EXPANDING THE TOOL BOX:
USING THE MULTIPHASE OPTIMIZATION STRATEGY TO BUILD EFFECTIVE AND EFFICIENT INTERVENTIONS

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Outline

- Typical development of a behavioral/biobehavioral intervention
- A different scenario
- Introduction to MOST
- Overview of MOST
- Take away messages
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What is a behavioral/biobehavioral intervention (BBI)?

- BBIs are programs with the objective of improving and maintaining human health and well-being, broadly defined.
- In individuals, families, schools, organizations, or communities.
- Using a strategy that at least in part aims to modify attitudes, cognitions, or behavior.
- And may include biological, medical, pharmaceutical, or surgical aspects.
What is a behavioral/biobehavioral intervention (BBI)?

- BBIs may be aimed at, for example:
  - preventing disease
  - treating disease
  - promoting health
  - preventing violence
  - improving academic achievement
Example of typical development of a BBI

- **GOAL:** Dr. B wants to develop a BBI to reduce STIs among college students
- Dr. B reviews existing literature, devises a conceptual model, and identifies the following 5 intervention components that are important in reducing alcohol use and sexual risk behaviors that contribute to STI acquisition
Example of typical development of a BBI

- 5 intervention components:
  - Descriptive norms
  - Injunctive norms
  - Expectancies
  - Perceived benefits of using protective behavioral strategies (PBS)
  - Self-efficacy to using PBS
Example of typical development of a BBI

- Pilot test components, possibly revise a little
- Combine components into a BBI
- Evaluate the BBI
Example of typical development of a BBI

- Evaluation via a randomized control trial (RCT)
- Experiment with two conditions:
  - Treatment (BBI)
    - Descriptive norms, injunctive norms, expectancies, perceived benefits, and self-efficacy to prevent harm
  - Control (standard of care)
    - No descriptive norms, injunctive norms, expectancies, perceived benefits, and self-efficacy to prevent harm
Example of typical development of a BBI

- Subjects are randomly assigned to treatment or control
- Outcome variable: engagement in unprotected sex
- Would typically be analyzed using regression, with a dummy variable representing treatment and perhaps a few covariates
- If treatment group significantly better, SUCCESS!!
Typical development of BBI

Descriptive norms
Injunctive norms
Perceived benefits
Self-efficacy

STI preventive intervention

Excellent for evaluating whether the packaged intervention performs better than a control or standard of care
If the RCT shows a significant effect, we still do not know

- Which components are making positive contributions to overall effect
- Whether all the components are really needed
- Whether a component’s contribution offsets its cost
- How to make the intervention more effective, efficient, scalable and/or sustainable
Or if the RCT shows a non-significant effect, we do not know

- Whether any components are worth retaining
- Whether one component had a negative effect that offset the positive effect of others
- Specifically what went wrong and what the next steps should be
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Different scenario: Development of a leaf springs manufacturing process

Leaf Spring:
part of truck suspension system
Development of a leaf springs manufacturing process

- **GOAL:** An engineer, Dr. E, wants to develop a manufacturing process that will consistently produce leaf springs of the same length

- Dr. E hypothesizes that the following components are important in obtaining leaf springs of uniform length:
Development of a leaf springs manufacturing process

- Furnace temperature
- Heating time
- Time on the conveyor belt
- Time in the high pressure press
- Temperatures over which quench oil ranges

- Each can be low or high. Suppose Dr. E hypothesizes that higher is better.
Development of a leaf springs manufacturing process

- Let’s think of this as an intervention on the manufacturing process
- How might Dr. B approach this?
- Create a new manufacturing process:
  - High furnace temp, longer heating time, longer time on conveyor belt, longer time in the press, quench oil ranges over higher temps
- Then compare its performance directly to that of the old process
Development of a leaf springs manufacturing process

- OK, how might Dr. E approach this?
- Dr. E might eventually compare a new process to the old one
- But first, would develop and optimize the new manufacturing process
- How?
Development of a leaf springs manufacturing process

- First, Dr. E wants the new process to be implemented as designed
- So, will start by looking at any important constraints
- Example: owners say they must be able to manufacture springs for a cost of < $25
Development of a leaf springs manufacturing process

- Then, Dr. E would assess what resources are available to conduct research
- Carefully select an approach to find out:
  - What is the size and direction of each component’s effect?
  - Is the performance of a component affected by other components?
  - Why are these questions important?
Development of a leaf springs manufacturing process

- Don’t want to waste resources on components that have very small/null/counterproductive effects
- In selecting components, important to account for how one component may impact the performance of another
- Different components have different resource demands, may want to consider in relation to effect
- Want to be able to improve process further in the future
Development of a leaf springs manufacturing process

- Dr. E cannot obtain the necessary information from an RCT
- Each component must be manipulated individually
- Dr. E will select an experimental design that enables this
- Based on the information, will select the set of components that provides the best process that can be obtained while observing $25 limit
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Can behavioral scientists approach a problem more like Dr. E does?

- The multiphase optimization strategy (MOST) has been developed to enable this
- MOST has been inspired by engineering principles
- Framework for preparation, optimization, and evaluation of BBIs
- What if Dr. B took this approach?
Suppose Dr. B took an engineering-like perspective

- Would not immediately include all five components in BBI; instead, would see them as *candidates*
- Goal: to engineer a BBI that
  - Meets specific criteria for
    - Effectiveness
    - Efficiency
    - Economy and/or
    - Scalability
    - i.e. has been optimized, AND
  - Demonstrates statistical and practical significance
Suppose Dr. B took an engineering-like perspective

- Suppose insurers will pay up to $50 per college student for an STI prevention BBI
- Then Dr. B wants to identify the set of components that produces the most effective STI preventive outcome for < $50 per person
- How can Dr. B identify this set?
Suppose Dr. B took an engineering-like perspective

- Dr. B needs to gather the following information:
  - How each individual component performs
  - How components perform together, i.e., impact each other’s performance
    - Are there synergistic relations?
    - Are there antagonistic relations?
- This information will enable screening out poorly performing components
- Experiment to gather this information is called a screening experiment
Suppose Dr. B took an engineering-like perspective

- Let’s assume a factorial experiment was conducted
- Dr. B analyzes the data using factorial ANOVA
  - Gets estimates of main effects and interactions
- Uses this information to weed out poor performers and arrive at the *screened set* that can be later evaluated using an RCT
The MOST approach to STI preventive intervention

Component screening experiment

Descriptive norms

Injunctive norms

Perceived benefits

Expectancies

Self-efficacy

Optimized intervention

Component

Component

Component

RCT
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Figure 1.2: Flow chart of one cycle of the multiphase optimization strategy (MOST) to develop, optimize, and evaluate a behavioral or biobehavioral intervention (BBI). Rectangle = action. Diamond = decision. Round-cornered rectangle = the product, which is the new optimized intervention to be released.

**PREPARATION**
- Review existing information
- Derive/review conceptual model
- Identify set of candidate components
- Conduct pilot/feasibility work
- Identify optimization criterion

**OPTIMIZATION**
- Select approach to experimentation based on resource mgmt principle
- Conduct experiment; collect and analyze empirical data
- Based on results, identify BBI that meets optimization criterion

**EVALUATION**
- Confirm effectiveness of optimized BBI via RCT

**RELEASE OF OPTIMIZED BBI**

- Optimized BBI expected to be sufficiently effective?
  - Yes
    - Use resources to start over, building on what was learned
  - No
    - Resources mgmt principle: Develop improved intervention, building on what was learned

- Optimized BBI sufficiently effective in RCT?
  - Yes
  - Use resources to start over, building on what was learned
  - No
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**RELEASE OF OPTIMIZED BBI**
Preparation phase

- Purpose: to lay groundwork for optimization
- Development/revision of conceptual model
- Pilot testing of components
- Identification of optimization criterion
The conceptual model

- Expresses how the BBI under development is to intervene on the behavioral or biobehavioral process
  - All of what is known, hypothesized, or conjectured

- Should be explicitly theory-based
  - May be informed by more than one theory, if different theories apply to different aspects of the model.

- Should be directly informed by peer-reviewed empirical literature
The conceptual model

- Should be comprehensive
  - Even if not every aspect of it is to be investigated in a particular study

- Should clearly describe the process to be intervened on
  - Key outcome variables
  - Hypothesized causal influences
  - Mediators and moderators of causal mechanisms
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**OPTIMIZATION**
- Select approach to experimentation based on resource mgmt principle
- Conduct experiment; collect and analyze empirical data
- Based on results, identify BBIs that meet optimization criterion

**EVALUATION**
- Confirm effectiveness of optimized BBIs via RCT

**RELEASE OF OPTIMIZED BBIs**
- Use resources to start over, building on what was learned
- Continuous optimization principle: Develop improved intervention, building on what was learned

Optimized BBIs expected to be sufficiently effective?

NO

Yes

Use resources to start over, building on what was learned
Optimization phase

- Purpose: to build an optimized intervention
- Select approach to experimentation
- Conduct fully powered experiment
- Based on results, identify optimized BBI
Definition of optimization of a BBI

- Optimization of a BBI is the process of identifying the BBI that provides the highest expected level of effectiveness obtainable within key constraints imposed by the need for efficiency, economy, and/or scalability.

- Note:
  - Process
  - Key constraints
  - Highest expected level obtainable
Optimization phase

- What kind of approach?
  - For fixed, multicomponent interventions → Factorial experiments (or fractional factorial experiments)
  - Adaptive interventions → SMART (Sequential Multiple Assignment Randomized Trial)
  - Just-in-time adaptive intervention → mRT (Microrandomized Trial) or systems identification
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**EVALUATION**
- Confirm effectiveness of optimized BBI via RCT

If **Optimized BBI expected to be sufficiently effective?** is NO, use resources to start over, building on what was learned.

If **Optimized BBI sufficiently effective in RCT?** is NO, use resources to start over, building on what was learned.

If **Optimized BBI expected to be sufficiently effective?** is YES, use resources to start over, building on what was learned.

**RELEASE OF OPTIMIZED BBI**
Evaluation phase

- Evaluate optimized intervention in RCT compared to a control or standard of care
### Evaluation and optimization: Both important, not the same thing

<table>
<thead>
<tr>
<th>Optimization: Is the intervention the most effective obtainable within key constraints?</th>
<th>Evaluation: Is the intervention's effect statistically significant?</th>
</tr>
</thead>
<tbody>
<tr>
<td>No</td>
<td>No</td>
</tr>
<tr>
<td></td>
<td>Identify stronger set of components via screening experiment</td>
</tr>
<tr>
<td></td>
<td>Intervention can probably be improved</td>
</tr>
<tr>
<td>Yes</td>
<td>Yes</td>
</tr>
<tr>
<td></td>
<td>Identify stronger components or optimize using different criterion</td>
</tr>
<tr>
<td></td>
<td>What we should be aiming for</td>
</tr>
</tbody>
</table>
The resource management principle

- Investigator using MOST must strive to make the best and most efficient use of available resources
  - Available = what the investigator has or can reasonably obtain
- Greatest amount and most appropriate scientific information for money and other resources spent
- MOST does not require an increase in research resources
  - But possibly a realignment
The continuous optimization principle

- Optimization is a process, not an end point
- Think about engineering of consumer products
- Ongoing quest for incrementally better BBIs
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Optimized BBI expected to be sufficiently effective?

**EVALUATION**
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Optimized BBI sufficiently effective in RCT?

**RELEASE OF OPTIMIZED BBI**

Use resources to start over, building on what was learned

Continued optimization principle.
Develop improved intervention, building on what was learned

Use resources to start over; building on what was learned

Optimized BBI expected to be sufficiently effective?
Returning to the preparation phase

- Three return arrows on figure
- If optimized BBI is not expected to be sufficiently effective
  - Resource management principle
- If optimized BBI turns out not to have a statistically significant effect
- After release of BBI
  - Continuous optimization principle
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Take away messages

- MOST is an engineering-inspired framework for building more effective and efficient BBIs
  - Requires an additional phase of optimization

- Resource management principle guides study design

- Using MOST can help move science forward faster
  - As constraints are lifted, new research questions can be asked
Thank you!

- Questions or comments, please contact me
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- Visit Methodology Center website:
  - http://methodology.psu.edu