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STANFORD BYERS CENTER FOR BIODESIGN CELEBRATING 20 YEARS

The Need for Transitional Coverage for Emerging Technologies

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Discussion Guide

Patient access to medical innovations result from a series of multi-stakeholder decisions including regulatory approval from the Food and Drug Administration (FDA), payer coverage and reimbursement, and physician adoption. Recent medical innovations such as devices with FDA's Breakthrough designation may have limited evidence on long term effects on health outcomes and treatment durability. This could impact payer's and physician's decisions to provide patient access even after FDA approval. The evidence available at the time of FDA approval for medical innovations, including breakthrough devices, may not be sufficient to substantiate CMS's assessment for Medicare coverage which can result in delays in broad patient access. Recent policy and legislative proposals have highlighted a need for timelier Medicare coverage for breakthrough devices. The Duke-Margolis Center for Health Policy and the Stanford Byers Center for Biodesign will hold a public webinar to explore stakeholder perspectives and potential policy solutions to inform CMS in their efforts to provide timely and appropriate coverage for breakthrough devices and other emerging technologies.

Background on Medicare Coverage for Breakthrough Devices

The Breakthrough Devices Program is an expedited regulatory approval pathway for medical devices that intends to provide more effective diagnosis and treatment for life-threatening or debilitating conditions. This program was developed as a result of the 21st Century Cures Act of 2016 which included provisions intended to bring promising new therapies to patients quicker. The goal of this program is to facilitate efficient device development and review for therapeutic areas with unmet needs and provide timely patient access.

Medicare coverage for a breakthrough device will depend on whether the treatment falls under a Medicare benefit category and a determination if it is "reasonable and necessary" for the diagnosis or treatment to improve the functioning of Medicare beneficiaries. CMS considers an item or a service reasonable and necessary if it is: (1) safe and effective, (2) not experimental or investigational, and (3) appropriate for use in Medicare beneficiaries. The process for CMS to determine coverage for breakthrough devices can take different pathways. Formal coverage decisions are made at the national or local level. At the national level, CMS issues National Coverage Determinations (NCDs) that define the scope and breadth of coverage for all Medicare beneficiaries. The NCD pathway has a statutorily defined process in which Medicare conducts evidence assessments and solicits public input to inform a coverage policy which can take up to a year following a coverage request. Absent a formal NCD, local Medicare contractors can still provide coverage for individual items or services at their discretion. This period between FDA approval and Medicare coverage is commonly referred to as the coverage gap for breakthrough devices where Medicare patients are unable to access the new treatment.

Given that the Breakthrough Device Program is inherently an expedited regulatory approval pathway, many breakthrough devices at the time of FDA approval may not have enough evidence developed to substantiate the "reasonable and necessary" standards for Medicare coverage. Historically, Medicare has used the longstanding policy of Coverage with Evidence Development (CED) to provide access to novel

technologies, including breakthrough devices, when there is insufficient evidence to substantiate a "reasonable and necessary" determination for Medicare coverage. Under CED, CMS provides limited access to novel technologies with data collection requirements as a condition for coverage. Data collection is designed to generate evidence that would substantiate that the treatment is "reasonable and necessary" for Medicare patients. While CED provides for some Medicare access, it necessitates a national coverage determination (NCD) process and requires the availability of a data collection infrastructure. Consequently, breakthrough devices would still have a coverage gap following FDA approval, meaning patient access would depend on whether a provider has a data collection infrastructure to satisfy Medicare's CED requirements.

The Breakthrough Devices Program has grown significantly since it was first established. Since the inception of the program there have been over 617 device submissions designated as breakthrough. We expect that an expedited coverage pathway will further motivate technological development such that the BDP will continue to grow. However, it is important to note that while the pace of breakthrough device designations is increasing, only a small fraction of these devices reach FDA approval, and fewer necessitate Medicare coverage consideration. Per CMS's analysis, as of September 2020, only 16 breakthrough devices received FDA approval. Ten of those breakthrough devices actually corresponded to a Medicare benefit category and only a subset of those ten required formal coverage assessments because they did not fall into existing reimbursement structures.

Proposals for Timelier Access to Breakthrough Devices

There have been several policy proposals to address the coverage gap that can impact patient access. One recent attempt to alleviate that impact was through the proposal of a new Medicare coverage pathway specific to breakthrough devices called the Medicare Coverage of Innovative Technology (MCIT) pathway. This pathway was first proposed in September 2020 as a way to streamline the approval, coverage, and coding processes and incentivize biomedical innovation. MCIT would have allowed for FDA approved devices with breakthrough designation to receive automatic national Medicare coverage following FDA approval for a period of four years. During the four years of MCIT coverage, manufacturers would develop evidence relevant to Medicare patients to substantiate the "reasonable and necessary" standard for long-term permanent Medicare coverage.

Public comments on the MCIT proposal were mixed, with many stakeholders lauding efforts to address coverage delays, and other stakeholders expressing concern over CMS' limited authority to ensure patient safety and enforce evidence development. The MCIT proposed rule was subsequently delayed, revised, and ultimately repealed in November 2021. Per CMS's responses to public comment, while they remain committed to balancing timely access to breakthrough devices and ensuring Medicare patient safety, they were concerned that the design of the proposed MCIT pathway would not ensure appropriate patient access. Their concerns included two main areas:

1) Medicare patient safety: MCIT limited the ability of CMS to ensure safety of Medicare beneficiaries. Clinical trial studies used for FDA approval for breakthrough devices are not required to enroll Medicare patients. As such, under MCIT, a breakthrough device could be used on Medicare patients without ever demonstrating that it was safe or effective in that patient population. Further, CMS would not have the authority to limit coverage in the presence of any safety issues of the breakthrough device.

2) Evidence generation: MCIT did not require manufactures to solicit feedback from Medicare on evidence generation that would substantiate the "reasonable and necessary" determination for long term Medicare coverage resulting in uncertainty in long term patient access.

CMS recently announced a series of public stakeholder meetings on "Transitional Coverage for Emerging Technologies." In the first meeting, held this past February, CMS solicited public comments on how to address the priorities of timely access for breakthrough devices through existing or new coverage pathways. A second public stakeholder meeting is scheduled on March 31st.

Another recent policy proposal to provide transitional Medicare coverage for breakthrough devices was through the second iteration of the 21st Century Cures bill, commonly referred to as Cures 2.0, drafted on June 22, 2021. Cures 2.0 strives to modernize CMS processes to provide more effective access to new technologies for Medicare beneficiaries, including through a coverage pathway very similar to the original MCIT rule. Given that CMS has already repealed MCIT and identified several issues with the proposal, this legislation as written may not gain administrative support. However, the bill underscores bipartisan congressional support to address patient access to innovative breakthrough devices.

Stakeholder Support for Expedited Coverage

The Stanford Byers Center for Biodesign recently published a study that characterizes support from the innovator community on the need for a redesigned, streamlined reimbursement pathway such as MCIT for novel breakthrough devices. This survey focused on:

- the existing reimbursement pathway for novel and breakthrough technologies and the time and development cost required after FDA authorization.
- the impact MCIT would have on the innovation ecosystem and subsequent patient access to novel technologies.
- a small number of exemplar technologies that FDA has designated as breakthrough devices to show how the acceleration of patient access might impact individual health outcomes and overall healthcare system costs once such devices demonstrate safety and efficacy sufficient for FDA authorization.

The survey results consist of responses from 253 innovators and 83 investors with experience and knowledge of the reimbursement process for novel technologies. Innovators described a pathway to establish coding, coverage and payment that took, on average, 4.7 years for national Medicare coverage[1], and investors indicated that reimbursement was the most important external risk factor to their investment decisions. In addition, respondents in large part felt that the current programs were not sufficient to support breakthrough product designation, with 54% of innovators and 79% of investors disagreeing with the statement: "The existing parallel review process with FDA and the CED pathway are sufficient to provide timely patient access for novel medical technologies."

[1] The survey used the phrase "national Medicare coverage" to encompass a National Coverage Determination (NCD) and nationwide coverage through the accumulation of local MAC coverage decisions.

Innovators were asked about the frequency with which their companies normally collect clinical data after FDA authorization. 87% of respondents indicated that collecting additional clinical data or real-world evidence was something that they do (53% always, 34% most of the time) as part of the development of a breakthrough product[2]. Thus, a requirement for further clinical data or the collection of real-world evidence on Medicare patients after FDA authorization already aligns with the clinical evidence generation expectations of most developers.

Additionally, an MCIT-like program would stimulate development of novel technologies. Innovators reported they would be more likely to take on a breakthrough technology as their next project if an MCIT-like accelerated program was in place. Investors, too, were influenced by the potential of such a program and expressed an increased interest in investing in breakthrough technology should a program be created. Positive impacts would be experienced in clinical areas important for Medicare patients such as cardiovascular, neurovascular disease and stroke, and neurological disease.

Four technologies were highlighted in the paper to bring to life the potential impact for patients and the healthcare system. Each technology is currently under development and demonstrates the promise of improving and saving lives through more effective treatment, avoiding expensive complications, and earlier diagnosis.

The session of the webinar titled, "Medicare Coverage of Innovative Technology Survey Results" will further explore these findings and their implications.

Implementation Considerations for an Expedited Coverage Pathway

The intent of any expedited coverage pathway is to ensure timely access of breakthrough devices for Medicare beneficiaries who could get substantial health benefits from their use. This pathway will offer an opportunity for a predictable and reliable framework through which device manufacturers can get earlier and sustained collaboration with CMS to support coverage needs. This will be particularly beneficial for smaller manufacturers with limited resources who may have uncertainties on how to address evidence needs efficiently. Importantly, the success of this pathway depends on both providing rapid coverage and supporting post-market evidence generation on key questions relevant to the Medicare population unaddressed at the time of FDA approval.

The dialogue between stakeholders and CMS throughout the various MCIT proposals highlight several overarching objectives for an expedited pathway which include:

- Timely access to new technologies, in this case breakthrough devices;
- Measures to ensure safety of Medicare patients;
- Earlier guidance from CMS to manufacturers on evidence generation relevant to Medicare beneficiaries to support a "reasonable and necessary" determination for long term Medicare coverage; and
- Earlier guidance from CMS on how to coordinate coding and payment applications to secure Medicare reimbursement

[2] FDA has the authority to require a manufacturer to conduct post-market surveillance studies of a class II or class III device that meets certain criteria.

The aforementioned objectives are some of the core issues raised during the first TCET Public Stakeholder meeting and will be further explored during the first panel session in the webinar "Potential Path Forward for Transitional Coverage for Emerging Technologies." Panelists will explore potential operational frameworks that can achieve these goals. Duke-Margolis has previously published recommendations for an operational framework for expedited coverage of breakthrough devices when the standard coverage process is not adequate for timely and appropriate access. Importantly, this special coverage process will not be needed for all breakthrough devices. Many breakthrough devices may not be relevant for the Medicare population, may not fall under an existing Medicare benefit category (eg. digital health products, molecular diagnostics, etc.), or already have a clear path to coverage through existing reimbursement structures.

The success of an expedited coverage pathway will largely depend on how efficiently manufacturers and providers can develop real world evidence (RWE) that supports CMS's "reasonable and necessary" determination for long term Medicare coverage. CMS evidence assessments generally center around two questions: whether the treatment improves health outcomes, and whether there are any characteristics of the patient, operator, or facility that can increase the likelihood of health outcomes. RWE can help provide valuable information on the impact of a treatment's safety and effectiveness across different populations and reveal patient benefits when breakthrough devices are incorporated into clinical practice. Thus, a key tenet of any expedited coverage pathway will be the extent to which RWE can be efficiently developed to address these questions. Early engagement across CMS, manufacturers, the FDA, the National Institutes of Health (NIH), the Agency for Healthcare Research and Quality (AHRQ) and other stakeholders can identify evidence gaps and inform how to efficiently generate RWE to address those evidence gaps. Public comments on the MCIT rule and in the recent public meeting on TCET show a general agreement among stakeholders that an opt-in expedited process centered around earlier engagement between FDA, CMS, and device sponsors would add predictability, reliability and transparency to a coverage process.

Earlier engagement across stakeholders will also allow manufacturers to plan for and navigate disparate coding and payment processes to ensure a new technology is adequately reimbursed at the hospital and provider level. While coverage is a determinant to patient access, reimbursement make breakthrough devices financially accessible to both providers and patients. These activities today are all conducted following FDA approval, which can extend existing coverage gaps. Earlier engagement on these issues, prior to FDA approval, will afford stakeholders time to ensure all structures and processes are in place to facilitate earlier patient access. During the second panel session, "Transitional Coverage and Practice," panelists will explore mechanisms to achieve earlier stakeholder engagement, hospital and provider needs to support adoption of breakthrough devices, and how to efficiently generate RWE to support long term coverage decisions.

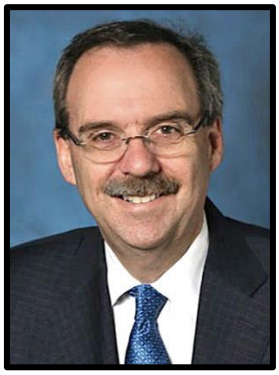
Finally, any new coverage initiative will have the greatest and most sustainable impact if it is accompanied by steps that help assure CMS has adequate processes and resources for implementation. Ongoing bipartisan support for the FDA through additional appropriations and user fees has resulted in hundreds of additional expert staff positions in recent years – enabling FDA to implement transparent, rapid, and updated approval processes. In contrast, CMS resources to provide timelier access to new technologies have declined. As the pipeline of breakthrough devices grows, further resources may be needed to more effectively integrate breakthrough devices into the care of Medicare beneficiaries. Additional supports and resources would allow CMS to keep pace with expected innovation within the breakthrough device program and formalize an operational framework with predictable timelines and expectations.

Speakers[3]

Josh Makower, MD is the Boston Scientific Applied Bioengineering Professor of Medicine and of Bioengineering at the Stanford University Schools of Medicine and Engineering, and is the Director and Co-Founder of the Stanford Byers Center for Biodesign. Josh also serves as a Special Partner on New Enterprise Associate's healthcare team supporting their medtech/healthtech practice. Lastly, Josh is the Founder and Executive Chairman of ExploraMed, a medical device incubator that has created 10 companies over the past 20 years. Josh currently serves on the board of directors for DOTS Devices, Eargo (NASDAQ: EAR), ExploraMed, Allay Therapeutics, Lungpacer, Moximed, Willow Innovations, SetPoint Medical and Coravin. Josh holds over 300 patents and patent applications for various medical devices in the fields of cardiology, ENT, general surgery, drug delivery, plastic surgery, dermatology, aesthetics, obesity, orthopedics, women's health and urology. He received an MBA from Columbia University, an MD from the NYU School of Medicine, and a bachelor's degree in Mechanical Engineering from MIT, and is a member of the National Academy of Engineering and a Fellow at the American Institute of Biomedical Engineering.

Mark McClellan, MD, PhD, is Director and Robert J. Margolis, M.D., Professor of Business, Medicine and Policy at the Margolis Center for Health Policy at Duke University. He is a physician-economist who focuses on quality and value in health care, including payment reform, real-world evidence and more effective drug and device innovation. Dr. McClellan is at the center of the nation's efforts to combat the pandemic, the author of COVID-19 response roadmap, and co-author of a comprehensive set of papers and commentaries that address health policy strategies for COVID vaccines, testing, and treatments, nationally and globally. He is former administrator of the Centers for Medicare & Medicaid Services and former commissioner of the U.S. Food and Drug Administration, where he developed and implemented major reforms in health policy. Dr. McClellan is an independent board member on the boards of Johnson & Johnson, Cigna, Alignment Healthcare, and PrognomIQ; co-chairs the Guiding Committee for the Health Care Payment Learning and Action Network; and serves as an advisor for Arsenal Capital Group, Blackstone Life Sciences, and MITRE

[3] Speakers listed in order of appearance



Lee A. Fleisher, MD, was named the Chief Medical Officer and Director of the Center for Clinical Standards and Quality for the Centers for Medicare and Medicaid Services in July 2020. In this capacity, he is responsible for executing all national clinical, quality, and safety standards for healthcare facilities and providers, as well as establishing coverage determinations for items and services that improve health outcomes for Medicare beneficiaries. He is also Professor of Anesthesiology and Critical Care and Professor of Medicine at the University of Pennsylvania Perelman School of Medicine. He was Treasurer of the Board of Directors and Chair of the Finance Committee of the National Quality Forum. He was a member of the Care Transformation Forum (CTF) of the Health Care Payment Learning and Action Network (LAN). He is currently an Affiliated Faculty of the Quattrone Center for the Fair Administration of Justice at the University of Pennsylvania Carey Law School and a Senior Fellow of the Leonard Davis Institute of Health Economics.

Sandra Waugh Ruggles, PhD, is an Associator Director at the Stanford Byers Center for Biodesign and President of Summit Rock Strategy Consulting, a consulting firm providing product definition, market research, and business strategy services to emerging and established medical device companies. Sandra has over 20 years of experience in medical device and biotechnology innovation, leading projects for next generation and breakthrough products in a wide variety of clinical areas. She was also a co-founder at Catalyst Biosciences (NASDAQ: CBIO) and is an inventor on 10 issued patents. Dr. Ruggles earned her PhD at UC San Francisco and is an alumna of the Stanford Biodesign Innovation Fellowship.

Dirksen Lehman is corporate vice president, public affairs, at Edwards Lifesciences in Irvine, CA. He joined the company in 2007 as vice president of government affairs, and later added responsibility for global health economics and reimbursement, global communications, corporate branding, corporate medical affairs, global corporate giving, and patient engagement. Earlier in his career he served in The White House as special assistant to the president for legislative affairs, and health counsel for the majority on the U.S. Senate Committee on Health, Education, Labor and Pensions. He serves as treasurer of the board of directors for the California Life Sciences Association, and is a member of the board of directors for Team Heart.

Parashar Patel is ViewRay's Senior Vice President, Government Affairs & Market Access. Parashar is an experienced health care executive with a demonstrated history of accomplishments in the medical device industry and public sector. His areas of expertise include health care delivery systems, payment and coverage policy, market access, medical devices, and clinical research. Parashar has held a variety of roles at CMS, the American Association of Health Plans, the Office of (then) Senate Majority Leader George J. Mitchell, the White House Office of Management and Budget, and Connecticut's Medicaid agency.

Dr. Schulman was appointed as Professor of Medicine, Associate Chair of Business Development and Strategy in the Department of Medicine, Director of Industry Partnerships and Education for the Clinical Excellence Research Center (CERC) at the Stanford University School of Medicine, and, by courtesy, Professor of Operations, Information and Technology at Stanford's Graduate School of Business. Prior to coming to Stanford, Dr. Schulman served as a Professor of Medicine at Duke University, directed the Health Sector Management Program at Duke's Fuqua School of Business for a dozen years, created and directed the Duke University Master's of Management in Clinical Informatics Program, and served as a Visiting Professor and Visiting Scholar at Harvard Business School.He is a co-founder of Bivarus (exit January, 2018), co-founder and Managing Member of Faculty Connection, LLC., and is a Board Member of Grid Therapeutics.

Dr. Marianne Hamilton Lopez is the Senior Research Director of Biomedical Innovation, an adjunct associate professor, and core faculty at the Duke-Margolis Center for Health Policy in Washington, DC. She leads the strategic design and direction of the Center's Biomedical Innovation portfolio, with a focus on medical products development and regulation, real world evidence, infectious disease preparedness, and payment, pricing, and coverage of drugs and medical devices. Prior to joining Duke-Margolis, Dr. Hamilton Lopez was a senior program officer with the National Academy of Medicine's Leadership Consortium for a Value & Science-Driven Health System and led the Consortium's Science and Technology portfolio and Clinical Effectiveness Research Innovation and the Digital Learning Collaboratives. She was a Senior Manager at AcademyHealth; a Public Health Community Advisor for the United States Cochrane Center; and the Federal Women's Program Manager and American Indian/Alaska Native Employment Program Manager for the National Institutes of Health.

Louise Guy is a registered nurse with extensive experience in healthcare delivery systems focusing on clinical care, clinical and capital sales, third-party reimbursement methods, operations and strategic business planning. For the past fifteen years, Louise has directed her efforts toward the areas of reimbursement, patient advocacy, medical policy, pricing, government relations, and managed care sales. Prior to joining Argenta, Louise spent six years in the biotechnology industry, where she successfully established reimbursement for both FDA and non-FDA approved products. She interfaced directly with senior officials at the Centers for Medicare and Medicaid Services (CMS), as well as with corporate medical directors for health plans across the country. Louise also brings ten years of experience as the manager of medical review and utilization management at BlueCross BlueShield of Massachusetts (BCBS MA).

Michael Mack, M.D. has practiced cardiothoracic surgery in Dallas, TX since 1982. He is board certified in Internal Medicine, General Surgery, and Thoracic Surgery and is currently the Director of the Cardiovascular Service Line for the Baylor Scott & White Health, Chair of the Baylor Scott & White Cardiovascular Governance Council, President of Baylor Scott & White Research Institute, and Director of Cardiovascular Research at The Heart Hospital Baylor Plano. He has over 650 peer reviewed publications.

Joe Franklin is a Product Counsel at Verily Life Sciences, where he works on legal, regulatory, and policy components of Verily's clinical evidence generation program. Before joining Verily, Joe held a variety of positions at FDA, including as senior advisor on data and evidence initiatives. Joe built and led the biosimilars policy staff in the Office of New Drugs and served as an attorney in the chief counsel's office for multiple periods during his career at FDA—including during COVID-19, when he advised FDA on emergency use authorizations and supported the U.S. government's international vaccine donations. Joe has a PhD in cell biology from his early career as a bench scientist.