How Unproven Assumptions Become Common Health Care Practice

By AUSTIN FRAKT

When your doctor gives you health advice, and your insurer pays for the recommended treatment, you probably presume it's based on solid evidence. But a great deal of clinical practice that's covered by private insurers and public programs isn't.

The British Medical Journal sifted through the evidence for thousands of medical treatments to assess which are beneficial and which aren't. According to the analysis, there is evidence of some benefit for just over 40 percent of them. Only 3 percent are ineffective or harmful; a further 6 percent are unlikely to be helpful. But a whopping 50 percent are of unknown effectiveness. We haven't done the studies.

Sometimes uncertain and experimental treatments are warranted; patients may even welcome them. When there is no known cure for a fatal or severely debilitating health condition, trying something uncertain — as evidence is gathered — is a reasonable approach, provided the patient is informed and consents.

"We have lots of effective treatments, many of which were originally experimental," said Dr. Jason H. Wasfy, an assistant professor of medicine at Harvard Medical School and a cardiologist at Massachusetts General Hospital. "But not every experimental treatment ends up effective, and many aren't better than existing alternatives. It's important to collect and analyze the evidence so we can stop doing things that don't work to minimize patient harm."

In many cases, routinely delivered treatments aren't rigorously tested for years. Benefits are assumed, harms ignored.

This might have killed George Washington. At 67 years old and a few months shy of three years after his presidency, Washington reportedly awoke short of breath, with a sore throat, and soon developed a fever. Over the next 12 hours, doctors drained 40 percent of his blood, among other questionable treatments. Then he died.

Washington surely had a serious illness. Theories include croup, diphtheria, pneumonia and acute bacterial epiglottitis. Whatever it was, bloodletting did little but cause additional misery, and most likely hastened his death.

Though the procedure was common at the time for a variety of ailments, its benefits were based on theory, not rigorous evidence. In the era of modern medicine, this may strike some as primitive and ignorant.

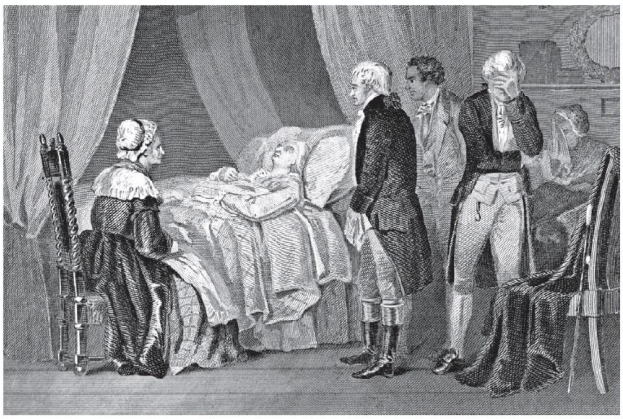
Yet, hundreds of years later, the same thing still happens (though fortunately not with bloodletting).

In the late 1970s, some doctors thought they had found a way to treat breast cancer patients with what would otherwise be lethal doses of chemotherapy. The approach involved harvesting bone marrow stem cells from the patients before treatment and reintroducing them afterward.

Fueled by encouraging comments from doctors, the 1980s news media reported higher chemotherapy doses as the means to survival. Yet there was no compelling evidence that bone marrow transplants protected patients.

But, told they would, many patients fought insurers in court to get them. Under pressure from Congress, in 1994 all health plans for federal workers were required to cover the treatment. Yet not a single randomized trial had been done.

Finally, in 1995, the first randomized trial was published, with impressive results: Half of women who received bone marrow transplants had no subsequent evidence of a tumor, compared with just 4 percent in the control group. But these results didn't hold up, with four subsequent clinical trials contradicting them. The approach was recognized for what it was: ineffective at best, lethal at worst.



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A depiction of George Washington on his deathbed. Doctors treated him for a fever by draining 40 percent of his blood, a common technique at the time that may actually have hastened his death.

Wishful thinking that runs ahead of or goes against research findings is behind today's opioid epidemic, too. Despite a lack of solid evidence, for years many believed that modern opioid medications were not addictive. It's now abundantly clear they are. But the damage is done.

There are countless other examples of common treatments and medical advice provided without good evidence: magnesium supplements for leg cramps; oxygen therapy for acute myocardial infarction; IV saline for certain kidney disease patients; the avoidance of peanuts to prevent allergies in children; many knee and spine operations; tight blood sugar control in critically ill patients; clear liquid diets before colonoscopies; bed rest to prevent preterm birth; the prescribing of unnecessary medications, to list just a few. In some of these cases, there is even evidence of harm.

It is not uncommon for newer evidence to contradict what had been standard practice. A study by an Oregon Health & Science University School of Medicine physician, Vinay Prasad, and colleagues examined 363 articles in the New England Journal of Medicine from 2001 to 2010 that addressed an existing medical practice. Forty percent of the articles found the existing practice to be ineffective or harmful.

Some of these reversals are well known. For example, three articles contradicted hormone replacement therapy for postmenopausal women. Another three reported increased risk of heart attacks and strokes from the painkiller Vioxx.

Looked at one way, medical reversals like these reflect a failure; we didn't gather enough evidence before a practice became commonplace. But in another way, they were at least a partial success: Science eventually caught up with practice. That doesn't always happen.

"Only a fraction of unproven medical practice is reassessed," said Dr. Prasad, who is co-author of a book on medical reversals, along with Adam Cifu, a University of Chicago physician.

Dr. Prasad's work is part of a growing movement to identify harmful and wasteful care and purge it from health care systems. The American Board of Internal Medicine's Choosing Wisely campaign identifies five practices in each of dozens of clinical specialties that lack evidence, cause harm, or for which better approaches exist. The organization that assessed the value of treatments in England has identified more than 800 practices that officials there feel should not be delivered.

It's an uphill battle. Even when we learn something doesn't make us better, it's hard to get the system to stop doing it. It takes years or even decades to reverse medical convention. Some practitioners cling to weak evidence of effectiveness even when strong evidence of lack of effectiveness exists.

This is not unique to clinical medicine. It exists in health policy, too. Much of what we do lacks evidence; and even when evidence mounts that a policy is ineffective, our political system often caters to invested stakeholders who benefit from it.

An honest assessment of the state of science behind clinical practice and health policy is humbling. Though many things we do and pay for are effective, there is a lot we don't know. That's inevitable. What isn't inevitable — and where the real problems lie — is assuming, without evidence, that something works.