The idea seemed transformative. The Affordable Care Act would fund a new research outfit evocatively named the Innovation Center to discover how to most effectively deliver health care, with $10 billion to spend over a decade.

But now that the center has gotten started, many researchers and economists are disturbed that it is not using randomized clinical trials, the rigorous method that is widely considered the gold standard in medical and social science research. Such trials have long been required to prove the efficacy of medicines, and similarly designed studies have guided efforts to reform welfare-to-work, education and criminal justice programs.

But they have rarely been used to guide health care policy — and experts say the center is now squandering a crucial opportunity to develop the evidence needed to retool the nation’s troubled health care system in a period of rapid and fundamental change.

“It’s the greatest irony,” said Gordon Berlin, president of MDRC, a nonprofit organization whose studies have influenced American policies on welfare, job training and education. In health care, of all areas, he said, in which every group that evaluates medical evidence ranks such studies as the most
reliable by far, they have rarely been used.

“The results,” Mr. Berlin said, “speak volumes about the path not taken — an extraordinary body of evidence has been built to inform welfare policy and practice while we have only just begun the process of learning what works in health policy.”

The studies that are regarded as the most reliable randomly assign people or institutions to participate in a program or to go on as usual, and then compare outcomes for the two groups to see if the intervention had an effect.

Instead, the Innovation Center has so far mostly undertaken demonstration projects; about 40 of them are now underway. Those projects test an idea, like a new payment system that might encourage better medical care — with all of a study’s participants, and then rely on mathematical modeling to judge the results.

Dr. Patrick Conway, the director of the center, defended its reliance on demonstration projects, saying they allowed researchers to evaluate programs in the real world and regularly adapt them. “Does it look like it is working?” he asked. “If it does not look like it is working, we can stop.”

He said that the center has had trouble getting such studies to yield solid results because those in the control groups — who do not get the innovation being tested — tend to drop out.

“We will use randomized designs when we can and when it’s appropriate,” he said.

Centers for Medicare and Medicaid Services, which houses the center, spends about $3.7 billion year on research and has the authority to implement programs that work on a national basis.

Researchers say the Innovation Center needs to do more randomized trials and fewer demonstration projects. And they note that the results of the few rigorous trials done in years past have rarely been implemented.

“Until we do these kinds of studies we don’t have evidence,” said William Savedoff, a senior fellow at
the Center for Global Development, a nonprofit group that advocates for rigorous evaluations.

The history of randomized trials in social policy has tended to follow a particular trajectory, said Robert F. Boruch, a professor in the departments of education and statistics at the University of Pennsylvania’s Wharton School. “One of the typical events is a trial that serves as a benchmark, a precedent to show these things are useful,” he said, although it is often years or decades after the first clinical trial before more randomized studies are initiated on a regular basis.

In criminal justice, for example, the Manhattan Bail Bond Experiment in the early 1960s was the pathbreaker. It randomly required poor defendants to post bail or not, and found that requiring bail did not affect the rate at which they appeared for trials.

In education, a randomized study in Tennessee in the 1980s found that smaller class sizes in the early grades resulted in lasting academic improvement.

In welfare, experiments in the 1970s found that guaranteeing incomes for the poor was at most only a slight disincentive for men to work. But that funding, which some called a negative income tax, was a disincentive for white and Hispanic women — though not black women — to work.

In health care, a seminal, large randomized study by the RAND Corporation in 1982 found that people used health care less, but that their health was not affected, when they had to pay a small amount — as compared with nothing — for doctor visits.

Since then, such rigorous studies in health care have been rare. But results from a new, large randomized study financed by the nonprofit National Bureau of Economic Research, among others, has upended a key assumption of the Affordable Care Act. It was thought that insured people would save the nation money because they would use emergency rooms less, but it has turned out that they have used them more.

Researchers acknowledge that not every question can be answered with a randomized study. Studies
that give everyone an intervention can move quickly and nimbly adjust when something does not work. And they can be hypothesis generators for more rigorous studies to follow.

The problem, critics say, is that the Innovation Center’s research is so one-sided.

“A lot of money is being spent,” said Jon Baron, president of the Coalition for Evidence-Based Policy, a nonpartisan group. But the Innovation Center’s approach, he added “is not going to provide credible answers.”

A four-year study funded by the center gives Medicare patients in North Carolina a variety of services, including coordination of care and help managing chronic diseases like diabetes. But there is no control group randomly assigned to the usual care, so the researchers will have to use mathematical modeling to see if the interventions save money and improve health.

Mr. Baron noted that randomized studies have often contradicted long-held assumptions based on demonstration projects.

For example, preliminary studies indicated that having a nurse call patients and help them manage multiple chronic conditions would save $3 to $7 for every dollar invested.

Then, in a rare move, Medicare tested the idea in 2005 with a randomized study involving 240,000 beneficiaries. On average, costs increased by 4 to 11 percent.

Even when a randomized study finds methods that work, results can languish.

That is what happened when Mary Naylor, a nursing professor at the University of Pennsylvania, and her colleagues tested the effects of having a trained nurse assist chronically ill older people in a hospital and once discharged. The help saved Medicare an average of $5,000 per patient within a year by avoiding repeat hospitalizations. She and her colleagues calculated that annual savings to Medicare if her findings were implemented nationwide could be $10 billion.

Dr. Naylor published her results in leading journals like the Journal of the American Medical
Association and Annals of Internal Medicine. She created and tested training tools that showed her results could be replicated.

“We tried to figure out how to make it happen.” Dr. Naylor said. But her efforts, over more than a decade, have been ignored nationally — just a few local medical systems have implemented the system and few insurers are paying for it.

The situation is different in the developing world. There, randomized trials have become common in health care and other areas, sponsored by a variety of groups like J-PAL, a global network of researchers that was organized by M.I.T. and Harvard economists.

So far, J-PAL has conducted over 440 randomized trials in 55 countries, according to Amy Finkelstein, an M.I.T. economist.

Dr. Finkelstein and Lawrence Katz, a Harvard economist, have now started J-PAL North America to spur randomized trials in, among other areas, health care.

Dr. Finkelstein was encouraged by the Medicaid study she and Katherine Baicker of Harvard did in Oregon. The state wanted to expand Medicaid coverage but could not afford to insure all, so it used a lottery.

The lottery was essentially randomizing people to have Medicaid or not, so Dr. Finkelstein and Dr. Baicker designed a study to see the effects.

Over 18 months, those who got Medicaid saw doctors and went to emergency rooms more often and got more health care. They were less depressed. But so far, their health is not better and they cost the system more.

“It is getting so much attention not just because it is important and credible,” said Dr. Finkelstein, “but because it is rare.”
Criticized In Group’s Health Policy Tests.