

CURES 2.0 ISSUE BRIEF

Building a Modern Health Data Infrastructure:

Cures 2.0 Act Provisions on Real-world Evidence and Federal Agency Communication

Authors: Trevan Locke, Rebecca Ray, Rachele Hendricks-Sturup, Marianne Hamilton Lopez, Morgan Romine, Mark McClellan

Background

Seeking to build on past efforts and incorporate lessons learned from the COVID-19 pandemic, policymakers are working to identify opportunities to accelerate medical research and foster patient access to innovative medical products and novel technologies. The recently released discussion draft for The 21st Century Cures Act 2.0 (“Cures 2.0 Act”) is one such effort.¹

The Cures 2.0 Act seeks to build on the bipartisan successes of the 21st Century Cures Act (2016) to support more effective medical product development and regulation. The Cures 2.0 Act includes provisions on long-COVID research, future pandemic preparedness, development of antimicrobial innovations, clinical trial representativeness, digital health technologies, cell and gene therapies, accelerated approval, and the proposed Advanced Research Projects Agency for Health (ARPA-H).

The Cures 2.0 Act also includes several provisions related to the use of real-world evidence (RWE) as well as improving communication between the US Food and Drug Administration (FDA) and the Centers for Medicare and Medicaid Services (CMS). Among these provisions are:

- 1) the creation of a new US Department of Health and Human Services (HHS)-led RWE Task Force,
- 2) a requirement for HHS to generate a framework to expand the use of RWE, and
- 3) the establishment of an automatic communication requirement between FDA and CMS for products designated by FDA as breakthrough therapies, fast track products, or products eligible for accelerated approval so that the agencies may share information regarding product approval and coverage decisions more efficiently.

While provisions in the Cures 2.0 Act represent another step forward for the implementation and use of RWE by Federal agencies, the legislation should go further to support the improvement and modernization of the entirety of US health data infrastructure. This data infrastructure is essential to collecting the high-quality real-world data (RWD) that is needed to generate RWE for decision making. The Cures 2.0 Act is an opportunity to lay the data infrastructure foundation needed to support a learning health care system,² while supporting alignment between FDA, CMS, and other Federal agencies like the Centers for Disease Control and Prevention (CDC) as they make their respective regulatory, payment, and public health decisions.

The Duke-Margolis Center for Health Policy’s (“Duke-Margolis” or “Center”) Real-World Evidence Collaborative (the “Collaborative”) and Value-Based Payment for Medical Products Consortium (the “Consortium”) have explored many issues impacting health data infrastructure in the US over the last several years. This issue brief, informed by these multi-stakeholder groups and [one of several Cures 2.0 Act-focused issue briefs developed by Duke-Margolis](#), offers recommendations for further improving US health data infrastructure to inform policy and legislation, accelerate medical research, and foster patient access to novel technologies.

A learning health care system is one in which science, informatics, incentives, and culture are aligned for continuous improvement and innovation, with best practices seamlessly embedded in the care process, patients and families active participants in all elements, and new knowledge captured as an integral by-product of the care experience.¹

Policy Strategies: How the Cures 2.0 Act Can Facilitate a Modern Data Infrastructure

Improve Communication Channels Between Federal Agencies

Policymakers have recognized the need to improve Federal agency collaboration – particularly between FDA and CMS – regarding product approval and reimbursement. There have been previous efforts to streamline processes to provide earlier patient access to novel technologies. In 2011, CMS established the Parallel Review Program in which both FDA and CMS provide feedback on the design of the pivotal clinical trial.³ Cures 1.0 also created programs intended to bring promising new therapies to patients quicker, like the pathway of approval for regenerative therapies⁴ and the Breakthrough Devices designation program.⁵ CMS can leverage these breakthrough designations as one basis for streamlining consideration of payment decisions for new products. Alongside Cures 2.0, newer legislative efforts, such as the NOVEL Act,⁶ are attempting to improve interagency coordination and reduce the amount of time between approval and reimbursement decisions.

While these efforts are encouraging and important starting points, without parallel infrastructure in place to ensure efficient FDA and CMS collaboration the pathway from approval to patient coverage will remain complex. Any effort to modernize these two agencies’ product review and approval processes or advance the use of RWE should consider, from a practical standpoint, how to reinforce communication and collaboration, such as:

Facilitating the expanded use of the Sentinel Initiative across Federal agencies. FDA has spent over a decade building infrastructure to support the Sentinel Initiative, a safety surveillance system that proactively monitors medical product safety using real world data (RWD). As recommended in Sentinel’s 2019-2023 five-year strategy,⁷ FDA, CMS, CDC, and other Federal agencies should partner to broaden the utilization of the Sentinel System where relevant for needs of each agency.

Supporting the creation of a coverage with evidence development (CED) infrastructure that synergizes with Sentinel and post-market studies required by FDA. CED has been used by CMS to provide access to innovative technology that has insufficient evidence to satisfy certain criteria

for Medicare coverage. However, there has been no reliable infrastructure in place to support a systematic and predictable approach to CED. Existing post-market evidence development initiatives, like FDA's Sentinel and National Evaluation System for health Technology (NEST), could help support a multipurpose CED infrastructure to facilitate regulatory surveillance and assess the benefits and risks in real world populations who are subject to these treatments, especially for the Medicare population.

Utilizing the proposed RWE Task Force to align on data needs between FDA, CMS, CDC, and other Federal agencies. The RWE Task Force included in the Cures 2.0 Act discussion draft has the potential to be an important vehicle for aligning data needs. In addition to the current tasks set by the discussion draft, the task force should work to align data and data infrastructure needs within and between Federal agencies to support both interagency and cross-agency coordination and decision-making. This work might include addressing questions, such as:

- How can non-FDA agencies leverage the Sentinel System?
- How should Federal agencies make use of the United States Core Data for Interoperability (USCDI) from the Office of the National Coordinator for Health Information Technology (ONC)?
- What opportunities exist to harmonize post-market data requirements for FDA and CMS?
- Should the Office of Management and Budget (OMB) update the current minimum requirements on race and ethnicity data collection?
- How might HHS' RWE Framework support the collection of additional data in rare disease settings?
- How do data use policies vary across Federal agencies and is there room to harmonize those policies?

Additionally, the RWE Task Force should be broadly representative of stakeholders in the RWE space including sponsors of both drugs and medical devices, academic data experts, patients and patient advocates, policy experts, clinicians, electronic health record vendors, data infrastructure developers, payers, and data curators. Representatives from CDC should also be included.

The RWE Task Force could also consider how to expand the role of the private sector in developing the US's data infrastructure. This might include funding public-private partnerships or encouraging the creation of standards for data analytics and data curation, including defining specific requirements for regulatory grade data provenance information. The Task Force could help coordinate a consistent approach to these issues that can address the needs of Federal agencies and other stakeholders and support a learning health care system.

Developing a more comprehensive coordination process between FDA and CMS. Well controlled protocols and procedures should be developed, implemented, and enforced to standardize effective communication between the two agencies while also preserving and upholding each agency's independence and expectations. As they align with what is proposed in the Cures 2.0 Act discussion draft, such protocols and procedures might include:

- Cross-agency working groups focused on individual disease types or therapeutic classes. Among other benefits, this can provide CMS staff with the opportunity to benefit from the expertise and experience of FDA reviewers.

- CMS could establish provisional codes and payment for novel technologies to ensure availability of codes and payment at the time of FDA approval.
- Controlled protocols and procedures that describe cross-agency communication frequencies and standards.
- Evaluation of overlapping data needs and prioritization of those needs to develop improved communication channels.

Any coordination effort between FDA and CMS should acknowledge the agencies’ unique evidence needs, avoid creating burdensome administrative processes, and focus on increasing efficiency.

Providing the necessary resources, particularly to CMS, to implement these recommendations.

Implementing new systems and expanding existing systems are not without cost. Funding for adequate resources should be appropriated to support the modernization, expansion, and harmonization of infrastructures and systems that can support the use of RWE at Federal agencies. While all agencies involved need more resources, personnel and resource needs at CMS for coverage and analysis decisions are particularly acute. CMS currently lacks the increased resources and targeted hiring authority that were granted to the FDA under the 21st Century Cures Act to support FDA in its efforts to implement Cures-mandated policies and programs. It is highly likely that CMS will therefore face more substantial challenges implementing new or expanded systems without additional Congressionally-provided support.

Table 1: Translating these recommendations into the Cures 2.0 Act

Recommendation	Location in Legislation
-Facilitating the expanded use of the Sentinel Initiative across Federal agencies. -Supporting the creation of a coverage with evidence development (CED) infrastructure that synergizes with Sentinel and post-market studies required by FDA -Developing a more comprehensive coordination process between FDA and CMS	Include additional provisions under “SEC. 305. IMPROVING FDA-CMS COMMUNICATION REGARDING TRANSFORMATIVE NEW THERAPIES”
-Utilizing the proposed RWE Task Force to align on data needs between FDA, CMS, CDC, and other Federal agencies	Include additional provisions under “SEC. 304. INCREASING USE OF REAL WORLD EVIDENCE”
-Providing the necessary resources, particularly to CMS, to implement these recommendations	Add additional sections under TITLE III “FOOD AND DRUG ADMINISTRATION” and TITLE IV “CENTERS FOR MEDICARE & MEDICAID SERVICES”

Promote Data Linkage

Health data is generated from an ever-increasing array of sources. Electronic health records, insurance claims, registries, wearables, genomic tests, social media, and more are all potential contributors to a more complete picture of personal and population health.

For this wealth of data to make the largest contribution, systems and databases housing such data must be linkable and interoperable in a way that preserves data subject privacy without compromising the integrity or utility of the data itself. Responsible data linkage, the bringing together of information about the same person from different sources, is critical to creating a holistic, longitudinal view of a person's health. Although privacy-preserving tools that can accurately and efficiently link datasets are readily available, existing and sometimes antiquated policies often hinder or do not fully support their use in practice.

To promote responsible data linkage and help accomplish the goals of the Cures 2.0 Act, policymakers and other data stakeholders should consider:

Prioritizing the linkage of electronic health record and claims data. The Cures 2.0 Act could encourage the use of existing technology and standards to better link electronic health record and claims data by ensuring that policies around patient privacy and the identification or de-identification of data accurately reflect today's technologies. As part of this linking effort, consideration should also be given on how to integrate patient-reported and patient-generated data into health records.

Providing support to modernize the National Death Index (NDI). The NDI is the official US system for recording mortality rates, including causes of death, and is a critical resource for researchers. However, its current design and structure does not facilitate the use of modern data science.⁸ Working with vital statistics offices in each state, the CDC should develop new data use agreements and cost structures that encourage states to quickly and completely upload their data while enabling researchers to conduct vital research on causes of death in the US. A modernized NDI will enable higher quality data linkage, which would contribute to more complete pictures of health and causes of mortality.

Supporting the creation of a national registry framework that includes a minimum dataset of variables relevant to FDA and CMS. To address existing challenges with data collection, policymakers could encourage the utilization of modern RWD systems to develop near real-time, continuously monitored national registries. Such registries could be built from a framework consisting of a minimum dataset of variables of interest to both FDA and CMS and based on the USCDI. From this common framework, disease- or therapy-specific registries could be developed that include the variables that are most relevant to post-market monitoring of novel health products and therapies. These registries should have system linkage capabilities, built on Fast Healthcare Interoperability Resources (FHIR) data standards, to connect with other relevant datasets such as electronic health records or claims data and serve as an additional high-quality data source for distributed data networks such as Sentinel and NEST. In addition to supporting the work of Federal agencies, these registries could support researchers seeking to answer vital public health questions by querying a large, standardized national dataset. A national vaccine registry may be a good initial application for this approach.

Overturing the ban on funding for unique patient identifiers. One of the biggest hurdles to linking records across datasets are legal barriers to developing unique patient identifiers (UPIs). While there are valid concerns about patient privacy that must be addressed, the current Federal ban on funding UPI development limits the development of privacy-preserving UPIs and slows efforts to develop a comprehensive, privacy-preserving national health data infrastructure. The cost of these barriers was evident when comparing the United Kingdom’s ability to set up the UK RECOVERY trials,⁹ which were responsible for much of the best data available on potential COVID-19 therapies, to the US’ inability to quickly conduct trials at a similar scale. Congress should again explore overturning its ban on funding what could be privacy-preserving UPI development and innovation. Any effort to advance a UPI should also be paired with a national education campaign to help citizens understand the value and purpose of UPIs, not only for their individual health care needs, but also for a collective effort to support a secure learning health care system.

Table 2: Translating these recommendations into the Cures 2.0 Act

Recommendation	Location in Legislation
-Prioritizing the linkage of electronic health record and claims data.	Include additional provisions under “SEC. 304. INCREASING USE OF REAL WORLD EVIDENCE”
-Providing support to modernize the National Death Index (NDI) -Overturning the ban on funding for unique patient identifiers	Add an additional section under “Title I PUBLIC HEALTH”
-Supporting the creation of a single national registry that includes a minimum dataset of variables relevant to FDA and CMS	Include additional provisions under “SEC. 304. INCREASING USE OF REAL WORLD EVIDENCE”

Implement Strategies to Address Data Infrastructure Gaps Identified During the Pandemic

The COVID-19 pandemic provided a renewed focus on existing data infrastructure gaps. Incomplete or delayed data delivered often through antiquated methods in unstandardized formats made COVID-19 case and mortality tracking especially difficult throughout much of the pandemic. On the other hand, the pandemic pressure tested new approaches to data generation and delivery that offer lessons for the future.¹⁰

To address data infrastructure gaps identified during COVID-19 and ensure that the US is better prepared and more resourceful during future public health emergencies, policymakers and agencies should consider:

Providing sustained support for infrastructure investments to enable the use and interoperability of various health data systems across the country. Investing in public health infrastructure on a sustained basis will enable efficient and real-time high-quality data collection, reporting, and sharing among and across modernized data systems and databases in both emergency and non-emergency settings during current and future pandemics

Supporting local, state, and Federal agencies in designating, adopting, and implementing data and technology standards for public health data. Alongside improved infrastructure, consistent

data collection and analysis standards will foster clear and streamlined communication between key stakeholders, including public health authorities and Federal agencies, researchers, and the public. Consensus-driven public health data standards should be prioritized, with precedence given to those adopted by ONC.

Harmonizing data collection requirements. Having harmonized data collection requirements across Federal, state, tribal, territorial, and local public health authorities and agencies could promote increased interoperability across databases and support the rapid dissemination of new requirements in response to health emergencies. The Cures 2.0 Act or similar legislation could facilitate this process by charging HHS to prepare reports on current data collection requirements and encouraging Federal public health authorities to work with their counterparts at the state, tribal, territorial, and local levels. Such requirements would also enable easier sharing of data between stakeholders.

Implementing these recommendations could help create an early public health warning system and, alongside the modernization of the US’ national health data infrastructure, could help advance medical research and drive further innovation.

Table 3: Translating these recommendations into the Cures 2.0 Act

Recommendation	Location in Legislation
<ul style="list-style-type: none"> -Providing sustained support for infrastructure investments to enable the use and interoperability of various health data systems across the country. -Supporting local, state, and Federal agencies in designating, adopting, and implementing data and technology standards for public health data. -Harmonizing data collection requirements. 	<p>Add a new section under “TITLE I PUBLIC HEALTH”</p>

Upholding Data Subject Privacy, Security, and Access

National data modernization and infrastructure development efforts draw important opportunities to ensure that new and updated systems are human-centered. As these efforts evolve, additional consideration should be given to ensuring that patients and their caregivers have secure access to their personal health data.

Data infrastructure modernization, interoperability, and linkage also warrant a strengthened assessment of data privacy and security gaps. Many novel sources of insightful health data are not covered or protected by the Health Insurance Portability and Accountability Act (HIPAA) and include but are not limited to wearable heart rate monitors or step trackers. Therefore, the Cures 2.0 Act could go further to help policymakers align their efforts towards the development of comprehensive privacy legislation that would cover and protect sensitive categories of non-HIPAA-covered health data as well as patient and consumer choices regarding the allocation, sharing, and use of their sensitive data. Such protections may include giving patients and consumers the right to provide or revoke consent for both initial and secondary

uses of their data for clinical or product development research. Overall, updating or considering policies to reflect today’s technologies could give patients increased choice and consent regarding how their data are used.

Table 4: Translating these recommendations into the Cures 2.0 Act

Recommendation	Location in Legislation
-Ensuring that patients and their caregivers have secure access to their personal health data	Add a new section under “TITLE II PATIENTS AND CAREGIVERS”

Conclusion

The Cures 2.0 Act is a vital legislative step toward the establishment of further and timely support for innovative, data-driven research and medical product development, payment, and regulation. Yet, it must go further to help ensure that the US’ health data infrastructure is modernized enough to support a learning health care system that can leverage novel technologies and data sources in critical scenarios today and in the future. Modernized and privacy-preserving health data infrastructures and systems can help identify and address public health threats faster and offer high-quality evidence to support responsible and timely regulatory and payer decision making. If considered under the Cures 2.0 Act, the recommendations herein could help ensure that the nation’s health data infrastructure is ready to meet the ever-evolving challenges faced today and in the future.

Importantly, infrastructure challenges cannot be addressed in a vacuum. Alongside infrastructure development, stakeholders, including FDA and CMS, must also be working together to determine relevant real-world endpoints and outcomes to ensure. Duke-Margolis will continue to explore issues surrounding data infrastructure and common data needs for both regulators and payers through the Center’s work with the Collaborative and Consortium. The Center looks forward to future opportunities to provide input on this vital work.

References

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Disclosures

Mark B. McClellan, MD, PhD, is an independent director 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 receives fees for serving as an advisor for Arsenal Capital Partners, Blackstone Life Sciences, and MITRE.

About the Duke-Margolis Center for Health Policy

The Robert J. Margolis, MD, Center for Health Policy at Duke University is directed by Mark McClellan, MD, PhD, and brings together expertise from the Washington, DC, policy community, Duke University, and Duke Health to address the most pressing issues in health policy. The mission of Duke-Margolis is to improve health and the value of health care through practical, innovative, and evidence-based policy solutions. Duke-Margolis catalyzes Duke University’s leading capabilities, including interdisciplinary academic research and capacity for education and engagement, to inform policy making and implementation for better health and health care. For more information, visit healthpolicy.duke.edu.

About the Duke-Margolis Real-World Evidence Collaborative

The Duke-Margolis RWE Collaborative, guided by an advisory group consisting of leaders representing medical product developers, payers, research groups, providers, patient networks, and regulators, engages stakeholders to guide high-priority efforts aimed at improving the development and use of RWE. The Collaborative also strives to drive progress in the use of real-world data and evidence to improve patient treatment options and outcomes more broadly.

About the Duke-Margolis Value-Based Payment for Medical Products Consortium

The Duke-Margolis VBP Consortium, composed to patient advocates, payers, manufacturers, and providers, as well as experts on regulatory affairs, law, and policy, develops approaches to payment reform that support better outcomes for patients and better value across the systems. The Consortium seeks to overcome current barriers to VBP arrangements by identifying and developing solutions to legal and regulatory issues, and by addressing operational challenges such as fragmented and difficult-to-track patient outcome data.

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Boston Scientific

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Subhara Raveendran
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Ben Taylor
Aetion

David Thompson
Syneos Health

Aracelis Torres
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Zac Wessler
Amgen

David Wormser
Novartis

Vani Vannappagari
GlaxoSmithKline – ViiV

Dick Wilke
ISPOR

Sepideh Varon
Abbvie-Allergan