (IRB) to conduct research with data collected already collected for clinical purposes (retrospective)
    - Assuming data is not publicly available (otherwise Exempt from IRB review)
    - **Seek a waiver of informed consent**
  - Seek approval from an IRB to conduct research with data to be collected for clinical purposes (prospective)
    - Obtain consent/parental permission for use of data for research purposes

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Research

- Goal of research is to protect the welfare of the participant
  - Participant may or many not benefit directly
  - Future patients benefit regardless of outcome

Ethical Framework for Biomedical Research

- Collaborative Partnership
- Social Value
- Scientific Validity
- Fair Participant Selection
- Favorable Risk-benefit Ratio
- Independent Review
- Informed Consent
- Ongoing Respect for Participants

Source: Emanuel et al (2008)

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Social Value

- Social value requires that the research is beneficial to the participants, community, and research community of health system without wasting resources
  - Who will benefit from the conduct and results of research?
  - What is the potential value of the research for each of the prospective beneficiaries?
  - How will the social value of the research be enhanced?
  - How can adverse impacts, if any, of conducting the research be minimized?

Sources: Emanuel et al (2008); Tsoka-Gwegweni and Wassenar (2014)

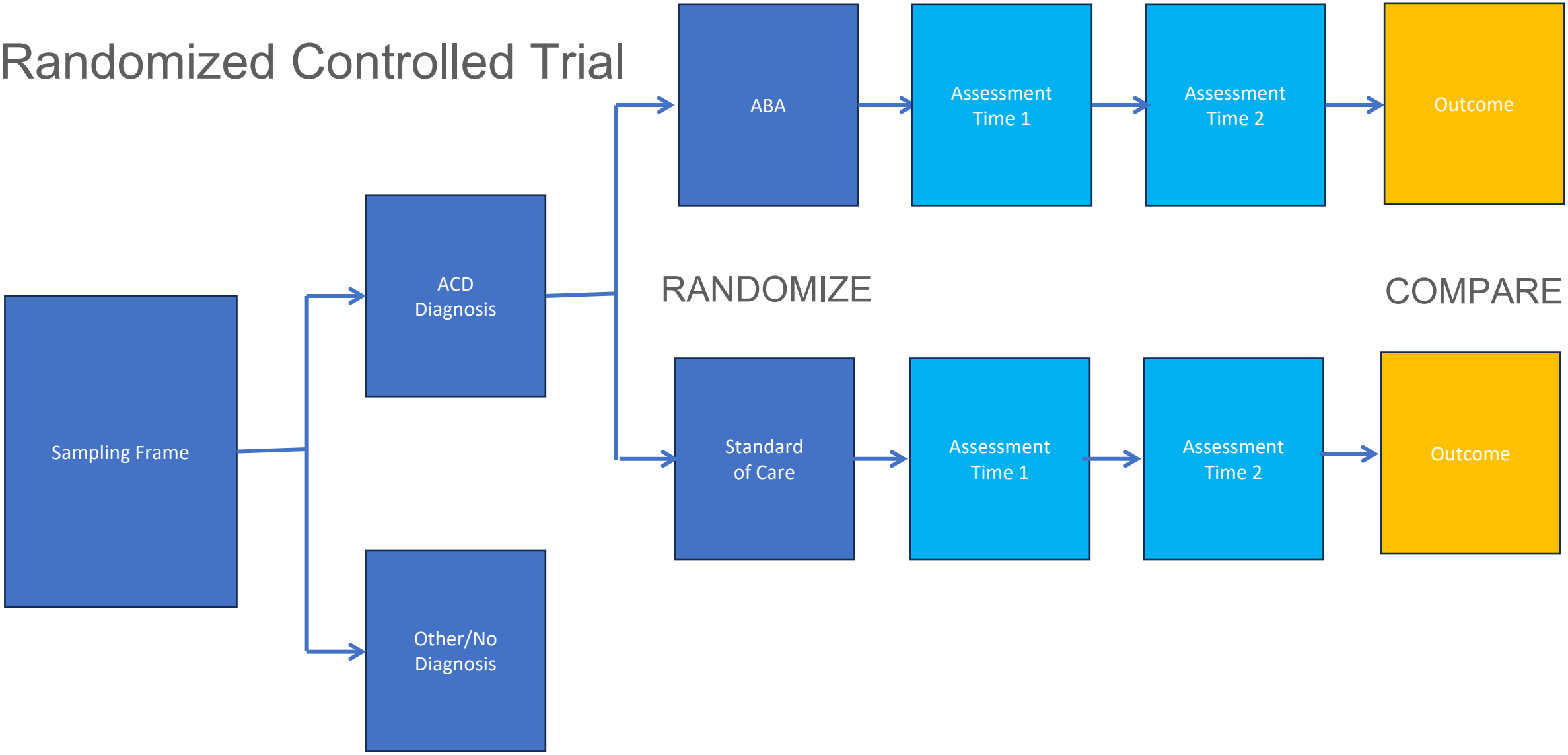
Scientific Validity

- Scientific validity requires that the proposed research uses reliable and valid research design and methods of obtaining data and is relevant to the objective; the findings obtained must be relevant to the health problem being studies; and study design should not affect provision of health care services and should be feasible within the local context of the research setting.
  - Do the scientific and statistical design and methods satisfy generally accepted standards and achieve the objectives of the study? If not, is there clear justification for the deviations?
  - Will the research results be interpretable and useful in the context of the health problem?
  - Does the study design ensure participants health-care services they are entitled to? If not, are there methodologically compelling reasons and are participants protected from serious harm?

Scientific Validity

- Research Question(s)
  - Is access to Applied Behavior Analysis Treatment of Autism Spectrum Disorder (ABA) as good or better than the standard of care?
    - What is the standard of care?
  - Randomize individuals to standard of care or ABA to answer the research question
    - Eligibility for enrollment including diagnosis via validated tool
  - Assessment over time
    - Reliable and valid measures
  - Compare outcomes

Randomized Controlled Trial



Randomized Controlled Trial of ABA

- Not ethical to conduct a randomized controlled trial if there is a clinically effective standard of care

- ABA is the "community standard" outside of DHA
- Specificity and sensitivity of diagnostic criteria for ASD
- Assessments
  - Pervasive Developmental Disorder Behavior Inventory (PDDBI)
  - Vineland Adaptive Behavior Scale Third Edition (Vineland-3)
  - Social Responsiveness Scale, Second Edition (SRS-2)
  - Parenting Stress Index, Fourth Edition, Short Form (PSI-4-SF)

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Waiving Informed Consent

NO EMPIRICAL DATA

POOR

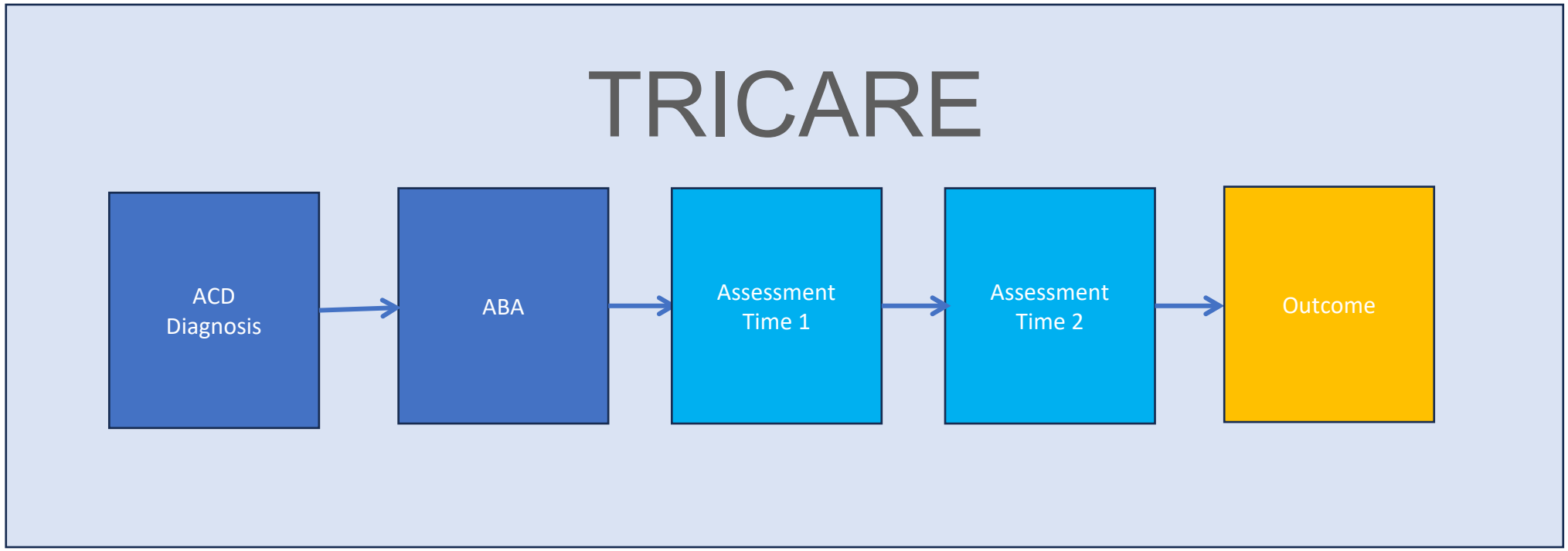
NONE BUILT FOR ABA OUTCOME ASSESSMENT

Quality Improvement/Assessment

- How is quality improvement different from research?
  - Intention is to answer a question about the implementation of an intervention within an organization
    - Apply findings to improve “local” delivery, not to produce generalizable findings
  - Little or no manipulation of patients or environment
  - Risks, benefits no more likely than the usual care
    - Privacy and confidentiality, benefit to patients served by local organization
  - Participation unlikely to restrict reasonable decision making
    - No requirement for obtaining informed consent, notification at entry respectful
  - No requirement for IRB review/oversight
    - Unless institution has voluntarily adopted QI/A review mechanism

Source: Taylor, Pronovost, Fadel et al (2010); Taylor (2018); Kass, Faden, Angus et al (2024)

Quality Improvement Evaluation of ABA



Quality Improvement/Assessment of ABA

- How well is ABA implemented as an intervention for individuals diagnosed with ASD receiving services via TRICARE/ACD ?

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Additional Limitations

- Fidelity of implementation of ABA
  - Organizations with experience delivering ABA face multiple barriers in working with DHA
    - Delays in initiation of ABA
    - Strict penalties for non-compliance
    - Documentation burden
    - Session note expectations
    - Timely reimbursement
  - Low rates of adherence

Additional Limitations

- Assessment Battery
  - Providers
    - Burden of completion knowing value is limited
  - Parents
    - Not trained to use PDDBI
    - Different parents/guardians completing assessments

References

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Kass N, Faden RR, Angus DC, Morain SR. Making the Ethical Oversight of All Clinical Trials Fit for Purpose. JAMA 2024; Online ahead of print.

Waiving Informed Consent

1) At 45 CFR 46.116(c), the regulations identify when IRBs may waive or approve an alteration of informed consent in some research examining state or local public benefit or service programs, or certain features of those programs.

Waving Informed Consent

- the research or demonstration project is to be conducted by or subject to the approval of state or local government officials and is designed to study, evaluate, or otherwise examine:
- public benefit or service programs;
- procedures for obtaining benefits or services under those programs;
- possible changes in or alternatives to those programs or procedures; or
- possible changes in methods or levels of payment for benefits or services under those programs; 45 CFR 46.116(c)(1).

Waiving Informed Consent

2) At 45 CFR 46.116(d) the regulations identify when IRBs may waive or approve an alteration of informed consent in research that meets four specified criteria.

Waiving Informed Consent

- the research involves no more than minimal risk to the subjects;
- the waiver or alteration will not adversely affect the rights and welfare of the subjects;
- the research could not practicably be carried out without the waiver or alteration; and,
- whenever appropriate, the subjects will be provided with additional pertinent information after participation.