PENNSTATE

The Methodology Center

PREVENTION IN THE 21ST CENTURY: ADAPTING ENGINEERING OPTIMIZATION STRATEGIES TO CREATE LEANER, MEANER, BETTER INTERVENTIONS

LINDA M. COLLINS
PENN STATE

Presidential address at the Annual Meeting of the Society for Prevention Research , 2011

Overview

- Prevention in the 21st century (so far)
- Cancer prevention and manufacturing of truck leaf springs
- What is optimization, and how can it get me a leaner, meaner, better preventive intervention?
- Statistically significant does not mean optimized
- Examples of optimization
- Summary and concluding remarks

Prevention in the 21st century:

- Need for prevention remains high
- Resources (time, money, etc.) for implementation of prevention programs flat at best
- How to convince the public of worth of prevention?
  - Achieve and maintain high levels of
    - Efficacy
    - Effectiveness
    - Cost-effectiveness

Prevention research in the 21st century:

- Resources for prevention research flat at best
- Prevention research must squeeze most scientific information out of available resources
- We need to be extremely efficient about how we conduct research

The challenge:

- We need to develop preventive interventions that are highly efficacious, effective, and cost-effective…
- …without exceeding the current level of research resources.

Can we meet this challenge?

Yes —

if we adopt a different mindset about prevention research…

…an *engineering-based* mindset.

Scenario 1: Cancer prevention: Developing a smoking cessation intervention

- Goal: choose from set of components/component levels to maximize probability of successful quitting

Definition: intervention components

- Intervention components: *Any aspects of an intervention that can be separated out for study*
  - Parts of intervention content
    - e.g. : topics in a drug abuse prevention curriculum
  - Features that promote compliance/adherence
    - e.g.: reminder phone calls
  - Features aimed at improving fidelity
    - e.g.: enhanced teacher training
- Can impact efficacy, effectiveness, cost-effectiveness

Before I go on…

- To keep things simpler, I am going to refer only to effectiveness
- Everything I say applies to efficacy too!

Scenario 1: Cancer prevention: Developing a smoking cessation intervention

- Goal: choose from set of components/component levels to maximize probability of successful quitting

Scenario 1: Cancer prevention: Developing a smoking cessation intervention

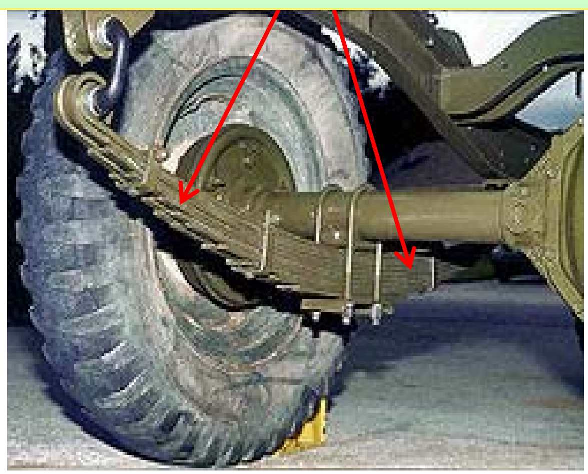
- Goal: choose from set of components/component levels to maximize probability of successful quitting
- Components:
  - Precessation nicotine patch (No, Yes)
  - Precessation nicotine gum (No, Yes)
  - Precessation in-person counseling (No, Yes)
  - Cessation in-person counseling (Minimal, Intensive)
  - Cessation phone counseling (No, Yes)
  - Maintenance medication duration (Short, Long)

Scenario 1: Cancer prevention: Developing a smoking cessation intervention

- How to build a behavioral intervention out of these components?
- Construct new intervention by setting each component at highest level, put them together
  - Intervention = precessation patch and gum and counseling, intensive cessation in-person and phone counseling, long medication duration
- Then compare to control group via RCT
- Possibly conduct post-hoc analyses
- Let's call this the **treatment package approach**

Scenario 2: Developing a way to manufacture truck leaf springs

- Goal: Choose from set of components/component levels to optimize amount of variability in length of leaf springs (less variability is better)



- Pignatiello and Ramberg (1985) in Wu & Hamada (2000)

Scenario 2: Developing a way to manufacture truck leaf springs

- Goal: Choose from set of components/component levels to optimize amount of variability in length of leaf springs (less variability is better)
- Components (suppose for each one higher hypothesized to be better):
  - Furnace temperature (lower, higher)
  - Heating time (shorter, longer)
  - Transfer time on conveyor belt (shorter, longer)
  - Hold down time in high pressure press (shorter, longer)
  - Quench oil temperature range (lower temps, higher temps)

Scenario 2: If engineers thought like behavioral scientists

- Would use the treatment package approach
- Construct new manufacturing process = higher furnace temp, longer heating time, longer conveyor belt time, longer time in high pressure press, higher temp quench oil
- Compare this process as a package to the old way, see if it is demonstrably better
- Conduct post-hoc analyses

Scenario 2: Developing a way to manufacture truck leaf springs

- But an engineer would not use the treatment package approach, because:
  - If the new process IS better, doesn’t indicate which components make a difference
  - If the new process IS NOT better, doesn’t indicate which (if any) of the components did effect an improvement
  - When repeated, no guarantee of systematic incremental improvement, so not a good long-run strategy
  - Does not take cost or other constraints into account

Scenario 2: Developing a way to manufacture truck leaf springs

- What WOULD an engineer do?
- Start with a clear idea of the goal, including constraints
  - e.g. Least variability AND must cost less than $1/spring
- Using the resources available, design an efficient experiment to gather needed information (e.g. individual effects of components)
- Based on the results of experiment, choose components and component levels to achieve stated goal. THIS IS OPTIMIZATION
- THEN compare new process to old process

Back to Scenario 1: If behavioral scientists thought like engineers

- We might want to <u>optimize</u> the smoking cessation intervention
- Using an approach that
  - Indicates which components are active
  - Ensures an incremental improvement, and therefore is the fastest way to the best intervention IN THE LONG RUN
  - Readily incorporates costs/constraints of any kind
  - Enables optimization using any desired criterion

I think we can all agree on this:

- We all want to help make preventive interventions better.
- But we have different ideas about what constitutes "better."
- That's OK!
- Instead let's talk about "optimized."

Definition of better: optimized

- "The best possible solution… subject to given constraints" [emphasis added] (*The Concise Oxford Dictionary of Mathematics*)
- **Optimized does not mean best** in an absolute or ideal sense
- Instead, realistic because it includes constraints
- Optimization always involves a clearly stated optimization criterion
  - A working definition of what YOU mean by "better"

Identifying your optimization criterion, or what you mean by "better"

- Your definition of "best possible given constraints"
- This is the goal you want to achieve

One possible optimization criterion:

- **Intervention with no inactive components**
  - Example: *School districts are finding it difficult to incorporate all of Program NODRUGS into their packed school day. The investigators want to be confident that every component is necessary.*
  - Achieve this by: Selecting only active intervention components.

Another possible optimization criterion:

- **Most effective intervention that can be delivered for ≤ some $$**
  - Example: *To have a realistic chance of going to scale, Program NODRUGS must cost no more than $50/student to deliver, including materials and health educator time.*
  - <u>Achieve this by</u>: Selecting set of components that represents the most effective intervention that can be delivered for ≤ $50.

Another possible optimization criterion:

- **Most effective intervention that can be delivered in ≤ some amount of time**
  - Example: *A smoking cessation intervention, Program QUIT, is to be administered in health care settings. Interviews with staff suggest that the intervention has the best chance of being implemented well if it takes no more than 15 minutes to deliver.*
  - Achieve this by: Selecting set of components that represents the most effective intervention that can be delivered in ≤ 15 minutes.

Another possible optimization criterion:

- **Most cost-effective intervention**
  - Example: *To have a chance at being adopted statewide, Program NODRUGS must be convincingly cost-effective.*
  - Achieve this by: Selecting set of components that represents the most cost-effective intervention
    - NOTE: May not be least costly or most effective

Evaluation and optimization: Both important, not the same thing

		Evaluation: Is the intervention's effect statistically significant?
Optimization: Is the intervention the best possible, given constraints?	No	Yes
No	**May wish to optimize using effect size as criterion**	**Intervention can probably be improved**
Yes	**Different intervention strategy needed**	**What we should be aiming for**

- OK, you've decided on an optimization criterion
  - No inactive components
  - Most effective that can be delivered for ≤ $50
  - Most effective that can be delivered in ≤ 15 minutes
  - Most cost-effective
  - OR something else
- **Now what?**
- **Engineer an intervention to achieve the optimization criterion.**

Example 1: Clinic-based smoking cessation study funded by NCI

Tim Baker

Mike Fiore

University of Wisconsin

Team includes: B.A. Christiansen, L.M. Collins, J.W. Cook, D.E. Jorenby, R.J. Mermelstein, M.E. Piper, T.R. Schlam, S.S. Smith

Example 1: Clinic-based smoking cessation study funded by NCI

Tim Baker

Mike Fiore

University of Wisconsin

<u>Objective: Develop a "lean" clinic-based smoking cessation intervention</u>

Six components being considered for the smoking cessation intervention**

- Precessation nicotine patch (No, Yes)
- Precessation nicotine gum (No, Yes)
- Precessation in-person counseling (No, Yes)
- Cessation in-person counseling (Minimal, Intensive)
- Cessation phone counseling (No, Yes)
- Maintenance medication duration (Short, Long)

**Note: these are not all the components in the intervention, just the ones being examined

Optimization criterion

- Maximize probability of successful quitting…
- Given these six components…
- Selecting only demonstrably active intervention components
  - Leaner!
- (In actuality, we will be examining several different optimization criteria)

Decisions in smoking cessation study

- These decisions involve whether or not a component should be included in the intervention:
  - *Decision 1*: Should intervention include use of the nicotine patch during precessation?
  - *Decision 2*: Should intervention include use of nicotine gum during precessation?
  - *Decision 3*: Should intervention include precessation counseling?

Decisions in smoking cessation study

- These decisions involve <u>which level of a component</u> should be included:
  - *Decision 4*: Should intervention include intensive or minimal level of counseling during cessation?
  - *Decision 5*: Should intervention include intensive or minimal level of telephone-delivered counseling during cessation?
  - *Decision 6*: Should intervention include standard or extended period of cessation NRT?

Decisions in smoking cessation study

- How can we make the decisions we need to make?
- Gather empirical evidence about
  - effectiveness of each component or
  - relative performance of each level of a component
- Experimental manipulation of each component necessary
- Approach: randomized experiment(s) that identify the effects attributable to each component

Examples of outcome variables

- Number days abstinent in 2-week period post-quit
- Post-quit self-efficacy
- Feelings of withdrawal and craving

How to conduct an experiment to examine individual component effects

- We decided to conduct a factorial experiment
- N=512 subjects TOTAL provides power $\geq$ .8
- We can talk about the design more during the roundtable

Engineering the intervention

- Experiment will give us
  - Main effect of each individual intervention component on outcomes
  - Selected interactions between intervention components
- This information will be used to select components/component levels
- **Result: optimized clinic-based smoking cessation intervention**
- Plan to conduct an RCT to establish statistical significance of effect

Example 2: School-based drug abuse/HIV prevention study funded by NIDA

Linda L. Caldwell Edward A. Smith

Penn State

Team includes: D. Coffman, L. Collins, J. Cox, I. Evans, J. Graham, M. Greenberg, J. Jacobs, D. Jones, M. Lai, C. Matthews, R. Spoth, L. Wegner, T. Vergnani, E. Weybright

Example 2: School-based drug abuse/HIV prevention study funded by NIDA

Linda L. Caldwell Edward A. Smith

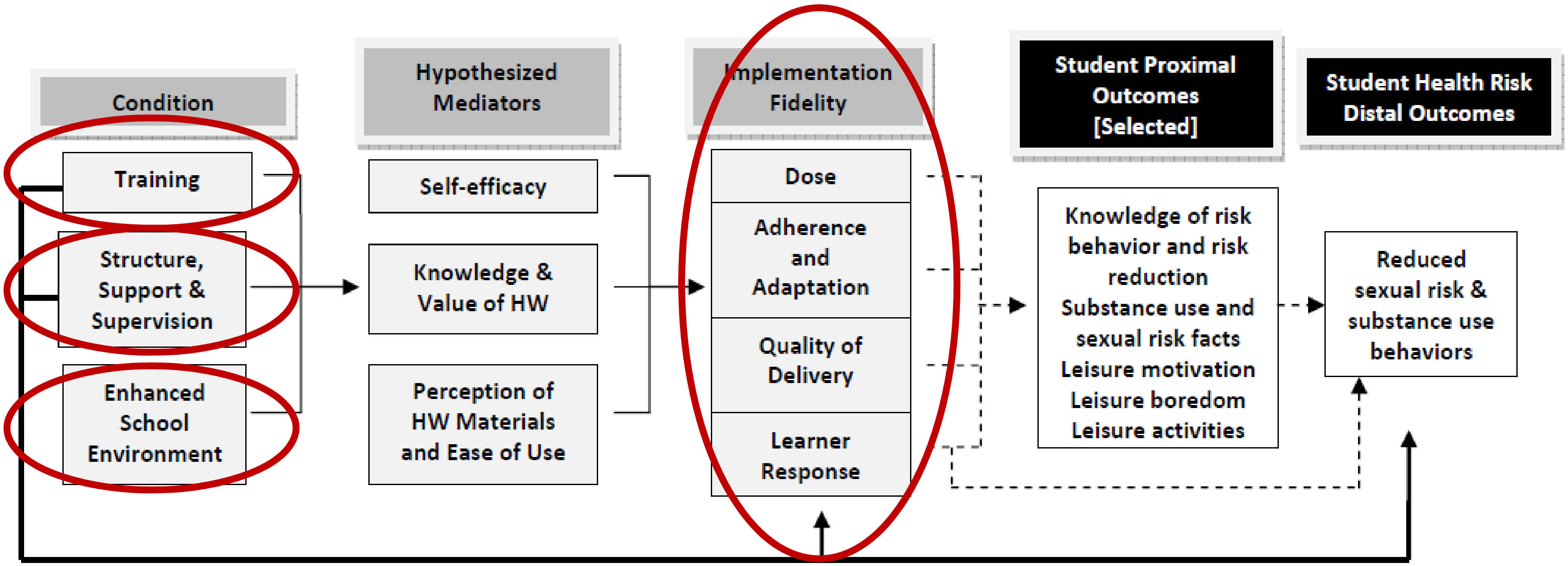
Penn State

<u>Objective: To develop a strategy for maintaining implementation fidelity</u>

Background

- *HealthWise* school-based drug abuse/HIV prevention intervention
- Has previously been evaluated in South Africa
- Metropolitan South Education District in South Africa wants to implement HealthWise in all its schools
- Question: how to maintain fidelity?
- Metro South allowed us to conduct an experiment

Caldwell & Smith's model of implementation fidelity



Optimization criterion

- Maximize implementation fidelity…
- Given these three components…
- Selecting only active components
  - Leaner!

Component 1: Training

- Levels of Component 1:
  - Standard training = one and one-half days
  - Enhanced training = three days + two additional days four months later
- *Decision 1:* Should the intervention include Standard or Enhanced teacher training?

Component 2: Structure, support, and supervision (SS&S)

- Levels of Component 2
  - No additional teacher SS&S
  - Additional teacher SS&S, e.g.: weekly text messages; monthly visits from support staff; option to call support staff with questions as needed
- *Decision* 2: Should the intervention include teacher SS&S?

Component 3: Enhanced school climate

- Levels of Component 3
  - No school climate enhancement
  - School climate enhancement, e.g., form committee of parents and teachers to promote *HealthWise*; develop visuals; issue newsletter
- *Decision 3*: Should the intervention include efforts to enhance school climate?

How to conduct an experiment to examine individual component effects

- We decided to conduct a factorial experiment. Why?
- Enables examination of individual component effects AND
- Requires **smaller** sample sizes than alternative designs
  - Yes, I mean **smaller**
- BUT they also usually require more experimental conditions than we may be accustomed to
- Experiment uses all 56 schools in district

Multilevel structure in this example

- Children nested within classrooms, classroom nested within schools
- Unit of analysis for primary outcomes is school or teacher

HealthWise experiment in South African school district. **56** schools in all; ***7*** schools assigned to each experimental condition

Experi-mental condi-tion	*N* of schools	Health-Wise program	Training	Structure, support, & super-vision	Enhan-ced school climate
1	*7*	✓			
2	*7*	✓			✓
3	*7*	✓		✓	
4	*7*	✓		✓	✓
5	*7*	✓	✓		
6	*7*	✓	✓		✓
7	*7*	✓	✓	✓	
8	*7*	✓	✓	✓	✓

Are 7 schools per experimental condition enough?

- We estimated power ≥ .8 for main effects
- Remember that each main effect estimate is based on ALL schools
- In a factorial experiment you DO NOT compare individual conditions

Main effect of Training is mean of (5,6,7,8) vs. mean of (1,2,3,4).

Note that all 56 schools are used in estimating the main effect.

Experi-mental condi-tion	*N* of schools	Health-Wise program	Training	Structure, support, & super-vision	Enhan-ced school climate
1	7	✓			
2	7	✓			✓
3	7	✓		✓	
4	7	✓		✓	✓
5	7	✓	✓		
6	7	✓	✓		✓
7	7	✓	✓	✓	
8	7	✓	✓	✓	✓

Main effect of Structure, support, & supervision is mean of (3,4,7,8) vs. mean of (1,2,5,6).

Note that all 56 schools are used in estimating the main effect.

Experi-mental condi-tion	*N* of schools	Health-Wise program	Training	Structure, support, & super-vision	Enhan-ced school climate
1	7	✓			
2	7	✓			✓
3	7	✓		✓	
4	7	✓		✓	✓
5	7	✓	✓		
6	7	✓	✓		✓
7	7	✓	✓	✓	
8	7	✓	✓	✓	✓

Main effect of Enhanced school climate is mean of (2.4.6.8) vs. mean of (1,3,5,7).

Note that all 56 schools are used in estimating the main effect.

Experi-mental condi-tion	*N* of schools	Health-Wise program	Training	Structure, support, & super-vision	Enhan-ced school climate
1	7	✓			
2	7	✓			✓
3	7	✓		✓	
4	7	✓		✓	✓
5	7	✓	✓		
6	7	✓	✓		✓
7	7	✓	✓	✓	
8	7	✓	✓	✓	✓

Engineering the intervention

- Experiment will give us
  - Main effect of each individual intervention component on
    - Implementation fidelity outcomes
      - Adherence
      - Dose
      - Quality
      - Participant responsiveness
  - Also interactions between intervention components
- This information will be used to select the best set of the three components
- **Result: Intervention engineered to optimize fidelity according to our criterion**
- Note: optimized ≠ the best possible

"Optimized" is a working definition of "better"

- Want leaner? Go for "only active components."
- Want cheaper? Go for "most effective I can get for less than $$$."
- Want efficient? Go for "most effective I can get for less than *x* minutes of implementation time."
- Or use a different definition!

Statistically significant and optimized are different

- The RCT is a great way to evaluate preventive interventions
- It is not a good way to optimize preventive interventions
- Clinically or practically significant do not mean optimized
- Even the best evidence-based interventions have not been optimized

It is feasible to optimize preventive interventions

- There are efficient experimental designs that provide the information needed
  - Even when there is a multilevel structure
  - Given currently available levels of funding
- Both examples discussed estimated power $\geq .8$ for main effects

Optimization is a process

- Any intervention can be improved
- An intervention that has been optimized using one optimization criterion and set of components can be further improved
  - Made shorter or cheaper
  - Optimized again incorporating new components, approaches, boosters, etc.
  - Optimized for performance in a particular subgroup

Some possible benefits of this approach

- Fewer failed RCT's
  - If not satisfactory after optimization, do not proceed to RCT
  - BUT you know which components worked and which did not
- Increased ability to build on prior work
- Improved understanding of what matters in prevention
- More precise mediation analyses
  - Link mediators with specific components

Effectiveness and cost-effectiveness can be goals

- Don't have to accept effectiveness or cost-effectiveness of an intervention as a "given"
- Possible to set goals and engineer interventions to meet them
- We can set goals for the field – and meet them

This approach may be used more in prevention if…

- Prevention scientists focus on both optimization and evaluation
- Review committees become more open to a variety of experimental designs
- NIH program officials encourage optimization of preventive interventions

Multiphase Optimization Strategy (MOST)

- Comprehensive framework for intervention optimization AND evaluation
- Example publication: Collins, L.M., Baker, T.B., Mermelstein, R.J., Piper, M.E., Jorenby, D.E., Smith, S.S., Schlam, T.R., Cook, J.W., & Fiore, M.C. (2011). The Multiphase Optimization Strategy for engineering effective tobacco use interventions. *Annals of Behavioral Medicine*, *41*, 208-226.

http://methodology.psu.edu

We can engineer leaner, meaner, better preventive interventions…

and help ensure that prevention maintains a central role in 21st century public health.

Join us at the roundtable in Regency A at 4:00 if you would like to:

- Learn more about details (e.g. designs)
- Discuss how these ideas could apply in your research

OR

- Express skepticism
- Argue