Decision Autonomy in Pragmatic Clinical Trials

Supplement to the TiME Trial

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Goals of Supplement Project

Context: In the service of learning, clinical trials curtail autonomy of individual patients and physicians to choose specific treatments.

We seek to learn:

– How patients and physicians view treatment autonomy
– How these valuations differ in the contexts of research versus clinical care
– Whether limitations on treatment autonomy should influence approach to informed consent
Motivated by the TiME Trial

Enroll and Randomize Facilities

- **Intervention Facilities**
  - $\geq 4\,\text{hr, 15 min. sessions}$

Enroll incident patients

- **Usual Care Facilities**
  - (duration not driven by trial)

Primary outcome: All-cause mortality

Secondary outcomes: Hospitalizations & Quality of Life
Aims

Aim 1: Assess qualitatively how patients treated with hemodialysis and their providers value physician autonomy to choose among treatment strategies that are within the range of the standard of care.

Aim 2: Quantify how curtailing treatment autonomy influences patients’ and providers’ willingness to participate in RCTs, and whether these influences differ in research vs. clinical care settings.

Aim 3: Measure the extent to which requirements for informed consent modify patients’ and providers’ concerns regarding the curtailment of treatment autonomy in research and clinical care.
Mixed Methods Approach

• Phase 1 (Aims 1 and 3): Semi-structured interviews
  – Patients: dialysis units in Philadelphia area (urban and suburban) not participating in the TiME Trial
  – Nephrologists: TiME Trial facilities

• Phase 2 (Aims 2 and 3): Conjoint analysis
  – Patients: dialysis units in Philadelphia area not participating in the TiME Trial
  – Nephrologists: TiME Trial facilities

Phase 1 informs Phase 2
Semi-Structured Interviews

• 2 vignettes presented to each participant, sequence varied
  – Research vignette: similar to TiME Trial
  – Clinical vignette: new facility policy
• Followed by open-ended questions
Interview Script – Research Vignette

If you were asked to participate in this study, what would your initial thoughts and reactions be?

• What factors would be important in your decision to participate or not?
• What do you like about the study, and what might motivate you to participate in it?
• In what ways might participating in this study benefit you?
• Are there any reasons that you might not want to participate in this study? What are they?
• What other concerns might you have about participating in this study?
Further prompts (only provide if needed)

Would participating in this study affect your relationship with your doctor? If so, how?

Suppose you were asked to help someone else decide whether to participate in this study. What would you tell this person in order to help them decide?

Are there other things you would want to know about the study before deciding whether or not to participate? What are these?

Further prompts (only provide if needed)

What else, if anything, would you like to know about the risks of being in the study?

What else, if anything, would you like to know about the benefits of being in the study?

How comfortable are you with the way patients are told about the study?

Do you have suggestions for a better way to inform and enroll patients?

Do you think it is appropriate that if patients do not want to participate, they can make a phone call to ask to be removed from the study?
Phase 2: Conjoint Analysis (“Discrete Choice Experiment”)

- Experimental design forces participants to **reveal** preferences rather than to **state** preferences
- Use questionnaires to present multiple hypothetical RCTs to assess how attributes identified as important in Phase 1 influence willingness of patients/nephrologists to participate in RCTs
  - Restrictions on autonomy
  - Rationale for restriction: RCT participation vs adoptions of clinical practice guidelines
  - Burdens of trial participation for patients or providers
  - Inclusion of processes for informed consent, notification or neither
- Attributes presented to participants are systematically varied
- Evaluate main effects and interactions
Hypothetical RCTs

• Restrictions on autonomy
  – Longer without individualization
  – Longer with individualization

• Rationale for restriction on autonomy
  – RCT vs Clinical Practice Guideline
  – Hypothesized benefit

• Burdens of trial presentation
  – Extra tests

• Informed consent process: traditional, streamlined, none
# Timeline and Progress

<table>
<thead>
<tr>
<th>Task Description</th>
<th>Year 1</th>
<th>Year 2</th>
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<tr>
<td>Develop and finalize interview guides</td>
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<td>IRB documentation and approval</td>
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<td>Complete semi-structured interviews</td>
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<td>Develop codebook, code interviews</td>
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<td>Qualitative data analysis</td>
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<td>Develop conjoint analysis surveys</td>
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<td>Participant recruitment, questionnaires</td>
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<td>Quantitative data analyses</td>
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<td>Integrate qualitative &amp; quantitative data</td>
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<td>Manuscripts and dissemination</td>
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Discussion

• Content linked tightly to the TiME Trial
• TiME Trial nephrologists included as participants