Committee on Energy and Commerce

Subcommittee on Health

Hearing on "Innovation Saves Lives: Evaluating Medicare Coverage Pathways for Innovative Drugs, Medical Devices, and Technology"

Tuesday, July 18, 2023, at 10:30 a.m.

2232 Rayburn House Office Building

Good morning, Chairman Guthrie, Ranking Member Eshoo, Vice Chair Bucshon and distinguished members of the Committee. I am Todd Brinton, Chief Scientific Officer, and head of Advanced Technology for Edwards Lifesciences. Edwards is the global leader of patient-focused innovations for structural heart disease and critical care monitoring. On behalf of Edwards, I would like to applaud the Committee for recognizing the importance of addressing the need to provide patients with more timely access to lifesaving technologies.

While I am here on behalf of Edwards, I'd also like to share a bit about my life's work helping patients and my own passion for innovation. I started my career as a biomedical engineer, following in my father and grandfather's engineering footsteps. However, I was soon drawn to medical school and a career in interventional cardiology, including 14 years on faculty of Stanford University as an interventional cardiologist and Clinical Professor of Medicine.

During my time at Stanford, I saw first-hand many patients with life threatening conditions who had no choice but to wait and hope for new innovations to save their lives. I realized that, by combining my passions for innovation and caring for patients "at the bedside," I could help develop technologies to improve millions of lives, more than the individual patients I encountered in the hospital or clinic. So, it is my hope that by helping shape this critical public policy initiative, we can improve the lives of millions more.

While at Stanford, I completed additional training in medical technology innovation at the Stanford Byers Center for Biodesign, and later led the graduate student and fellowship programs. I founded several medical device companies and served as an advisor or board member for others. It is this work that led me to join Edwards four and a half years ago. My blended clinical and innovation experience over the past 30 years has taught me the critical need for cutting-edge technologies and how it can improve the lives of countless patients.

My passion for innovation and patients has found a home at Edwards Lifesciences. At Edwards, we are deeply committed to delivering lifesaving innovations to patients, and to do so, we invest heavily in Research and Development. In fact, 17% of our revenue, on average more than twice the investment of those in our industry, goes back into R&D, so we can fuel the next generation of medical technologies to improve the lives of people fighting cardiovascular disease. As a result, Edwards' technologies often emerge as first-to market-therapies, many of which address significant unmet clinical needs for Medicare patients.

I continue to be energized by the process of innovation and finding solutions for patients like Jill, a 66-year-old registered nurse from Washington state who suffered from heart failure. Edwards' innovation was there for Jill when she was told there were no treatment options for her condition. I'll talk more about Jill later.

At Edwards, we are committed to innovation that can serve patients who have either no therapeutic options or unsatisfactory options. Streamlining the process to make coverage of breakthrough medical technologies more transparent and efficient is one way that we can ensure that medical technology innovation flourishes, rather than languishes. That is why today's hearing is so important.

Innovation is a powerful process and iterative force; it is not an end point. It is where we, at Edwards, find our passion to keep finding better solutions to help very ill patients with structural heart disease. Our belief in the innovation process is paired with our commitment to evidence development. Evidence allows us to better understand how innovative therapies can and should evolve over time and how we can help further improve outcomes for patients.

This evolution underscores how medical device technology and pharmaceuticals are different. Simply stated: once drugs are approved, they do not change. The manufacturer is responsible for replicating and packaging the drug, and the patient is administered the drug out of the package. For drugs, access and compliance represent some of the most significant variables impacting patients' outcomes, assuming the drug is made available to a well-defined patient cohort.

Conversely, for medical technology like our transcatheter heart valve therapies, we are constantly learning, which allows us to improve the technology and the procedures associated with intervention. When clinicians use medical technology, they learn and improve their skills over time and collect data on how to optimize patient outcomes. As an interventionalist, an engineer, and an innovator myself, I would argue we *need* that evidence to help us innovate for patients with unmet needs.

The Edwards experience with the development of an innovative heart valve replacement therapy known as transcatheter aortic valve replacement (TAVR), which enables a heart team to replace a patient's aortic heart valve without open-heart surgery, has provided us a unique perspective on coverage of innovative technologies. Over many years and multiple clinical studies, we have navigated the regulatory channels to bring this therapy to US patients as well

as patients around the world. Creating novel therapies involves significant challenges, and we are grateful for the commitment of thoughtful leaders at the Food & Drug Administration (FDA) and the Centers for Medicare & Medicaid Services (CMS) who worked with us to bring TAVR here. Together, we prioritized patients and embraced opportunities for better stakeholder collaboration with the agencies. Because of this paradigm-shifting experience, we believe there is still untapped potential to improve the Medicare coverage process, shortening the time between FDA approval and CMS coverage while also ensuring that patients receive reasonable and necessary care that is both safe and effective.

Edwards is committed to developing robust clinical evidence to help patients with structural heart disease, particularly those who currently have no treatment options. Because of our experience with TAVR, Edwards believes that a well-designed transitional coverage program could allow Medicare beneficiaries access to cutting edge medical innovations while ensuring that those innovations are used appropriately. Although TAVR was not designated as a breakthrough pathway technology by FDA (the program did not exist when it was approved, and, therefore, would not have been considered for “transitional” coverage), our experience with the approval and coverage of the first TAVR therapy approved for patients in the US does offer insight into how CMS might use a transitional coverage process to balance these sometimes-competing priorities.

Edwards worked with FDA, CMS, the medical and patient communities, and industry in the transcatheter space to manage the introduction of this technology to the US market and ensure that it was being used responsibly and safely. CMS crafted a coverage policy that recognized the need for a heart team to evaluate and treat TAVR patients and invoked “coverage with evidence development” to allow us to work with providers to collect necessary data aligned with the FDA post-market requirements.

While we continue to be concerned about the burden of the number of data fields in the patient registry, we believe the TAVR experience overall was a success story, highlighting the importance of FDA, CMS, and stakeholder collaboration. This was designed to help generate meaningful evidence that ultimately helped us to support continuous innovation and advancement in TAVR technology and continue to expand access to more patients.

It is through this data collection that we are able to identify disparities in patient access to transcatheter heart valve interventions – particularly in rural areas and other underrepresented communities whose hospitals do not have the resources to meet the site and operator requirements in the NCD for a TAVR program. We remain committed to ensuring that all patients have equitable access to transcatheter valve procedures in the future. We look forward to working with the members of this Committee to help address system-wide disparities in access to high quality healthcare. TAVR stands as a powerful example of the importance of ongoing, real-world data collection and the efficiencies in the regulatory process that can be gained when we all work together to measure performance and outcomes in new patient populations.

Transitional Coverage for Emerging Technologies (TCET)

CMS issued a proposed procedural notice for Transitional Coverage for Emerging Technologies (TCET) for FDA designated Breakthrough Devices on June 22, 2023. This guidance is a positive step forward. The proposed TCET guidance holds promise to address the needs of patients through access to breakthrough therapies. Innovative companies like Edwards will embrace the policy elements that enable direct CMS engagement while on the pathway to FDA

approval. We support a transitional coverage pathway that will ensure patients have access to breakthrough lifesaving and life-enhancing therapies that are not available to them today.

According to research from Stanford Biodesign, it takes an average of five years for medical technologies to achieve nationwide coding, coverage, and payment following FDA approval. CMS has the authority to call for additional studies and datapoints to justify its assessment that a new product is reasonable and necessary. These studies can prolong the coverage process under normal circumstances, meaning that an FDA-approved breakthrough device would still be subject to those CMS studies without a TCET option, leaving patients without access to cutting-edge treatments in situations where there are no other options available.

CMS has an important role in identifying key questions that remain after breakthrough-designated technologies are FDA-approved and, when necessary, ensure that essential data are collected. The implementation of a voluntary, timely, and predictable coverage process will allow CMS to achieve its goal of providing Medicare coverage for innovative technologies while ensuring CMS covers items and services based on scientifically sound clinical evidence and with appropriate safeguards for patients. Transitional coverage of breakthrough designated medical devices approved by the FDA will also allow CMS an alternative process to acknowledge where there may be clinical evidence gaps for new technology that may not necessarily be indicative of a failure in the technology or a lapse on the part of the applicant, but rather an opportunity to study the intervention's performance in real world populations.

As a founder of several medical device companies, an advisor or board member for others, and a leader at medical technology innovator Edwards Lifesciences, I understand that predictability is critical to the innovation and development process. Predictability is not the same as certainty. As an entrepreneur, I know it is critical to understand the task and identify obstacles to success.

Once those are known, there is truly an opportunity to take on the challenge and meet patients' needs. The proposed TCET guidance is a foundational step toward greater predictability. That said, more predictability is needed around the concepts CMS describes, including Evidence Preview, Evidence Review, and Evidence Development Plans, as well as the agency timelines.

Edwards is encouraged by CMS's openness to incorporating robust fit-for-purpose evidence development. In the guidance, CMS describes fit-for-purpose as those studies in which the design, analysis plan, and study data can credibly answer the research question. We urge CMS to continue its efforts to further define fit-for-purpose evidence development for breakthrough technologies. We believe fit-for-purpose data collection should be designed with patients and their needs in mind. It should be streamlined to minimize administrative burden and limited to the data necessary to demonstrate to CMS that the innovation is reasonable and necessary to cover. The process of identifying the core, essential data could involve CMS working with the FDA, manufacturers, patients, and specialty societies to outline data requirements and collection techniques to be used. Such an approach would ensure that coverage of the technology is directly linked to evidence of its benefit to the Medicare population as well as support the implementation of appropriate safeguards for patients.

In addition, clinical data collected during the initial coverage period could support a longer-term coverage decision. While registries might be needed in some cases, routinely collected real-world data, such as payer claims and electronic clinical and patient data, could replace the need for extensive clinical data outside the scope of CMS' key questions. Fit-for-purpose data collection aligns with CMS's goal of pursuing timely patient access to innovative technologies and supports the innovation process by leveraging focused evidence generation to benefit development and iteration of the next medical technologies.

Edwards has concerns with the CMS proposal to share the TCET Evidence Preview with the Medicare Administrative Contractors (MACs). Recognizing the previews would be conducted to identify material evidence gaps that will require further evidence development during transitional coverage, we are concerned that they may not reflect the current state of evidence when reviewed by the MAC. The Evidence Preview will have been conducted up to a year prior to FDA approval and developed by the sponsor and CMS to support expedited nationwide coverage of a novel therapy. Therefore, they may not be scalable or even relevant for consideration by a MAC for local coverage.

In the proposed TCET guidance, CMS states that it does not anticipate the TCET pathway will accept more than five candidates per year. This limitation is in part due to limited resources in the CMS Coverage and Analysis Group. While Edwards believes there is a need for more resources and expertise in this part of the organization, we also understand the need to prioritize. As such, we urge CMS to establish clearly defined and transparent criteria that it will use to select candidate technology for TCET review. In establishing these criteria, CMS should consider the following questions:

- Is this device a life-saving medical technology?
- Will the device have a significant (not incremental) impact on the lives of Medicare beneficiaries, their families, and communities?
- Will the technology address unmet patient needs, and does it demonstrate potential for significant clinical benefits?
- How does consideration of this device for transitional coverage contribute to addressing health disparities by expanding access and improving health outcomes?

The lack of clarity in the coverage timeline outlined in the TCET proposal also underscores our continuing concern that CMS lacks adequate resources to meet current demands. Edwards, like many other innovators, patients, and other stakeholders, is concerned that transitional coverage

will put more burden on an already stressed system. Specifically, we believe that CMS needs the ability to hire additional clinical and research experts from outside the agency as full-time CMS employees. FDA, National Institute of Health (NIH), Centers for Disease Control (CDC) and Health Resources and Services Administration (HRSA) have been afforded the authority to hire candidates with doctorates or clinical and research expertise under Title 42 of the Public Health Service Act. However, CMS is governed under the Social Security Act and is therefore not subject to these expanded authorities. As a result, CMS has had difficulty competing with other federal agencies for talent. Bipartisan members of this committee supported expanding FDA's hiring authorities in the 21st Century Cures Act, to help the agency with its responsibility to review and regulate medical products. As medical technology continues to evolve, CMS should have the same authority to hire clinical experts uniquely qualified to understand the technologies its programs cover.

This brief discussion of TCET laid out both opportunities and concerns. Edwards plans to submit more fulsome comments to CMS on the TCET proposed guidance. In summary, it is essential to keep up the momentum of the proposed TCET notice. Further delay of TCET will continue to prevent Medicare patients and our country from getting timely access to needed breakthrough medical innovations.

The Patient Experience

The reason we are having this discussion today is because of our shared goal of improving the lives of patients. Edwards is passionate about the power of direct patient engagement to drive innovation. Throughout the year, we bring patients and their care partners to Edwards to share their stories, connect with our employees, and engage with our development teams. This allows us to incorporate the voice of the patient early in the process and drives patient-centered innovation. Though I am no longer seeing patients in clinic, through Edwards' commitment to

patient engagement, I am still able to connect with the patients who benefit from our therapies. I would like to take a moment to share a little more about one of our patients I mentioned earlier, Jill Poole.

Jill is a 66-year-old registered nurse from Washington state. She's a hard-working, compassionate caregiver who has worked for the VA in Portland, Oregon, for more than 30 years. She comes from a family of healthcare providers. Her mother was a nurse; her sister is a critical care nurse; and her son is a vascular surgeon.

Jill has always been active. She is up every morning at 5:00 a.m. to feed her cows and tend to her garden. After that, she bikes to her mother's house to cook breakfast. All this takes place before her official workday begins.

About five years ago, Jill started experiencing symptoms that forced her to slow down. Her father died of heart failure at age 70, so she knew she needed to be vigilant, but she refused to give in to her fatigue and shortness of breath, as it became more significant, and found ways to compensate. She would kneel in the garden instead of standing up to weed it. She would feed the dog up high instead of bending over to put food in his dish. Eventually, it got to be too much. Simple acts of daily living became a real struggle. Jill was short of breath climbing a short flight of stairs or even putting on her shoes. She no longer had the energy to feed the cows, garden, or take care of her family. When she sought medical help, Jill was told there were no treatment options for the extremely common type of heart failure she was experiencing – a devastating prognosis for her and her family.

Eventually, Jill found a heart failure cardiologist who referred her to a physician at Oregon Health Science University Hospital, Dr. Firas Zahr. Dr. Zahr believed she might be a good

candidate for an investigational device for heart failure that was being studied in an early feasibility trial sponsored by Edwards. Jill, her sister, and her son all researched the therapy but because it was so new, there was not much information available online. In spite of this, and after long discussions with her physician, her family, and her priest, she took the leap of faith and consented to be enrolled in the trial.

Jill's story is not unique. Heart failure impacts more than six million patients and is the number one cause of hospitalization in the US – costing our health system billions of dollars while patients wait for life-saving therapies. Early feasibility studies, like the one Jill participated in, are challenging to enroll. It takes a special patient to raise their hand and say yes, that they will be among the first to be treated. When they make their decisions, there are no published clinical data and no known odds to calculate. There is just trust in their physician, a belief in the promise of the therapy, and the hope they will benefit and regain their quality of life.

We are so grateful to the patients like Jill who raise their hands to participate in these early studies and help us further the science and bring innovative therapies to those with no other therapeutic options, but who have a lot of life left to live. These brave patients, and our collective commitment to generate and analyze robust and accurate data about their experiences, help to ensure breakthrough innovations get to patients who need them most.

I am pleased to share that, in August of 2021, Jill underwent her procedure. Less than a week later, she jumped on a plane and flew halfway across the country to walk in a parade to honor her late father in his hometown. In a matter of days, Jill went from not being able to put on her walking shoes, to marching in a parade to honor her late father. Today, she is as active as ever: gardening, taking care of her cows, her dog, her family, and the veterans she helps at work every day.

It’s been my privilege to get to know Jill and her family and a tremendous honor to know that our work not only helped save her life, but also helped her regain her quality of life.

I share Jill’s story to remind us of all the additional patients who might be helped if we are able to bring coverage and access to new, innovative technologies sooner. These technologies may help patients live their lives and spend time with their families while continuing to add value in their communities. I urge Congress to work with CMS to improve and finalize the TCET guidance and consider what additional improvements can be made to help better treat patients in the US with innovative medical technologies.

In conclusion, time is of the essence to address the gaps that breakthrough technologies often face between FDA approval and CMS coverage. Further delay may prevent Medicare patients from gaining access to breakthrough medical innovations. For the millions of patients with cardiovascular disease like Jill, and others suffering from diseases with unmet needs, we cannot wait. We must enact change now. On behalf of Edwards Lifesciences, thank you for your time.