

New treatments better than standard ones just over half the time

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Benjamin Djulbegovic, M.D., Ph.D., a distinguished professor at the University of South Florida, has been studying the effectiveness of randomized clinical trials in evaluating the outcomes of new treatments for decades. Credit: University of South Florida

University of South Florida Distinguished Professor Benjamin Djulbegovic, MD, PhD, has studied the ethics of randomized clinical trials and their effectiveness in evaluating the outcomes of new treatments for decades.

Now, in a paper published Aug. 22 in the top journal *Nature*, Dr. Djulbegovic and colleagues report that on average new treatments work better than existing ones just over half the time. On scientific and ethical grounds, they say, the [randomized controlled trial](#) (RCT) system's little more than 50-50 success rate over the past half century is evidence that the system is working as intended.

The researchers analyzed 860 phase III published and unpublished RCTs performed by academic institutions or pharmaceutical companies. These [trials](#) collectively involved more than 350,000 patients.

"Our retrospective review of more than 50 years of [randomized trials](#) shows that they remain the 'indispensable ordeals' through which [biomedical researchers'](#) responsibility to patients and the public is manifested," the researchers conclude. "These trials may need tweak and polish, but they're not broken."

People who consent to participate RCTs are willing to be randomly allocated to new or existing treatments. While RCTs are considered the gold standard for comparing the effects of one treatment to another, the gradual progress they yield can seem frustratingly slow—particularly for patients with poor standard treatment options.

Yet, the genuine uncertainty associated with individual RCTs has been vital to the gains in therapeutics, said Dr. Djulbegovic, professor of medicine and oncology at the USF Health Morsani College of Medicine and Moffitt Cancer Center. If there was significant likelihood that one treatment in a comparison was better than the other, it would be

unethical to deny some patients the superior treatment, and well-informed patients would probably refuse to participate in the study, he said.

Incremental advances in treatment generated by RCTs over time – such as childhood leukemia cure rates moving from zero to 80 percent even though only 2 to 5 percent of new treatments provided a breakthrough – have translated into important improvements in health and lifespan, the authors say. However, they suggest trials could still benefit from more rigorous design, implementation and reporting –with widespread publication of trial results, including negative findings.

More information: "Medical research: Trial unpredictability yields predictable therapy gains;" Benjamin Djulbegovic, Ambuj Kumar, Paul Glasziou, Branko Miladinovic, and Iain Chalmers, *Nature*, August 22, 2013, pp 395-96. www.nature.com/nature/journal/.../63/full/500395a.html

Provided by University of South Florida

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