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Accelerating Medical Breakthroughs for American Patients and their Families

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Key Points:

- Rational and forward-thinking regulatory reforms can stimulate the uptake of new technologies that make good health more accessible and affordable.
- CMS has an opportunity to reform and focus Medicare's coverage with evidence development (CED) program to better achieve its goals of generating evidence while ensuring new technologies are covered.
- Medicare Advantage can be transformed into a laboratory for forward-thinking reforms that stimulate valuable investments in prevention by aligning payers', innovators', and patients' interests, serving as a model for the commercial insurance market.
- Finally, there is opportunity for prudent regulatory reform in healthcare price transparency that best serves the needs of today's patients and the generations of patients to come.

Chairman Burlison, Chairman Grothman, Ranking Member Frost, Ranking Member Krishnamoorthi, and Honorable Members of the Subcommittees, thank you for the opportunity to testify today about improving regulation to accelerate medical innovation.

My name is Darius Lakdawalla, and I am an economist, a professor at the USC Mann School of Pharmacy & Pharmaceutical Sciences and the USC Price School of Public Policy, and the Chief Scientific Officer at the USC Schaeffer Center for Health Policy & Economics. By way of background, I have been studying innovation in the health care sector for nearly three decades, I co-wrote the chapter in the *Handbook of Health Economics* on intellectual property and biomedical research, I co-authored the book *Valuing Health* on modern methods for valuing medical technology, and I am an elected member of the National Academy of Medicine. The opinions I offer today are my own and do not represent the views of the University of Southern California or the USC Schaeffer Center.

The Role of Regulation in Medical Progress

In 1958, the British scientist Sir James Black began researching ways to decrease the need for oxygen among patients with oxygen-depleting arterial disease. This research culminated in the discovery of propranolol, the world's first beta-blocker, which would revolutionize the treatment of cardiovascular disease in the decades to come and would yield for Sir James the 1988 Nobel Prize for Medicine. As early as 1965, propranolol was available to patients in Europe. However, despite its initial approval for a smaller arrhythmia indication in 1968, propranolol's widespread regulatory approval and clinical use in the United States would take much longer. As late as 1974, an editorial in the *Journal of the American Medical Association* complained about delays in FDA approval of propranolol for hypertension treatment and about American physicians' lack of access to novel cardiovascular drugs already in regular use within the United Kingdom. Only in 1976 did FDA approve propranolol as a hypertension treatment. The human costs of this delay became more apparent in the early 1980s, when the Medical Research Council (MRC) trial showed that propranolol treatment regimens lowered stroke risk by nearly 25% for non-smoking patients with even mild hypertension, and the NIH-funded Beta-Blocker Heart Attack Trial demonstrated that propranolol reduced mortality by nearly 30% among patients that had previously suffered a heart attack. USC Schaeffer research found that these benefits flowed disproportionately to the least educated households, which more often lacked the means to implement the complex diet and exercise regimens that were previously central to treating cardiovascular disease.

The story of propranolol was not an isolated one during this earlier era. From 1972 to 1987, new drugs were more than twice as likely to launch first overseas than in the US, with the crisis reaching a breaking point during the AIDS epidemic of the late 1980s. In response, the FDA pioneered a series of reforms, beginning with the "fast track" approval of the HIV drug AZT in 1987. More efficient regulatory pathways at the FDA coincided with retrenchment in approvals by our overseas peers, with increasing restrictions on reimbursement and access to new medicines. As a result of this turnabout, new drugs are now more likely to launch

first in the United States, with American patients the beneficiaries of forward-thinking regulatory reforms at home and shortsighted restrictions abroad.

This era in drug development illustrates both the potential chilling effects of regulation on innovation and the potential for regulatory reform that extends and improves lives. Today, we face a variety of new challenges to medical innovation and corresponding opportunities for pathbreaking regulatory reform that can help sustain continuing improvements in life expectancy and health for American patients.

Reforming Medicare's Coverage with Evidence Development Process

A case in point is Medicare's current coverage with evidence development (CED) policy. CED begins with a rational and appropriate goal. It was created to provide Medicare beneficiaries access to new technologies that CMS would otherwise judge to lack the evidence needed to meet its standard of "reasonable and necessary" care. In these cases, Medicare has a legitimate interest in calling for additional evidence, and CED aims to achieve this goal.

However, despite its name, the coverage with evidence development process limits both coverage and the development of much-needed evidence on the value of new technologies. In practice, CED creates disparities in coverage and access that ultimately hamper the development of new evidence and unfairly allocate the benefits of new technology.

Under the auspices of CED, CMS often limits coverage for new technologies in a bid to reduce their potentially unknown risks. To take one example, CMS imposed CED requirements on transcatheter aortic valve replacement (TAVR) technology when it first launched, stipulating that TAVR could only be performed at institutions with a center of excellence designation, judged to provide a requisite level of technology and expertise. Our research using CMS data revealed that centers of excellence were distributed unequally across the country, precluding access for patients in access-challenged parts of the country.[1] For instance, the proportion of rural patients treated at CED-qualified hospitals is 50% lower than the rate of rural patients at non-CED qualified hospitals. Similarly, CED-qualified hospitals treat fewer low-income subsidy (LIS)-enrolled patients and fewer patients residing in socioeconomically disadvantaged neighborhoods. Not only do these kinds of constraints reduce equitable access, but they also undermine a key goal of CED: to generate evidence on a nationally representative sample of clinically eligible patients. Instead, restricted coverage skews the use of technologies like TAVR to patients with ready access to academic medical centers and

[1] USC Schaeffer researchers began with a full list of CED decisions published electronically by CMS. For each decision, researchers reviewed CMS information reporting the associated CED clinical trials or registries and their associated clinicaltrials.gov sites (see, for example, the relevant site for TAVR) or registry-specific sites if applicable (see, for example, a registry site for TAVR). From the clinicaltrials.gov site, researchers gathered the full list of hospitals participating in the relevant clinical trials or registries. Researchers then linked the participating hospital sites to Medicare's list of all hospitals. This then allowed linkage to CMS data summarizing each hospital's discharges in 2024 ("Hospital Readmissions Reduction Program Supplemental Data File") to compare hospitals that participated in each CED with those that did not participate.

other high-end medical facilities. Moreover, additional Schaeffer research has shown that CED requirements have distorted markets and skewed TAVR adoption towards physician practices facing less competition: requiring substantial prior procedural volume and high upfront investments favored practices earning higher profits on larger market shares.

These kinds of restrictions are widespread. CMS has statutory authority to limit use of technology under a national coverage determination (NCD), and according to our research, there are over 300 active NCDs that limit coverage. Such restrictions will distort the applicability of the evidence that gets collected, unless they happen to limit use by every relevant subgroup of patients and by exactly the same proportional amount, a circumstance that seems unlikely. Moreover, these restrictions tend to persist. Fewer than 20% of the CED requirements issued between 2005 and 2023 have been retired. There is often a lack of clear guidance around what exactly constitutes sufficient evidence to satisfy the CED requirement.

Several steps can be taken to help CED achieve its stated goals. First, the agency can adopt and announce transparent criteria that govern when CED can be considered. This step requires a clear definition of CMS's "reasonable and necessary" standard, including specific guidance on the nature of evidence required to meet this standard. Second, coverage constraints under CED should be proportionate to their purpose—allowing access while addressing targeted evidence gaps. Access limits should focus only on the specific risk or uncertainty and reflect patients' tolerance for it. In the TAVR case, early stroke concerns prompted broad restrictions, though patient preference data indicated the risk was acceptable and the limits unlikely to improve safety. Third, every instance of CED should be accompanied by clear guidance on the evidence needed for the technology to exit the CED process. The recent notice on Transitional Coverage for Emerging Technologies (TCET) provides a model of a strategy for specifying the process for removing CED requirements from a technology.

Risk-Adjustment in the Medicare Advantage Program

CMS also faces opportunities to encourage private Medicare Advantage (MA) insurers to expand access to innovative technologies that prevent disease. The MA marketplace is well-positioned to serve as a laboratory for investments in long-term prevention, because MA insurers retain their beneficiaries for five to seven years. Contrast that with the typical commercial insurer, where beneficiary tenure may be only two to four years. Thus, MA insurers can afford to make preventive investments with longer-term payoffs. However, CMS's current approach to risk-adjustment weakens or sometimes even eliminates incentives for long-term prevention.

Like CED, CMS risk-adjustment begins with a logical rationale: sicker patients cost more to insure, and without higher premiums as incentives, insurers would seek to avoid covering them. Thus, the sickest beneficiaries would be the ones to remain in the traditional Medicare program, leaving taxpayers to cover their costs. This phenomenon, known to economists as

"adverse selection," favored MA and penalized Traditional Medicare for several decades before premiums were aligned with beneficiary illness via risk-adjustment.

Nonetheless, risk-adjusted premiums feature a well-known downside: they discourage prevention. To illustrate with a simple example, suppose beneficiaries with type-2 diabetes cost on average $5000 extra to insure every year. If an insurer makes an investment in preventing one case of diabetes, it ordinarily stands to gain $5000 annually from that proactive investment. With accurate risk-adjustment, however, the net gain is zero on average, because the insurer also loses an incremental premium increase worth $5000. This trade-off can ultimately deprive patients of evidence-based preventive technologies. For instance, academic research shows that including pneumonia in MA risk-adjustment calculations coincided with reduced influenza vaccination for beneficiaries.

The stakes around prevention are even higher in the case of novel GLP-1 drugs to treat obesity and overweight. USC Schaeffer research demonstrates that investments in these drugs for clinically eligible patients pay substantial societal returns in the form of cost-savings to Medicare, along with longer and healthier lives for Medicare beneficiaries. All told, our analysis shows that the social internal rate of return (IRR) from treating obesity with novel GLP-1s is greater than 13% for all eligible patients, due to medical costs avoided and health improvements from the prevention of diabetes, cardiovascular disease, and other chronic illness.[2] As context, 13% annual returns meet or exceed other valuable private and public investments, including the 7% annualized return on the S&P 500 between 2000 and 2024 and the 13% annual return on investments into early childhood education programs for disadvantaged children.

Our research proves the value of investing in obesity treatments for the Medicare population and the wisdom of expanding access through initiatives like the Medicare coverage expansion recently announced by the Trump Administration. However, successful implementation will require a framework that encourages MA insurers to provide preventive treatments to beneficiaries—whether within the context of a CMS demonstration or more broadly. A variety of options have been proposed that merit careful consideration. First, as USC Schaeffer research has proposed, CMS could allow multiyear enrollment in MA plans, with premiums fixed over the enrollment period and set to reflect average growth in costs, or even somewhat below average growth in costs. Thus, insurers are encouraged to make

[2] Our paper titled, "Lifetime Social Returns From Expanding Access to Anti-obesity Medications" reports the 30-year social IRR for expanding access to all eligible adults over the age of 25 years. However, supplemental subgroup analysis from the project shows that the IRR for Medicare age adults 65-74 years ranges from 15-31% over 20-years depending on patients' BMI range at treatment initiation. Similarly, the 30-year social IRR for patients age 45-64 ranges from 16-23% across the same BMI categories, and ranges from 15-17% for the youngest patients age 25-44 year. Although younger patients tend to accrue greater health gains from treatment, they also accrue more years of treatment. The IRR calculations take this tradeoff into account.

investments that lower cost growth for their multiyear enrollees. These changes could be complemented by reforms to the rules around Alternative Payment Models (APMs) like Accountable Care Organizations (ACOs). Currently, provider groups are rewarded for savings only over 12-month horizons. Lengthening the payback period would align their incentives with the health of beneficiaries and with insurers enrolling beneficiaries over longer horizons.

Building a More Efficient Healthcare Ecosystem

An additional opportunity for meaningful regulatory reform lies in healthcare price transparency. Despite important progress on surprise billing, healthcare pricing remains frustratingly opaque. Nearly every American can attest to the difficulty of understanding healthcare prices and the cost consequences of alternative treatment choices. Even large employers find it difficult to learn how much they are paying for medical technologies, no matter how widely used. Basic economics implies that consumers, physicians, and employers cannot make efficient decisions when they cannot see the real costs of their choices. Imagine taking your weekly grocery trip to a store with all the price tags removed and being obliged to pay a single, aggregated credit card charge at the end of your visit, without any insight into its components. Such a system would reward suppliers for raising prices and frustrate consumers seeking to make good choices for their families.

This is more than just a thought experiment. Schaeffer Center research demonstrates that healthcare systems profit from complex and opaque pricing rules. Recent federal guidance on improving transparency in negotiated hospital prices is a step in the right direction. The focus of regulators should be on making negotiated rates transparent to the firms and individuals ultimately paying those rates, namely consumers and employers. In the past, firms and consumers have been able to observe, at best, what amount to "sticker prices" in the form of hospital and physician charges, pharmaceutical list prices, and the like. Imagine the process of buying a car without ever knowing what discounts each dealer would offer before you have to decide where to purchase.

Some academics and federal agencies have pushed back against calls for price transparency, asserting[3] that it provides a means for pharmaceutical firms, hospitals, physician groups, and other suppliers to cooperate with each other in raising prices. This argument is specious, proceeding from a flawed analogy to a 20 year-old study of a failed price transparency policy in the Danish ready-mix concrete industry. Moreover, this criticism also rests on the inaccurate premise that confidential healthcare pricing has yielded vigorous price-competition that benefits consumers and employers. On the contrary, when consumers and employers cannot see real transaction prices, they cannot push back by deserting higher-priced suppliers for lower-priced alternatives. Indeed, USC Schaeffer research provides evidence suggesting this dynamic in the branded pharmaceutical industry, where

[3] See page 362.

competition may be associated with higher—not lower—list prices for drugs, and correspondingly higher costs for patients paying co-insurance for their medicines.

While confidential pricing is common in business-to-business transactions, it becomes uniquely distortionary in the healthcare marketplace. When a local hardware store gets a better deal on nail guns, shoppers do not need to see the price the store negotiated with their upstream suppliers. Rather, they can be reasonably confident that the price on the shelf will fall, and even if it does not, they can readily observe the price they are asked to pay. In healthcare, however, the out-of-pocket costs of patients and the aggregated payments made by employers mask the prices of individual services and technologies, making it nearly impossible to craft an efficient portfolio of choices.

This is not an easy problem to solve. A small body of evidence suggests that price transparency may help reduce negotiated healthcare prices, but significant questions remain. In theory, buyers with market-leading discounts may lose them, resulting in higher prices. However, inaction is not the right response to this uncertainty. A prudent, deliberate path forward is more helpful. For instance, reporting average product and service prices by geographic locale provides consumers and employers with useful guidance on how to allocate their healthcare dollars while limiting the disclosure of sensitive commercial data. Alternatively, CMMI can pilot price transparency programs in a handful of key product or service areas to learn how markets, costs, and health outcomes adjust. Transparency programs must also be complemented by strategies to make data useful and accessible to patients and other stakeholders. For instance, the 2022 health plan price transparency rule could be enhanced by requirements that harmonize reporting standards and otherwise ensure ease of access and interpretation.

Clearing a Path for Medical Technology

Medical technology has produced remarkable gains in life expectancy and in the quality of life over the past century, but the most advanced technology means little if sick patients in need cannot access it. Rational and forward-thinking regulatory reforms can stimulate and enable the uptake of new technologies that make good health more accessible and affordable. CMS has the tools to restore both coverage and evidence-development to the center of its CED program. Similarly, Medicare Advantage can lead the way forward in stimulating investments in prevention that disseminate to the rest of the healthcare system. And, prudent steps to ensure price transparency may help us forge a more efficient and patient-centered healthcare system for today's patients and the generations of patients to come.