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Randomised controlled trials

In praise of human guinea pigs

Doctors use evidence when prescribing treatments. Policymakers should, too

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BEFORE the 20th century, the sick were wise to stay away from doctors. Medical treatments were often worthless and sometimes dangerous: “heroic measures” such as blood-letting and purging often killed. It was the discovery of micro-organisms, vaccines and antibiotics, that eventually equipped doctors with weapons that whacked diseases, not patients. But as many lives may have been saved by a more recent innovation, the randomised controlled trial (RCT). The idea is simple, yet brilliant. A new treatment is tested by giving it only to some randomly chosen patients, with the rest (the “controls”) receiving standard care. Before RCTs became common in the 1950s it was easy for a doctor to believe that patients who died did so despite his best efforts, while those who survived owed him their lives. He might harm patient after patient and never spot the pattern. Now almost all medicines are tested with RCTs before being widely prescribed.

What works in the fight against disease can also work in the fight against poverty. In the past decade aid organisations and governments in the developing world have rushed to make use of RCTs, encouraged by donors and philanthropists who like evidence that their cash will be spent well. This week two leading researchers released the results of RCTs for two schemes that gave destitute people assets (usually goats or a cow) and trained them to manage them (see article (http://www.economist.com/news/international/21679811-how-test-everything-sluggish-teenagers-corrupt-bureaucrats-measure-measure)). The results were impressive: in India recipients were much better off five years after the programme ended. More important, the trials showed that it really was the aid programmes that made the difference, and not some other factor.

RCTs have their limitations. They are impossible when an intervention affects everyone (for example, a change in interest rates) and unethical when it is known to be harmful (doctors who want to know just how unhealthy smoking is cannot ask human guinea pigs to light up). But the biggest problem with RCTs is that they are not used nearly often enough.

Even as policymakers in developing countries harness the power of randomisation, those in rich countries resist—especially for their most cherished schemes. About 100 education-related RCTs are under way in Britain, mostly co-ordinated by the Education Endowment Foundation, a charity set up by the government. But they deal with small-bore questions, such as whether teenagers learn more if the school day starts later. Meanwhile the government is radically reshaping the management and funding of schools nationwide—without testing the changes first, let alone running trials. That is reckless.

Other countries have done a bit better. An RCT run decades ago informed the design of America’s main safety-net housing programme. More recently, experiments have tested the impact of smaller classes, charter schools, sex education and pre-school for poor children. But just as in Britain, RCTs are rarely used to evaluate big policy shifts. The Affordable Care Act (Obamacare) could have been an opportunity for a series of trials to optimise its rules. That opportunity was missed.

The electoral cycle is one reason politicians shun RCTs. Rigorous evaluation of a new policy often takes years; reformers want results before the next election. Most politicians are already convinced of the wisdom of their plans and see little point in spending time and money to be proved right. Sometimes they may not care whether a policy works, as long as they are seen to be doing something. Stiff prison sentences make a government look tough even if they do not cut crime; very high taxes on top earners may be popular even if they raise no extra cash.

Doctors, at least, generally want to do some good. Even so, they were slow to embrace RCTs. Many bristled at what they saw as an aspersion cast on their professional judgment, or worried about the ethics of denying randomly selected patients a promising new treatment. They were won round only by seeing many established treatments proved to be harmful, and supposedly promising new drugs proved to be useless. Now doctors regard RCTs as the gold standard of evidence.

Heroic policymaking

To live in a modern democracy is to be experimented on by policymakers from cradle to grave. Education is intended to mould an upstanding future citizen; a prison sentence, to reshape someone who has gone astray. But without evidence, those setting policy for schools and prisons are little better than a doctor relying on leeches and bloodletting. Citizens, as much as patients, deserve to know that the treatments they endure do actually work.

From the print edition: Leaders