Platform Trials: Marrying research and quality improvement at global scale

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Research provides the knowledge base necessary to deliver high quality clinical care. Clinical research proceeds from the assumption that the best care of patients with a disease can be inferred from an objective evaluation of what happens within a population when two or more alternative approaches are compared: the mechanism for such a comparison is the randomized clinical trial (RCT). RCTs minimize bias and so enhance confidence that research findings reflect the impact of the intervention studied. Particularly within the discipline of critical care, the results of an RCT may differ from conventional wisdom, or from what might be inferred from physiology. Thus the provision of the best care to critically ill patients mandates a continuing assessment of the impact of that care. Yet health care systems are not currently structured to enable such a process.

Platform trials are emerging as a research model that can integrate scientific discovery and quality improvement, and do so at an unprecedented scale. A platform trial studies a cohort of patients with a common illness, rather than a single intervention to treat that illness. As a consequence, it can study multiple different interventions at the same time, expediting the generation of knowledge about any of those interventions, and at the same time, enabling assessment about how these interventions might interact. Conducting a platform trial using the principles of a Bayesian adaptive analytic model enables the trial to better integrate with clinical care. It acknowledges biases and perspectives that may exist before the trial starts, and even more importantly, can accommodate accruing knowledge as the trial proceeds through the process of response adaptive randomization (RAR), which adjusts randomization proportions as the trial proceeds, preferentially recruiting patients to interventions that appear to be performing better. In its most fully developed form, RAR results in all patients being randomized to an intervention that has met a priori criteria for efficacy, and so marries the generation of knowledge with its uptake into clinical practice.

As a mechanism to support collaboration at a grand scale, the platform trial holds the promise of uniting our small planet in a common investigative endeavor, and accelerating the generation of new knowledge that can benefit the most vulnerable amongst us.