

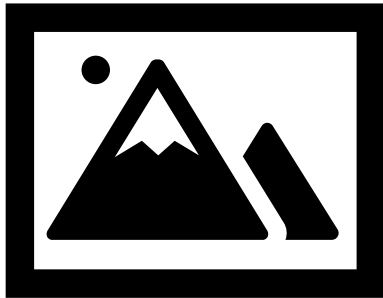
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CONFERENCE

10th Anniversary Edition

Efficacy is Essential in Behavioral Intervention Research



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PLENARY SESSION

How to Navigate the Efficacy, Effectiveness, and Implementation Continuum

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Everybody loves efficacy



So why talk about it?

- Efficacy is essential in behavioral intervention research.
- But its essential role in our work is being challenged by several trends.

What is Efficacy (as opposed to Effectiveness)?

- Merriam-Webster Dictionary definition
 - The ability to produce a desired or intended result.
 - 'Produce' implies a causal effect – that the intervention causes the outcome.
- NIH definition*
 - **Efficacy** can be defined as the performance of an intervention under ideal and controlled circumstances, whereas **effectiveness** refers to its performance under 'real-world' conditions.
 - This may seem like an either/or dichotomy, but it's not.
 - There's an efficacy-effectiveness (a.k.a. explanatory-pragmatic) **continuum**. **

*Gartlehner G. et al. (2006). *Criteria for Distinguishing Effectiveness from Efficacy Trials in Systematic Reviews*. Rockville, Maryland: AHRQ

**Loudon K. et al. The PRECIS-2 tool: designing trials that are fit for purpose. *British Medical Journal* 2015;350:h2147

Relativity

- Efficacy (and effectiveness) are *relative findings* – not inherent properties of our interventions.
 - An intervention is efficacious only if its outcome(s) are superior to those of a comparator.
 - Trials of the same intervention vs. different comparators can produce different effect sizes.
- We may casually claim that a particular intervention is “effective.”
 - But that’s not a complete thought.
 - “Effective” relative to *what?* And under which conditions in which contexts?
 - *An intervention may be superior to one comparator but not to another.*
 - It doesn’t do patients any good when we ignore this inconvenient truth.

Major Translational Research Frameworks Incorporate Efficacy

Example: ORBIT Model

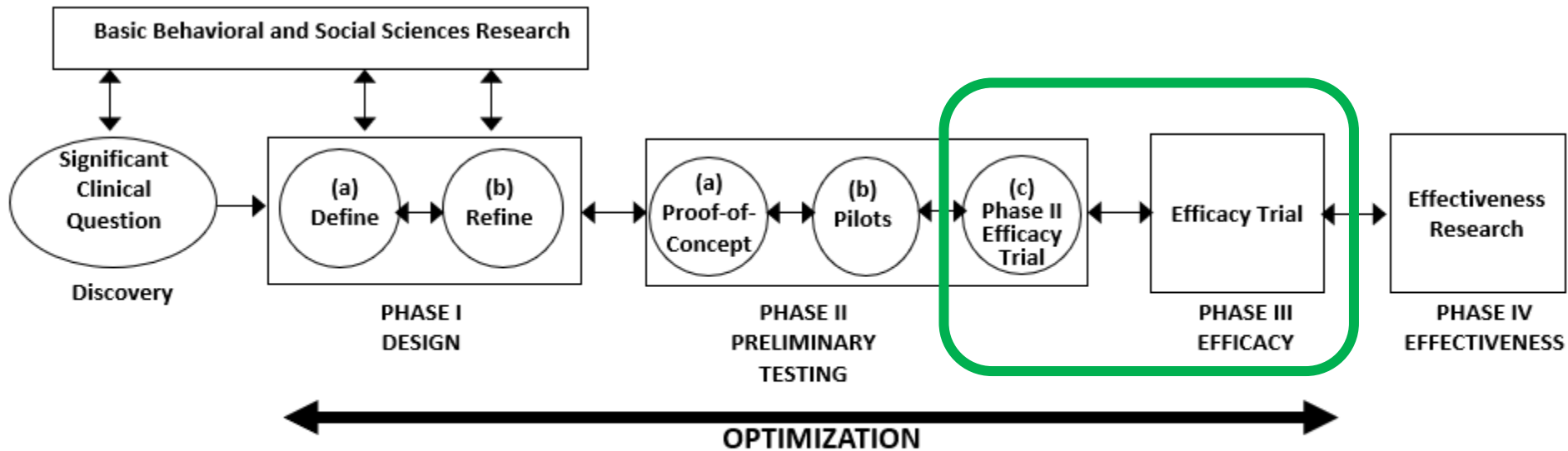
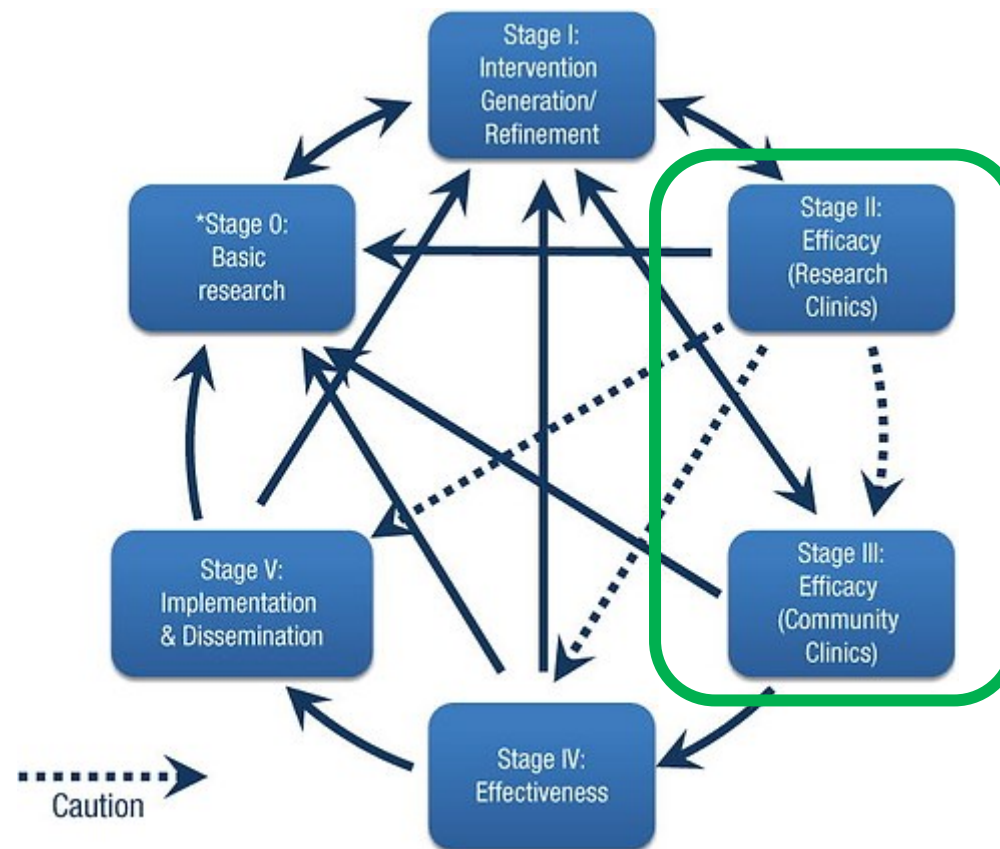


Figure 1. The ORBIT Model for Behavioral Treatment Development

Major Translational Research Frameworks Incorporate Efficacy

Example: NIH Stage Model



What Do Rigorous Efficacy Trials Tell Us About Our Interventions?

- Whether there is a causal relationship between the intervention and the outcome(s).
 - Thanks to experimental design and rigorous controls for internal validity.
- *If the results are favorable*: How well does the intervention work under optimal conditions?
 - Provides a benchmark against which to judge the intervention's 'real-world' effectiveness.
- *If the results are disappointing*: Why didn't the intervention perform as well as we had hoped?
 - Efficacy trials enable us to differentiate between two basic explanations:
 - The intervention underperformed due to inadequate fidelity and/or adherence.
 - The intervention underperformed *despite* adequate fidelity and adherence.

Three Challenges to the Role of Rigorous Behavioral Efficacy Trials

1. The persistent plague of preliminary efficacy trials (PETs).
2. Scalability über alles.
3. Impatience.

Preliminary Efficacy Trials (PETs)

- Leading experts on clinical trial methodology agree:
 - Pilot trials should be designed to evaluate the *feasibility* of a future, full-fledged RCT.
 - They should *not* be designed to be miniature, severely underpowered PETs.
 - Other kinds of preliminary studies – not PETs – should be conducted to assess the readiness of the intervention for testing in a rigorous efficacy trial.
 - Other approaches should be pursued to choose effect sizes for RCT power analyses.
 - RCTs should be powered to detect *clinically significant* effects, not PET effects.
- **The CONSORT extension for pilot trials is completely consistent with this view.**

Preliminary Efficacy Trials (PETs)

- PETs are still very popular.
- Their methodological flaws aren't the only problem.
 - Only about 5% of published PETs are ever followed by a rigorous RCT.
 - The published PET results often become the last word on the intervention's efficacy.
 - All too often, they're taken all too seriously by clinicians, health science reporters, etc.
 - When PETs *are* followed by full-fledged RCTs, the effect sizes usually shrink.
 - They may even shrink to zero.
- **For these reasons, PETs are a threat to our standards for judging the efficacy of our interventions.**

Scalability is All That Matters: NOT!

- Scalability is an important consideration in behavioral intervention research.
 - In this context, 'scalable' means 'deliverable on a larger scale' or 'implementable in other settings.'
- We've been drilling scalability into the heads of our trainees for years.
 - But perhaps we've overdone it.
- Too many investigators are trying to develop highly scalable interventions without paying sufficient attention to efficacy and clinical significance.
- What does it profit a patient to gain easy access to an intervention that doesn't work very well?



Impatience



The answer is 17 years, what is the question: understanding time lags in translational research

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Impatience

- We've all heard that it takes an average of 17 years for research evidence to reach clinical practice.
 - *(Shhh! Don't tell that to the trialists who've tested semaglutide and tirzepatide!)*
- Concern about the slow pace of translation into practice has led many behavioral scientists to jump off the deep end of implementation science.
 - They don't care about efficacy.
 - Some of them don't even care about 'preliminary efficacy.'
 - All they seem to care about is rushing their intervention into 'real-world' implementation.

Impatience



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VIEWPOINT

Testing Psychosocial Interventions in the Contexts They Are Meant to Be Delivered

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- The authors argue that efficacy trials are usually unnecessary.
 - And that they're often useless because they're not conducted in 'real-world' contexts.
- They propose moving as expeditiously as possible to hybrid trials and other implementation studies in the 'real world' contexts in which the interventions are meant to be delivered.

COMMENTARY

Testing Psychosocial Interventions in Context: Commentary on [Beidas et al. \(2023\)](#)

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In press

Conclusion

- Efficacy good!
- PETs bad!
- Scalability & efficacy friends!



Thank you !

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