



Comparative Effectiveness Research: Foundational to Healthcare Value Efforts

A high-value, patient-centered healthcare system that is equitable, efficient and produces uniformly high health outcomes requires good evidence. Comparative effectiveness research (CER) provides a key piece of evidence by comparing the effectiveness of alternative medical treatments and thereby helping providers, payers and patients determine which courses of treatment are best.

Alarming, the majority of our care is not based on CER. In 2009, the Institute of Medicine estimated that more than half of the treatments delivered do not have clear evidence of effectiveness.¹ Similarly, Clinical Evidence, a project of the *British Medical Journal*, found that little was known about the effectiveness of nearly 50 percent of 3,000 medical treatments that had been the subject of randomized controlled trials (RCTs).²

Increased funding for CER, along with getting study results into practice, could lead to more effective use of our healthcare dollars. More clarity about which treatments work best—and for which types of patients—could create potential for shifting money to those interventions and

away from less effective treatments. But better funding for CER is not enough on its own—increasing both the adoption of CER in clinical practice and the dissemination of results is critical to reducing waste, slowing spending growth and improving outcomes.

What is Comparative Effectiveness Research?

Comparative effectiveness research answers questions about the effectiveness of alternative medical treatments and can take many forms, such as:

- systematic reviews of the literature;
- creation of large research databases;
- prospective registries and cohort studies; and
- randomized controlled trials.

In addition to comparative effectiveness data obtained from trials, researchers are also using real world evidence (RWE) to compare the effects of different interventions. This data is generally collected in electronic health records (EHRs) and can offer insights on how treatments perform in different patients. For example, one such study on obesity used RWE to compare gastric bypass, sleeve gastrectomy and adjustable gastric band procedures, in addition to trial data.³

RWE may be a good complement to RCTs, especially because patients enrolled in RCTs are usually healthier and younger compared to the general patient population. RWE analyses using patient registries and administrative data can also be performed at low cost compared to data collected in clinical settings.⁴ Additionally, socioeconomically disadvantaged patients and racial and ethnic minorities are underrepresented in RCTs, whereas data collected from EHRs represents a more diverse set of patients.

SUMMARY

Comparative effectiveness research (CER) helps providers, payers and patients compare the effectiveness of alternative medical treatments to help them determine which courses of treatment are best. Despite being a promising strategy to reduce waste, slow spending growth and improve outcomes, the majority of our care is not based on CER. Increased funding for CER, along with getting study results into practice, could lead to more effective use of our healthcare dollars.

Experts believe using RWE may help to close the “efficacy-effectiveness gap,” which reflects the difference between an intervention’s effects in RCTs and real-world practice.⁵ However, hurdles to using RWE in CER remain. Though EHR adoption has increased, lack of interoperability, or ability to exchange data with and use data from other systems, is preventing patient data from flowing across research and care settings.⁶

Lack of CER Undermines Healthcare Value Approaches

Our health system is steadily moving to incentivize quality and improve equity. Lack of clear treatment evidence undermines our ability to improve how our health system works—contributing to treatment variation, waste and a wide disparity in costs and outcomes across the country. Reliable CER is foundational to key activities designed to make our health system work better, such as:

- creating evidence-based guidelines for providers;
- drafting decision aids to assist with patient and provider shared decision-making;⁷
- setting up payment rules under value-based provider payment approaches;⁸
- designing health plan benefits that adhere to value-based benefit design principles;⁹ and
- measuring provider quality.

Rising Investments in CER

In 2008, the U.S. devoted just 1 percent of healthcare spending to learning what works best, for whom and under what circumstances.¹⁰ By way of comparison, 10 percent of healthcare spending may be spending for overtreatment and low-value care.¹¹

For nearly two decades, spending on CER has been increasing, but not enough to rein in wasteful healthcare spending. In 2003, the Medicare Prescription Drug, Improvement, and Modernization Act expanded the Agency for Healthcare Research Quality’s (AHRQ’s) responsibility to conduct CER by creating the Effective Health Care Program.¹² In 2009, the Institute of Medicine released a major report detailing priorities for CER. Later that year, the American

Recovery and Reinvestment Act (ARRA) authorized \$1.1 billion to fund CER.¹³ In 2010, Congress authorized the Patient Centered Outcomes Research Institute (PCORI) to fund CER that engages patients and other stakeholders throughout the research process as part of the Affordable Care Act (ACA). According to the authorizing legislation, “The purpose of the Institute is to assist patients, clinicians, purchasers, and policy-makers in making informed health decisions by advancing the quality and relevance of evidence concerning the manner in which diseases, disorders, and other health conditions can effectively and appropriately be prevented, diagnosed, treated, monitored, and managed through research and evidence synthesis.” As of 2020, PCORI had invested nearly \$2.6 billion in more than 700 patient-centered CER studies¹⁴ (see box below). Though the U.S. has increased its investment in CER, research alone is not enough to mitigate waste, limit cost growth and improve outcomes.

EXAMPLE

One PCORI-funded study on diabetes treatments found no statistically significant differences between type 2 noninsulin-treated diabetics who performed self-monitoring and those who did not.¹⁵ Over five years, discontinuing self-monitoring in this population would save more than \$12 billion in healthcare costs.¹⁶ However, these savings depend on all eligible patients not testing their blood sugar daily.

Incentivizing Adoption of CER

Undertaking CER alone does not necessarily save money or lead to better outcomes. Even with an evidence-base in place, it can sometimes take upwards of 17 years to get study results into practice¹⁷ (see box below). Studies have shown that disseminating results via clinical practice guidelines led to initial increases in utilization of effective therapies. However, less effective treatments did not replace more effective ones as the standard of care, potentially indicating a need for financial and non-financial provider incentives.¹⁸

Even simple protocols like requiring physician justification of medical necessity or creating checklists to

EXAMPLE

One RCT comparing diuretics, Angiotensin Converting Enzyme (ACE) inhibitors, calcium channel blockers and alpha blockers for the treatment of hypertension found that diuretics were more effective than alternative treatments, in addition to being less expensive. Sadly, these findings had very little effect on prescribing patterns.¹⁹

remind providers to prescribe certain medications can have a significant impact on outcomes. Intermountain Healthcare implemented a checklist recommending that physicians provide a specific type of heart medication after CER studies revealed effectiveness. This simple protocol reduced deaths from congestive heart failure by 23 percent and saved \$3.5 million a year.²⁰

Financial Incentives

Decisions about coverage, benefit design and provider payment can influence the pace of adoption. The secret may lie in changing the way we pay for care (see box below). For example, payers could offer bonus payment to providers who deliver clinically effective treatments.²¹ Other strategies rely on coverage determinations, like using step therapy to encourage the use of certain therapies over others or value-based insurance designs that limit coverage or increase cost sharing for therapies that have not demonstrated clinical benefit. However, critics warn that these strategies could be seen as limiting access to care.²²

In addition to changing the scope of covered treatments, payers can change reimbursement policies using CER findings. For example, if a treatment produces evidence of superior clinical effectiveness, Medicare could pay providers based on usual pricing, while ones that produce insufficient evidence could be paid via dynamic pricing. In other words, payments would be set according to the current cost-plus reimbursement formulas, which involve predetermined margins, and are reassessed after three years. If the treatment was still unable to demonstrate

clinical advantages, payment would be lowered to Medicare reimbursement rates for a relevant alternative option.²³

For example, after Medicare set higher reimbursement rates for intensity-modulated radiation therapy than three-dimensional therapy, providers around the country abandoned conventional three-dimensional therapy.²⁴

EXAMPLE

Physician engagement and financial incentives led to the elimination of early elective birth inductions (before week 39 of a pregnancy) after the American College of Obstetricians and Gynecology found that early inductions lead to poor outcomes, including increases in neonatal intensive care unit (NICU) admissions and ventilator usage. Intermountain worked with SelectHealth to cease paying for non-medically indicated inductions prior to 39 weeks. Clinical leaders held meetings to garner support for the goal to eliminate all early elective inductions. SelectHealth also created a program for new mothers focused on providing prenatal help and education.²⁵ As a result, early elective inductions dropped from 28 percent of all elective inductions to zero percent and resulted in shorter labor, fewer C-sections and cost savings of \$2.5 million a year.

Non-Financial Incentives

Non-financial incentives may also drive adoption of CER results. These can focus on peer comparisons, peer recognition, eliminating barriers and providing institutional support and leadership.²⁶ Educating providers and medical students is key to getting CER results into practice.

CER can highlight services that might be better for certain patients, leading to a more personalized approach. However, the treatment decision is not the provider's alone to make. Patient shared decision-making (PSDM) is a process that goes beyond traditional informed consent in healthcare—it is an interpersonal,

interdependent process in which healthcare providers and patients collaborate to make decisions about the care that patients receive. Shared decision-making not only reflects medical evidence and providers' clinical expertise, but also the unique preferences and values of patients and their families. There is strong evidence that PSDM improves outcomes and increases patient and physician satisfaction, and should become the standard of care.²⁷ However, for patients to be involved in their care decisions, there needs to be evidence comparing the effectiveness of treatments and data on how a specific patient might respond to different treatments.²⁸

Effective take-up of CER findings can go beyond provider- and patient-focused efforts. Research from the Alliance of Community Health Plans (ACHP) found the collaboration between health plans, physicians and communities sped up the adoption of evidence-based care. ACHP has highlighted best practices to accelerate uptake.²⁹

Conclusion

Insufficient investments in comparative effectiveness research undermine our nation's efforts to produce better value and more equitable outcomes from our healthcare system. Evidence about which treatments work best—and for which types of patients—provide the foundation for our value-based provider payment efforts, patient-shared decision-making, quality measurement and much more.

Increased, targeted investments in CER are essential to achieving a high-value, patient-centered healthcare system. It is likely these investments will “pay us back” in terms of future savings and better outcomes. However, CER's impact also depends, in large part, on getting the results into provider treatment and prescribing practices. Fortunately, research highlighting strategies that lead to the effective dissemination of CER results can guide the way. A variety of financial and non-financial incentives can be used to influence provider behavior and promote the adoption of evidence-based care.

Notes

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