Better Science, Better Health: New Trial Pathways and Better Patient Data

Blazing Trails - New Development Pathways in Action

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Moving Beyond the Randomized Clinical Trial

The FDA needs to "turn the clinical trial paradigm on its head. We are going to have to change the way drugs are developed. Period."

Janet Woodcock
Director
FDA Center for Drug Evaluation and Research

National Press Club
Washington, D.C.
May 21, 2013

Options for Improving Trial Design

- **When appropriate, encouraging the use of "adaptive" trial designs that allow design modifications as information about drug response accumulates, leading to more efficient studies**
- **When appropriate, encouraging "enrichment" strategies to enroll patients more likely to respond to drugs under study, thereby reducing trial size and helping to direct drugs to patients who will benefit from them**
- **Allowing the use of a wide range of study designs, including single-arm studies, when patient populations are extremely small, as in some orphan diseases, and the natural history of the disease is well-characterized and the drug's beneficial effects are large**

Janet Woodcock, Congressional testimony
July 11, 2014

Targeted Therapies Progress

In the early 1990s, only 5 percent of FDA's new drug approvals were for targeted therapies. Twenty years later, that number had risen to a quarter of new approvals, and in 2013, 45 percent of FDA's approvals were for targeted therapies.

Randomly Controlled Parachute Trials

Parachute use to prevent death and major trauma related to gravitational challenge: systematic review of randomised controlled trials

Objectives: To determine whether parachutes are effective in preventing major trauma related to gravitational challenge.

Design: Systematic review of randomised controlled trials.

Data sources: Medline, Web of Science, Embase, and the Cochrane Library databases; appropriate internet sites and citation lists.

Study selection: Studies showing the effects of using a parachute during free fall.

Main outcome measure: Death or major trauma, defined as an injury severity score > 15.

Results: We were unable to identify any randomised controlled trials of parachute intervention.

Conclusions: As with many interventions intended to prevent ill health, the effectiveness of parachutes has not been subjected to rigorous evaluation by using randomised controlled trials. Advocates of evidence based medicine have criticised the adoption of interventions evaluated by using only observational data. We think that everyone might benefit if the most radical protagonists of evidence based medicine organised and participated in a double blind, randomised, placebo controlled, crossover trial of the parachute.

Gordon C.S. Smith. BMJ 2003; 327 doi: http://dx.doi.org/10.1136/bmj.327.7429.1459 (Published 18 December 2003)

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The **Personalized Medicine Coalition**, representing innovators, scientists, patients, providers and payers, promotes the understanding and adoption of personalized medicine concepts, services and products to benefit patients and the health system.

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