MILKEN INSTITUTE

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The Future of US Biomedical Research and Innovation

RECOMMENDATIONS FOR ACTION

About the Milken Institute

The Milken Institute is a nonprofit, nonpartisan think tank focused on accelerating measurable progress on the path to a meaningful life. With a focus on financial, physical, mental, and environmental health, we bring together the best ideas and innovative resourcing to develop blueprints for tackling some of our most critical global issues through the lens of what's pressing now and what's coming next.

About Milken Institute Health

Milken Institute Health develops research and programs to advance solutions in biomedical innovation, public health, healthy aging, and food systems.

About FasterCures

The Milken Institute's FasterCures is working to build a system that is effective, efficient, and driven by a clear vision: patient needs above all else. We believe that transformative and life-saving science should be fully realized and deliver better treatments to the people who need them.

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Foreword

Over the course of four million years, average life expectancy increased by just 11 years, from 20 to 31. Since 1900, worldwide lifespans have more than doubled, from 31 to 73 years.

What drove this change, which is perhaps the greatest achievement in human history? Advances in public health and medical research, from sanitation and vaccines to innumerable breakthroughs in how we understand, prevent, and treat disease.

Many of these advances emerged from US hospitals, laboratories, and institutions. They stand among America's greatest gifts to humankind, strengthening global prosperity and serving as instruments of diplomacy and cooperation across nations. Preserving US leadership in the biosciences is of immense value not only to our own people but also to the world.

Fourteen years ago, that belief inspired FasterCures to convene a remarkable gathering at Lake Tahoe focused on Accelerating Innovation in the Bioscience Revolution. The goal of the 2011 retreat was to find solutions to a pressing challenge: Scientific knowledge was accelerating, but better treatments were not getting to patients fast enough. Roughly 80 leaders from various sectors discussed how progress depends as much on the systems that govern science as it does on the brilliance of those advancing our understanding. My analogy at the time was that while the engines on our scientific trains had advanced significantly, they were limited because we never upgraded the rails on which they travel.

The retreat took place during a period of deep political polarization, yet participants from across the aisle—including House Majority Leader Eric Cantor and Senate Majority Leader Harry Reid—found common ground around priorities more important than party politics. One of the key recommendations was the creation of a new US agency focused on bridging the gap between scientific discoveries and real-world treatments. Within months, Congress created and funded the National Center for Advancing Translational Sciences (NCATS), demonstrating that even in a complex landscape, a focused coalition of leaders can set aside differences and deliver meaningful change.

In the years since, we have seen extraordinary progress: new public–private partnerships, the emergence of the Advanced Research Projects Agency for Health (ARPA-H), unprecedented genomic and data-science capabilities, and powerful new tools in computing and artificial intelligence. Yet even as we have advanced, many of our systems remain fragmented. Other nations are investing aggressively, and as the pages that follow make clear, in some areas they are beginning to outpace US innovation. To remain the world's leader in biomedical research, the US must continue to modernize and fund its infrastructure, strengthen collaboration among sectors, and ensure that breakthroughs in the lab reach the patients who need them most.

In September 2025, we reconvened this conversation at the Milken Center for Advancing the American Dream in Washington, DC, bringing together many of today's foremost leaders from across the bioscience ecosystem to chart the next era of biomedical innovation. The insights and recommendations that emerged from those discussions form the foundation of this report, which presents a shared framework for accelerating discovery and improving health. Drawing on expertise from science, public health, philanthropy, policy, and finance, it focuses on a single objective: translating discovery into better health and longer, more productive lives.

Over the past century, improvements in health have accounted for more than half of global economic growth—a reminder that investing in health is not only a moral imperative but also an economic one. The opportunity before us is extraordinary: to extend healthy longevity, sustain prosperity, and secure a flourishing future for generations to come.

Michael Milken

Chairman | Milken Institute

Executive Summary

Long the world's leader in biomedical research and innovation, the United States has produced groundbreaking discoveries and lifesaving treatments that have shaped human health. Decades of sustained investment from the public, private, and philanthropic sectors have secured this position. While America faces challenges from fragmented systems, outdated processes, lack of coordination, and diminished federal investment, other nations are rapidly expanding their life sciences capabilities. Without modernization, the US risks ceding its leadership role and the economic and health benefits that flow from it.

This report outlines policy recommendations to preserve and strengthen US leadership in biomedical research and innovation. It offers near-term steps to streamline processes and remove barriers, alongside long-term reforms to build a stronger, more resilient ecosystem. The recommendations aim to ensure that scientific progress can be translated into real-world benefits for patients. With sustained leadership, collaboration across sectors, and investment, the US can continue to deliver breakthroughs to secure a healthier future for the world.

Recommendations

1 The Life Sciences Sector Is a Strategic National Asset

RECOMMENDATION 1 Commission an independent National Life Sciences Strategy and Implementation Plan

2 A National Health Data Infrastructure Should Power Research and Innovation

RECOMMENDATION 2A Establish national standards for data quality

RECOMMENDATION 2B Build a federated interagency data ecosystem

RECOMMENDATION 2C Launch national data missions to bring public and private sector data together through a federated learning network

RECOMMENDATION 2D Expand access to Centers for Medicare & Medicaid Services (CMS) and Food and Drug Administration (FDA) data for research and innovation

3 Every American Should Be Able to Control, Share, and Benefit from the Use of Their Data

RECOMMENDATION 3A Launch a national public educational campaign on the power of health data to enable medical breakthroughs

RECOMMENDATION 3B Establish a patient-controlled health data wallet pilot project

RECOMMENDATION 3C Expand the Trusted Exchange Framework and Common Agreement (TEFCA) to empower patients to contribute data for research purposes

4 Every American Should Have the Opportunity to Participate in Clinical Research

RECOMMENDATION 4A Establish a national agenda for clinical trials

RECOMMENDATION 4B Invest in national clinical trial infrastructure

RECOMMENDATION 4C Make it easier for patients and clinicians to participate in clinical trials

RECOMMENDATION 4D Reduce the administrative burden of conducting clinical trials

5 The Federal Government Should Be an Attractive Destination for the Biomedical Workforce

RECOMMENDATION 5A Create a National Biomedical Service Corps for students to receive educational support in exchange for federal service commitments in critical areas

RECOMMENDATION 5B Expand hiring and pay flexibilities and rotational programs to enable greater mobility between public and private sectors in the biomedical field

6 America's Health Agencies Should Adapt to Meet Today's Challenges

RECOMMENDATION 6A Clarify the Centers for Disease Control and Prevention's (CDC's) mission and role in the biomedical research enterprise

RECOMMENDATION 6B Strengthen the National Institutes of Health (NIH) to drive bold, cross-cutting biomedical research

RECOMMENDATION 6C Establish a regulatory sandbox at the FDA

We present a policy roadmap to preserve US leadership in biomedical research and innovation. Realizing its goals will demand strong leadership, sustained investment, and deep collaboration, guided by the recognition that the life sciences industry underpins the nation's future health, prosperity, and security.

The task ahead is to work with Congress, the administration, federal agencies, and the public to determine the most effective levers, allocate the necessary resources, and design a phased approach for moving forward.

Introduction

The US biomedical research and innovation enterprise has long been a source of global leadership, scientific breakthroughs, and lifesaving treatments. Decades of investments in research and development have positioned the US as the world's hub for biomedical discovery and innovation.

Today, the US stands at a critical juncture. Other nations are investing heavily in their life sciences sectors, while fragmented systems, outdated processes, lack of coordination, and inconsistent investment threaten to slow America's progress. Health outcomes in the US are below those of peer nations despite a spending level nearing 20 percent of GDP. FasterCures has engaged leaders across the public, private, and philanthropic sectors to examine what it will take to preserve and strengthen US leadership in life sciences and to produce the advances needed to deliver more effective and affordable health care.

Past efforts, such as FasterCures' convening on barriers to progress in 2011, highlighted the need for bold action. The imperative has grown since. The US must modernize its biomedical research and innovation ecosystem to ensure that it continues to lead in scientific breakthroughs and that effective interventions can be translated into real-world benefits for all Americans—or risk losing its global position to other countries.

This report presents a set of policy recommendations as a starting point for dialogue and action. We recommend near-term actions to streamline processes and reduce barriers, as well as longer-term reforms to build enduring infrastructure. From this foundation, leadership, cross-sector collaboration, and investment will be essential. The dividends will be clear: better health for all and a stronger, more secure future for the nation.

Vision

The American story is the story of scientific and technological achievement. Bold US investments in scientific discovery and innovation, from the Apollo space program to the Human Genome Project, led to societal breakthroughs that firmly established US leadership in the sciences.

But history teaches that future success is far from guaranteed. As countries around the world invest heavily in genomics, biotechnology, and artificial intelligence (AI), America faces a pivotal moment. It has been said that the 21st century will be the century of the life sciences. The US's ability to prevent disease before it starts, treat and cure it when it strikes, and extend the years of healthy living will determine whether the US can sustain and expand its leadership.

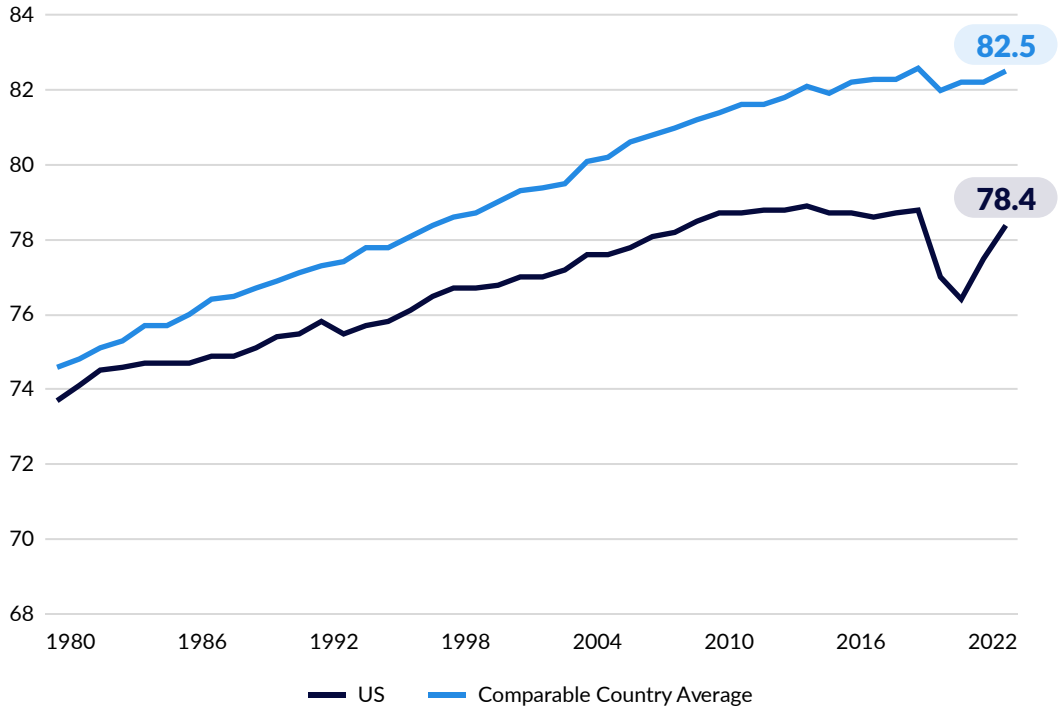
How will America lead? Finding an answer is not just about science but about health, prosperity, and security. Our vision is for the US to sustain its global leadership in biomedical research and innovation that leads to breakthroughs in health, drives strong economic growth, and maintains our national security.

Improving Population Health

Scientific advances, from antibiotics to genomic sequencing, have transformed medicine and public health. But the challenges ahead are profound. The US ranks poorly in life expectancy and health among peer nations, despite leading the world in scientific innovation (see Figures 1–3). An aging population, the growing burden of chronic disease, and persistent inequities in quality of care and access demand renewed national attention.

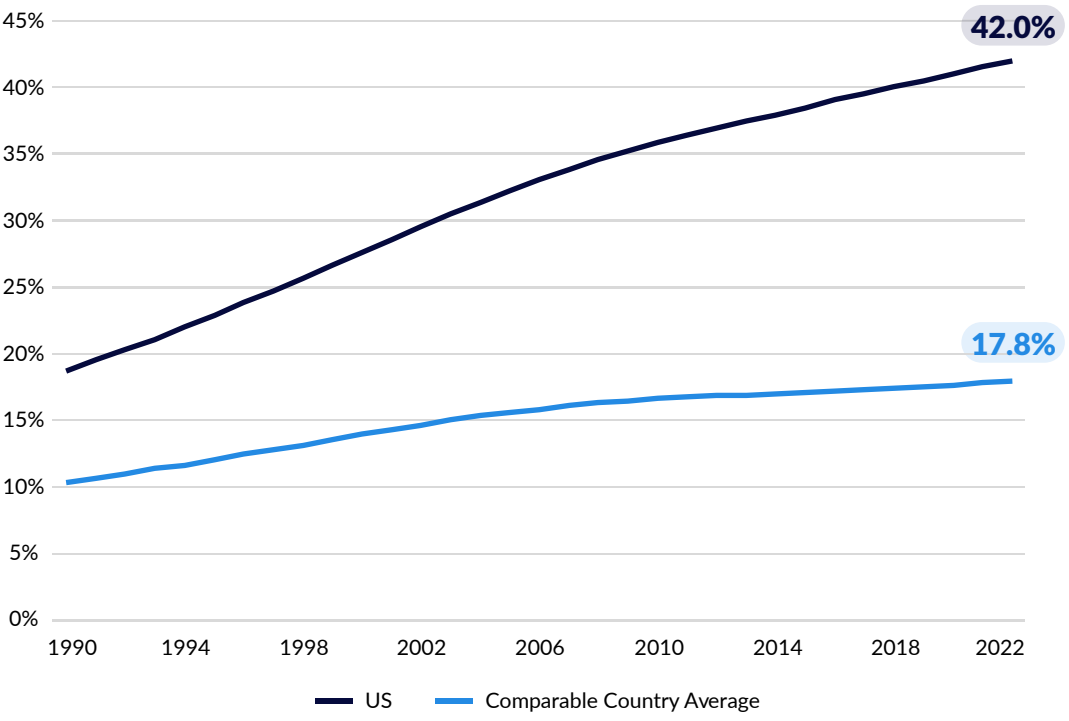
Strategic investment in the life sciences can be a powerful response, spurring advances that enable innovative prevention strategies, earlier disease detection, and more effective therapies. With AI-driven drug discovery and development, genomic advances, and digital technologies, the potential to extend life expectancy and the number of years spent in good health for all Americans is at our fingertips.

Figure 1: Life Expectancy Is Far Below That of Peer Countries



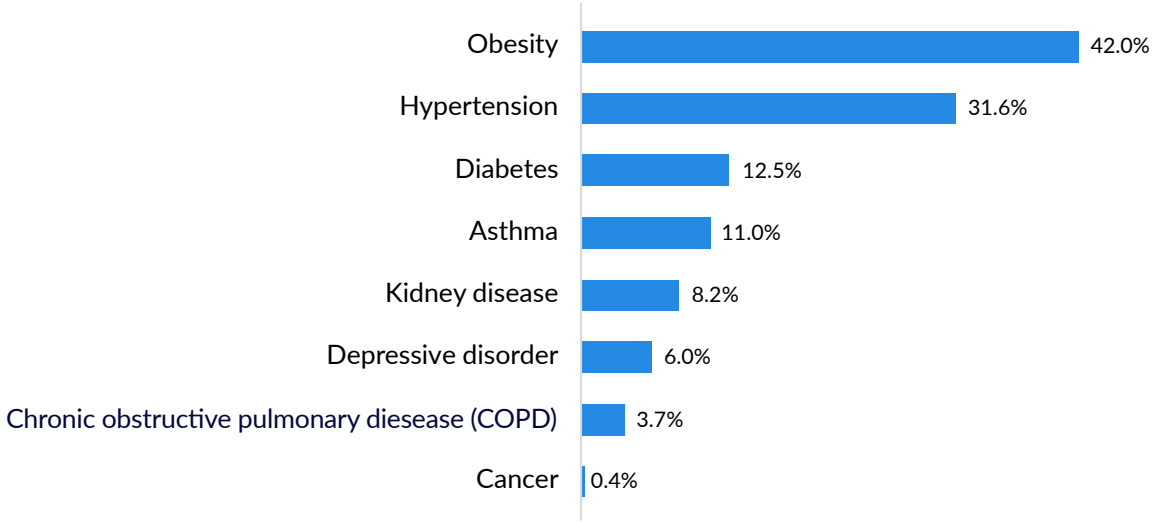
Source: Peterson-KFF Health System Tracker, based on KFF analysis of US Centers for Disease Control and Prevention; Organisation for Economic Co-operation and Development; Australian Bureau of Statistics; German Federal Statistical Office; Japanese Ministry of Health, Labour, and Welfare; Statistics Canada; and UK Office for National Statistics data (2025)

Figure 2: US Obesity Rate Is More Than Twice That of Comparable Nations



Source: Peterson-KFF Health System Tracker, based on KFF analysis of World Health Organization data (2025)

Figure 3: Share of the US Population Experiencing Chronic Diseases



Notes: Data represent age-standardized share of the US population who experience chronic diseases. Obesity, diabetes, and cancer are from 2022 data. Hypertension data are from 2019. Asthma, kidney disease, depressive disorder, and COPD data are from 2021.

Source: Peterson-KFF Health System Tracker, based on KFF analysis of World Health Organization data and Institute for Health Metrics and Evaluation Global Burden of Disease data (2025)

Driving Economic Growth

The life sciences sector is not only focused on improving health but is also a cornerstone of economic vitality. Biomedical industries generate millions of jobs, bolster regional economies through research hubs and academic medical centers, and fuel the creation of new businesses (see Figure 4, for example).

For every dollar the federal government has invested in basic research, the private sector has multiplied it several times over. These investments catalyze start-up ecosystems, spawn new industries, and expand domestic capacity. By leading in life sciences and fostering fertile and stable ground for innovation and entrepreneurship, the US can continue growing its economy sustainably, benefiting every region while lifting the health of its population.

Figure 4: NIH Research Supports Jobs and Fuels the Economy

Source: Adapted from United for Medical Research (2025)

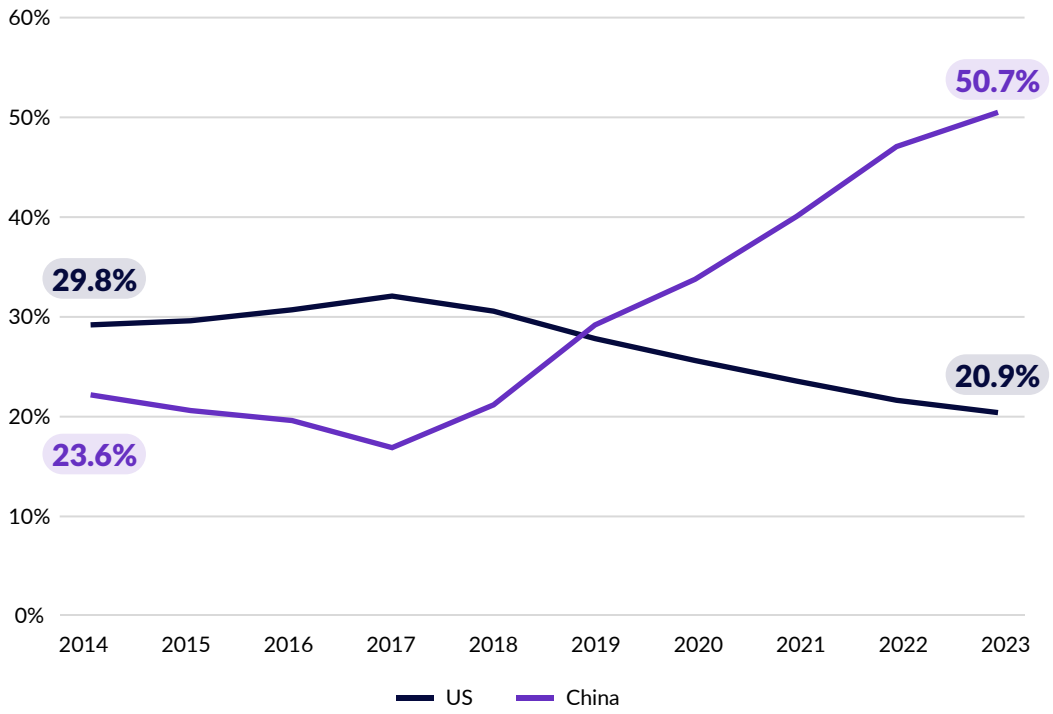
Maintaining National Security

China, Singapore, the United Arab Emirates, the United Kingdom, Australia, and others recognize the stakes and are developing long-term strategies to lead the next era of life sciences. These efforts combine long-term planning and significant public-private investments, aimed at capturing global market share in life sciences.

These countries recognize that leadership in life sciences is vital to national security and global influence. If the United States fails to match urgency with investment, it risks ceding economic advantage and the ability to set international standards for scientific quality, regulatory frameworks, and ethics. Most importantly, US patients may be left behind, waiting for new treatments and cures. (See Figure 5, for example.)

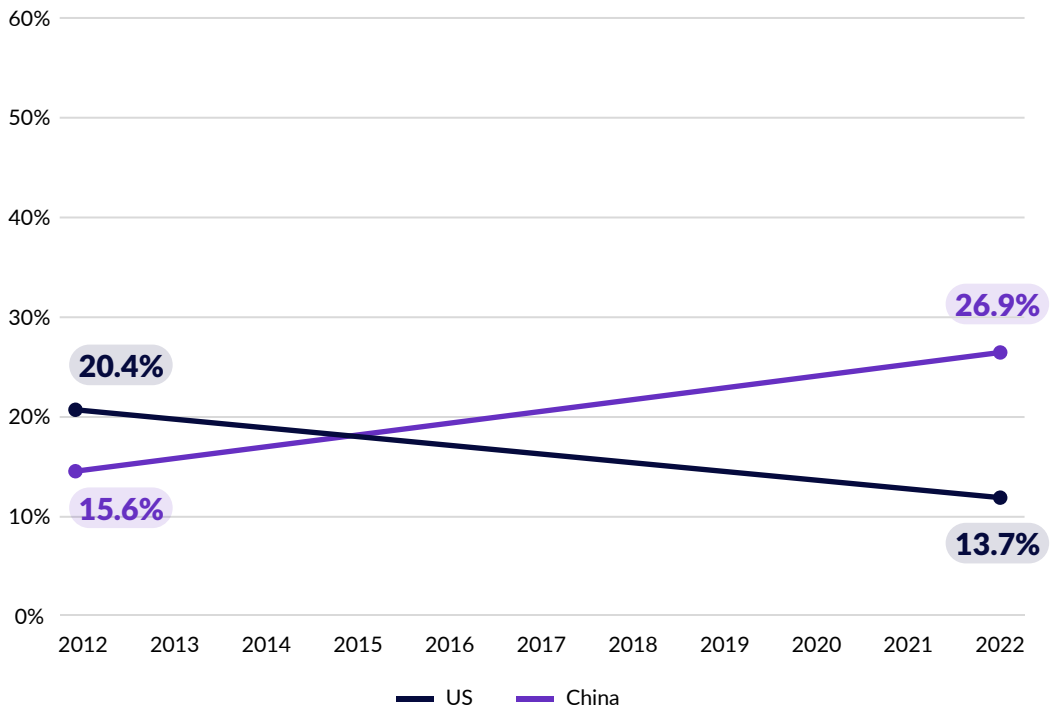
Figure 5: Percent of World Biotechnology Patents and Scientific and Engineering Publications

5a: Percent of World Biotechnology Patents (2014–2023)



Source: World Intellectual Property Organization (2025)

5b: Percent of World Scientific and Engineering Publications (2012–2022)



Source: National Science Foundation (2025)

Key Considerations

Our recommendations aim to sustain and expand US leadership in biomedical research and innovation, an area that underpins better health, economic prosperity, and national security. They are intended to stimulate debate, provide options, and outline a path forward.

Translating them into the real world requires careful examination of funding needs, statutory authority, and institutional capacity. Some proposals can be advanced at the discretion of agencies, while others would require direction from the executive branch or congressional authority and appropriation.

How to allocate finite resources is an important question. Building national infrastructure, supporting workforce pipelines, and establishing pilots, as we propose, requires bold vision and thoughtful budget strategies. But these recommendations are not intended to call solely on government resources. America's leadership in biomedical research and innovation is built on strong private and philanthropic sectors. Moving forward, it is crucial that these sectors work together and alongside the government.

The next step is to examine, in partnership with Congress, the administration, federal agencies, and the public, which levers to pull, which resources to commit, and how to phase in change in a way that is feasible yet transformative.

Recommendations

A set of guiding principles can help chart the course for America's biomedical ecosystem in the years ahead. At the outset, the life sciences sector is a strategic national asset, critical to health, economic growth, and national security.

Maintaining this position has requirements:

- High-quality data platforms must drive biomedical research and innovation.
- Individuals understand the value of their data in driving innovation and benefit from its use.
- Clinical trials must be an available option to all Americans.
- The federal government attracts and retains top biomedical talent.
- Federal health agencies must continually evolve and innovate to meet today's challenges.

The following six sets of recommendations aim to translate these principles into actions.

1. The Life Sciences Sector Is a Strategic National Asset

Past

For decades, the US developed national strategies that recognized biomedical capabilities as essential to national security and prosperity but stopped short of presenting a singular, unified life sciences vision.

A pivotal moment came in 2018 when Congress, through the FY 2017 National

Defense Authorization Act, mandated the creation of a National Biodefense Strategy. The strategy's focus was on biological threats, but its mechanisms centered on strengthening the science and technology base for life sciences, medical countermeasures, and the infrastructure to support rapid responses. While not a life sciences strategy in name, it made clear that biomedical research and innovation and national security are intertwined.

In 2022, the White House elevated biomedical research through the National Biotechnology and Biomanufacturing Initiative, established by an executive order (EO). The EO called on federal departments and agencies to identify bold goals for the future across health, climate, agriculture, supply chains, and cross-cutting advances. Within health, the initiative outlined five aspirations to accelerate medical breakthroughs: identify novel bioindicators of health and develop at-home diagnostic kits to enable patients to monitor their health, enable precision multi-omic medicine, biomanufacture cell-based therapies, accelerate the bioproduction of therapeutics, and enable scale-up of gene editing systems. The 2022 EO was later rescinded under a 2025 executive action.

Present

A patchwork of national initiatives and agency plans—serving as de facto strategies—guides the US biomedical research agenda. Examples include Healthy People 2030, led by the US Department of Health and Human Services (HHS) Office of Disease Prevention and Health Promotion, which sets population health targets.

Government departments and agencies publish strategic plans and roadmaps, such as the HHS Strategic Plan (FY 2022–2026) and the NIH Strategic Plan (FY 2021–2025), but these documents are often siloed and rarely connected. Within government, biomedical research priorities are set independently by agency funders.

Existing coordination mechanisms, such as the roles of the Office of Science and Technology Policy (OSTP) director and the deputy secretary of HHS, are important in science, technology, and health policy but do not have the mandate or capacity to provide sustained cross-agency leadership specific to biomedical research and innovation. Priorities often shift with leadership changes, resulting in fragmentation across the discovery, development, and delivery continuum, as well as within the workforce, data infrastructure, and clinical research enterprise, all critical enablers of biomedical progress.

Future

The absence of a national strategy for life sciences has implications for US competitiveness.

China, Singapore, the United Arab Emirates, the United Kingdom, Australia, and others have established national life sciences strategies that integrate industrial policy, workforce development, and long-term financing as fundamental components to strengthening their economies.

RECOMMENDATION 1

Commission an Independent National Life Sciences Strategy and Implementation Plan

Congress should commission a comprehensive five-year National Life Sciences Strategy and Implementation Plan for American biomedical research and innovation.

A national life sciences strategy would establish a clear framework for long-term priority-setting and investment. The US could anticipate health needs, scientific breakthroughs, and global competition. Like the National Aeronautics and Space Administration's (NASA's) Decadal Surveys, the strategy would provide Congress, agencies, and the scientific community with a roadmap, signaling priorities to industry, academia, and philanthropy.

Such a strategy would consider disruptive technologies and evolving health threats and identify vulnerabilities that could impede innovation. It would ensure the nation can seize opportunities for breakthrough discoveries while managing risks that could render current approaches obsolete.

Core features of the National Life Sciences Strategy and Implementation Plan would include:

- **Independent expert body.** The group would be housed within a neutral convening body designated by Congress.
- **Independent expert composition with rotating leadership.** Membership would consist of independent experts from science, medicine, industry, technology, academia, philanthropy, and patient communities. Members would be appointed at the direction of Congress. Leadership would rotate among members (e.g., alternating among scientific, technological, clinical, and patient/industry perspectives). Federal agencies would not serve as voting members, but liaisons from OSTP, CDC, NIH, the FDA, the Department of Veterans Affairs, the Department of Defense, and CMS would participate for visibility, data, and technical input.
- **Chair of the plan.** The members of the independent expert body would select a chair. The chair would be accountable for delivering the strategy and implementation plan.
- **National coordinator for implementation.** Congress would establish a national coordinator for life sciences strategy within HHS to facilitate agency responses. The coordinator would ensure that recommendations are systematically reviewed and, where feasible, integrated into agency planning and budget processes.

NASA Science Decadal Survey

Purpose: To establish a 10-year consensus-driven set of scientific priorities for planetary science, guiding NASA mission planning, investments, and cross-agency coordination

Commissioned by: NASA

Conducted by: National Academies of Sciences, Engineering, and Medicine

Inputs:

- Scientific community
- Public workshops/town halls
- Expert panels/subcommittees
- Cost and technical assessments

Update cycle: Every 10 years, with a mid-cycle update

Informs:

- Which missions get proposed, approved, or delayed
- How resources get allocated across competing missions

- **Federal oversight.** The strategy and implementation plan would be submitted to Congress every five years, and Congress would hold briefings on the strategy and the status of the plan.
- **Reassessment mechanism.** Recognizing that major scientific and technological breakthroughs can rapidly shift priorities, the coordinator would include a mid-cycle update mechanism, modeled on the NASA Decadal Survey. The update enables course corrections without undermining long-term investment commitments. HHS could request ad hoc updates from the chair if a breakthrough discovery or major disruption arises outside of the scheduled updates.
- **Public report card.** The independent expert body would develop a framework and metrics to ensure transparency, which would serve as a report card to the American people on progress against the strategy. The Government Accountability Office (GAO) would audit progress against these indicators and publish the official public report card, enabling policymakers and the public to see the impact of investments in biomedical research.
- **Strategy components.** The strategy should take into consideration the scientific opportunity, population health needs, and areas critical to economic growth and competitiveness. The strategy should also integrate the following components:
    - **Scientific Grand Challenges.** To accelerate progress on transformative opportunities, the strategy should identify national Grand Challenges in life sciences. It should evaluate innovative financing mechanisms—such as prize competitions and public-private challenge funds modeled after XPRIZES—to address these Grand Challenges. Grand Challenges should not be viewed as incremental projects, but as generational efforts on the scale of the Human Genome Project, requiring bold, coordinated investment and a long-term vision to yield breakthroughs that define the future of medicine. One such challenge could be to build a comprehensive library of cellular proteins, a resource that would fundamentally alter our understanding of biology and reshape drug discovery. How investments in such foundational resources translate into benefit for the ecosystem should be demonstrated and documented.
    - **Workforce alignment.** The strategy should include comprehensive workforce projections that identify emerging skill requirements and potential talent shortages. This analysis should map current educational pipelines against anticipated needs in prioritized areas. These projections should inform recommendations for educational investment, policy adjustments, and skills-based initiatives needed to maintain American competitiveness.
    - **Data gaps.** The strategy should include a systematic assessment of critical data gaps that impede biomedical research and innovation and determine which gaps most constrain progress in priority areas. These findings should inform recommendations for targeted data collection and infrastructure investment.
- **Input processes.** The strategy must be developed with robust input processes, which should include:
    - **Systematic horizon scanning** to identify emerging technologies, evolving health threats, and shifting global competitive dynamics
    - **Public engagement processes** that capture patient and caregiver as well as local and regional perspectives on research priorities
    - **Expert advisory panels** representing diverse disciplines and methodological approaches
    - **International benchmarking** to understand where American leadership is strongest and most vulnerable

2. A National Health Data Infrastructure Should Power Research and Innovation

Past

For decades, US health data have been generated and stored in silos: claims data at CMS, regulatory submissions and adverse event reports at the FDA, public health surveillance data at CDC, and data repositories, disease registries, and knowledge bases at NIH.

Each agency was tasked with building a system to support its mission, with no funding to analyze the data within or across systems. When data became available, they were often through heavily redacted public-use files or bilateral agreements that were often formed through personal relationships. As a result, the nation's most valuable health data resources remained largely stored away and underused.

Present

Federal agencies are accelerating data modernization with a stronger focus on AI, patient empowerment, and interoperability. The FDA completed its first pilot of a generative AI-assisted scientific review in May 2025, with plans to deploy AI tools across all centers to accelerate regulatory review. CMS seeks to modernize access through digital tools that empower Medicare beneficiaries to manage their own health. CDC's Data Modernization Initiative is upgrading surveillance systems to enable real-time, nationwide public health insights, while NIH is building large-scale platforms, such as *All of Us* and Data COUNTS, to generate research-ready datasets.

The current system demonstrates momentum but lacks the cohesion or consistency necessary to enable sustained interagency collaboration.

Future

To move beyond fragmented pilots and siloed agency resources, the US needs a coordinated system for health data.

This infrastructure should rest on four pillars: common standards, interagency linkages, expanded access to high-value datasets, and public-private experiments in federated learning. These pillars will strengthen the backbone for a data infrastructure capable of fueling biomedical innovation that meets the needs of people across the nation.

RECOMMENDATION 2A

Establish National Standards for Data Quality

Physicians and other providers have long been the primary source of health data (e.g., electronic health records, lab results, and public health reports). Most of the data are collected for billing and compliance purposes rather than in service of patient care or research. The result has been errors and inconsistencies in data collected, incomplete records, and delays in reporting.

Federal networks need substantial investment to clean and harmonize provider-generated data. Meanwhile, providers, who remain the frontline generators of health data, face rising burdens complying with a patchwork of duplicative reporting requirements. Interoperability rules have improved the infrastructure for data exchange. Yet, much of what flows through that infrastructure remains of low quality and is largely unusable. Without national standards and appropriate incentives, including financial incentives, data quality will continue to vary widely, especially in smaller and under-resourced practices, and the burden remains on clinicians.

Congress should clarify and strengthen HHS's authority to establish national data-quality standards that set baseline expectations for timeliness, completeness, and accuracy of data. These standards should also support and incentivize providers to automate cleaning, deduplication, and record linkage at the point of data entry. To make this framework implementable, the federal government should provide grants to under-resourced providers to upgrade systems, offer technical assistance to help providers meet standards, and create incentives that reward providers for contributing high-quality, analysis-ready data.

RECOMMENDATION 2B

Build a Federated Interagency Data Ecosystem

The federal government should link the strengths of CDC, NIH, FDA, and CMS into a health data ecosystem built on a federated model. Today, each agency maintains valuable datasets, including CDC's public health surveillance data, NIH's research repositories and knowledgebases, the FDA's safety and adverse event data, and CMS's claims. However, these resources are siloed and inconsistently connected. Thus, analyses that require combining evidence, such as linking regulatory safety data with real-world claims, are often insufficient.

To overcome this challenge, HHS should build on ongoing modernization efforts across HHS, such as Data COUNTS, and establish a coordinated interagency platform that enables federated analytics across agency data. This platform would allow secure queries across multiple datasets while the data remain under the stewardship of each agency responsible for its use. Common standards for access, privacy, and interoperability would ensure consistency, and privacy-preserving technologies would protect patient trust.

By transforming today's fragmented platforms into a federated, interagency data ecosystem, the US could combine the full breadth of federal health data without creating a single data warehouse, unlocking national-scale insights while respecting agency authority.

RECOMMENDATION 2C

Launch National Data Missions to Bring Public and Private Sector Data Together Through a Federated Learning Network

The White House, through OSTP and the HHS secretary, should establish a national framework for data missions—large-scale, time-bound initiatives that unite government, industry, academia, philanthropy, and patient groups to tackle urgent health challenges such as antimicrobial resistance, maternal mortality, or rare diseases.

Within this framework, at least one to two missions should be launched each year. These missions provide a steady commitment to action, deliver timely results, and serve as building blocks for the country's data infrastructure.

Missions would be enabled by a federated learning network, under which hospitals, research institutions, and companies would collaborate on analysis and AI model development without moving source data from their secure environments. This approach would protect privacy and preserve local control, while allowing insight sharing on a national level.

Each mission would harmonize multimodal data (e.g., clinical, imaging, genomic, behavioral, and environmental) under common rules for quality, de-identification, and record linkage. Beyond producing actionable insights for the mission at hand, the missions would leave behind reusable assets, such as governance frameworks, contractual templates, and technical standards that could be leveraged, lowering barriers for future efforts.

RECOMMENDATION 2D

Expand Access to CMS and FDA Data for Research and Innovation

CMS and FDA should expand access to their high-value datasets (e.g., Medicare and Medicaid claims, quality reporting data, adverse event reports, clinical study reports, and labeling and coverage decisions) by moving beyond small pilots and by using modern tools, including AI, to make them more usable, timely, and secure for researchers and patients.

Building on initiatives like CMS's Virtual Research Data Center (VRDC) and FDA's openFDA application programming interfaces (APIs), the agencies should strengthen these platforms to improve the timeliness of updates, expand dataset coverage, and enhance usability and analytics tools. The next generation of VRDC and openFDA should enable researchers to securely analyze data in place, supported by AI tools that automate de-identification and quality checks. Proprietary commercial information and patient privacy can be protected, while still making high-value data broadly usable to researchers. These efforts could be undertaken under existing statutory authorities but would likely require congressional appropriations to modernize and sustain securely and at scale.

3. Every American Should Be Able to Control, Share, and Benefit from the Use of Their Data

Past

Federal initiatives made strides in empowering patients to access their own health information. The shift from paper charts to electronic health records gave millions of Americans the ability to view, download, and transmit their medical data. Programs led by the Office of the National Coordinator for Health Information Technology (ONC), such as Meaningful Use and HealthIT.gov, reinforced that patients should be the subjects of health care and also active participants in managing their information.

Most efforts concentrated on individual access rather than collective impact. While patients were encouraged to log into portals and take charge of their records, there was little education about how sharing data more broadly could accelerate discovery, improve treatments and the quality of health care, and strengthen public health.

Communication during the COVID-19 pandemic illustrated the importance of health data for urgent decision-making. But outreach efforts were fragmented and temporary, rather than designed to foster lasting trust and participation.

Past efforts empowered patients as consumers of their data but stopped short of empowering them as partners in research, health-care quality, and innovation.

Present

Programs like CMS's Blue Button 2.0 allow Medicare beneficiaries to securely share their claims data with clinicians, apps, and researchers of their choosing, giving patients real control over how their data are used. The NIH *All of Us* Research Program engages participants as partners in building a diverse national dataset, while initiatives like Trusted Exchange Framework and Common Agreement (TEFCA) lay the groundwork for nationwide data exchange.

Yet, although each of these efforts delivers a component of what is needed, they lack the impact that an integrated approach can bring. The US also lacks a national campaign to explain how patient-contributed data fuel breakthroughs. Without a shared narrative and aligned incentives, patients are left with inconsistent messages and limited opportunities to see the impact of their choices.

Future

The moment is ripe to evolve empowerment from providing access or allowing downloads only to actively shaping biomedical progress through education and consent-driven tools.

RECOMMENDATION 3A

Launch a National Public Educational Campaign on the Power of Health Data to Enable Medical Breakthroughs

Congress should authorize and fund a dedicated, public-facing education initiative to lead a national campaign on the value of health data to medical research and care. This initiative could be housed within a federally chartered organization.

The focus of the initiative would be to build public understanding of how health data enable discoveries that improve health and prevent, treat, and cure diseases more effectively and efficiently. Governance of the initiative would be overseen by an advisory board, led primarily by patients and community representatives, supplemented by experts in research, clinical care, and ethics.

Federal agencies, such as NIH, FDA, CMS, CDC, and Assistant Secretary for Technology Policy/Office of the National Coordinator for Health Information Technology (ASTP), would serve as liaisons to ensure consistency and alignment. The campaign's deliverables would combine national resources with deep community partnerships. A central hub would provide plain-language explainers, myth-busters, case studies using multimedia, and multilingual materials.

Locally based, trusted messengers—including patient organizations, community health centers, libraries, and faith-based groups—would not only cocreate materials on widely used platforms, such as social media, but also serve as critical community partners to demonstrate the value of engagement in research. Other disciplines, including communications and marketing, should also be leveraged. CDC, NIH, FDA, and CMS could be tasked with curating compelling case studies through dynamic video messages illustrating the impact of data sharing to show the public how their contributions have enabled biomedical progress.

RECOMMENDATION 3B

Establish a Patient-Controlled Health Data Wallet Pilot Project

The vision for a health data wallet is to provide every individual in America with a secure tool to aggregate their health information from multiple sources, manage access with dynamic consent, and enable trusted sharing for care and research. Such a wallet would return value to patients by providing insights into their disease and clinical trial opportunities, reduce friction for providers and payers, and create higher-quality, patient-consented datasets for research. Use of a trust broker approach to patient-clinician-researcher communications would allow patients, particularly those with rare or intractable conditions, to communicate their desire to be contacted if opportunities for research become available to them.

Consider digital banking wallets, which enable customers to securely and safely aggregate financial assets, receive real-time information, and transact instantly. They drive efficiencies in the financial ecosystem by reducing transaction costs, accelerating payments, and providing liquidity. Similarly, when patients control their health records and can share them seamlessly with consent, it reduces waste from duplicative tests, enables companies to develop more effective therapies, and helps researchers efficiently recruit for trials.

HHS should commission a neutral convener to design and test a pilot project for a patient-controlled health data wallet. Existing initiatives provide a strong foundation: CMS's Blue Button 2.0 gives Medicare beneficiaries API-based access to their claims data, TEFCA is establishing a nationwide framework for health data exchange, and NIH's *All of Us* Research Program and Data COUNTS initiative are piloting new models for large-scale, patient-consented data sharing. Developing a proof of concept would leverage existing federal initiatives and convene patients and other experts to develop a governance framework and technical model.

RECOMMENDATION 3C

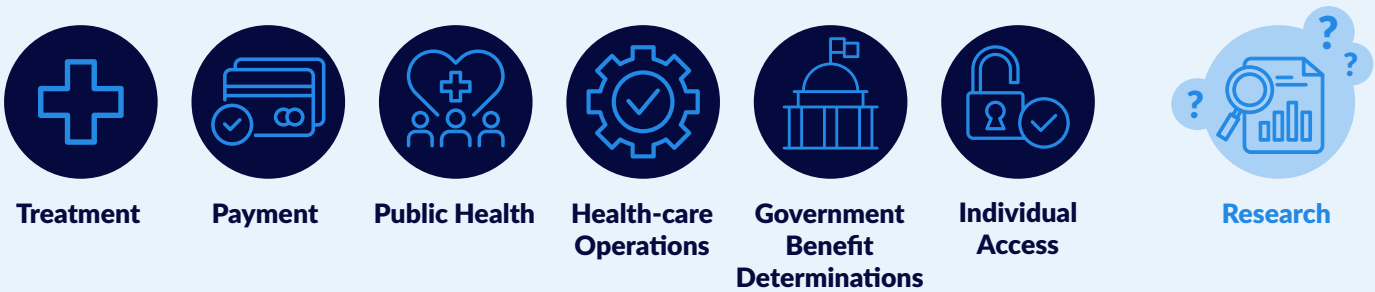
Expand TEFCA to Empower Patients to Contribute Data for Research Purposes

TEFCA enables the nationwide exchange of health information for treatment and care delivery, but its current scope falls short of encompassing research. Congress should expand ASTP's statutory authority to include research as an exchange purpose. Expanding TEFCA to include research would enable individuals to authorize data sharing directly from their providers, payers, and health systems into secure research environments.

Paired with a patient data wallet and incorporating privacy-preserving safeguards and dynamic consent, TEFCA could facilitate the transfer of patient data into studies and clinical trials, eliminating the need for patients to navigate multiple systems or undergo burdensome consent processes. This shift reframes TEFCA as a technical standard for interoperability and as a trusted infrastructure to empower patients to participate in and shape biomedical research.

TEFCA currently supports treatment, payment, public health, health-care operations, government benefit determinations, and individual access (see Figure 6). By adding research as a purpose of health data exchange, the US can accelerate data collection for evidence generation and ensure that national research efforts reflect the lived experience of those most affected.

Figure 6: Health Information Exchange Under TEFCA—Is Research the Missing Component?



Source: Milken Institute (2025)

4. Every American Should Have the Opportunity to Participate in Clinical Research

Past

Clinical trial enrollment in the US has historically been low, with fewer than 10 percent of Americans ever participating. Multiple barriers have contributed to this low participation rate. For many patients, especially those living in rural areas and in communities with limited or no research infrastructure, trials are geographically out of reach, with many trials occurring at academic medical centers. Practical challenges such as transportation, childcare, or time away from work are also barriers to patients enrolling in a clinical trial. Restrictive eligibility criteria, incentivized by the need for speed in regulatory submissions, further limit participation by excluding large segments of patients, including older adults and individuals with comorbidities.

Past strategies to address the low participation of individuals in clinical trials have relied on limited pilots and siloed efforts within academic institutions or tailored efforts by product sponsors to support regulatory review. None of these efforts have borne fruit at a national scale.

Present

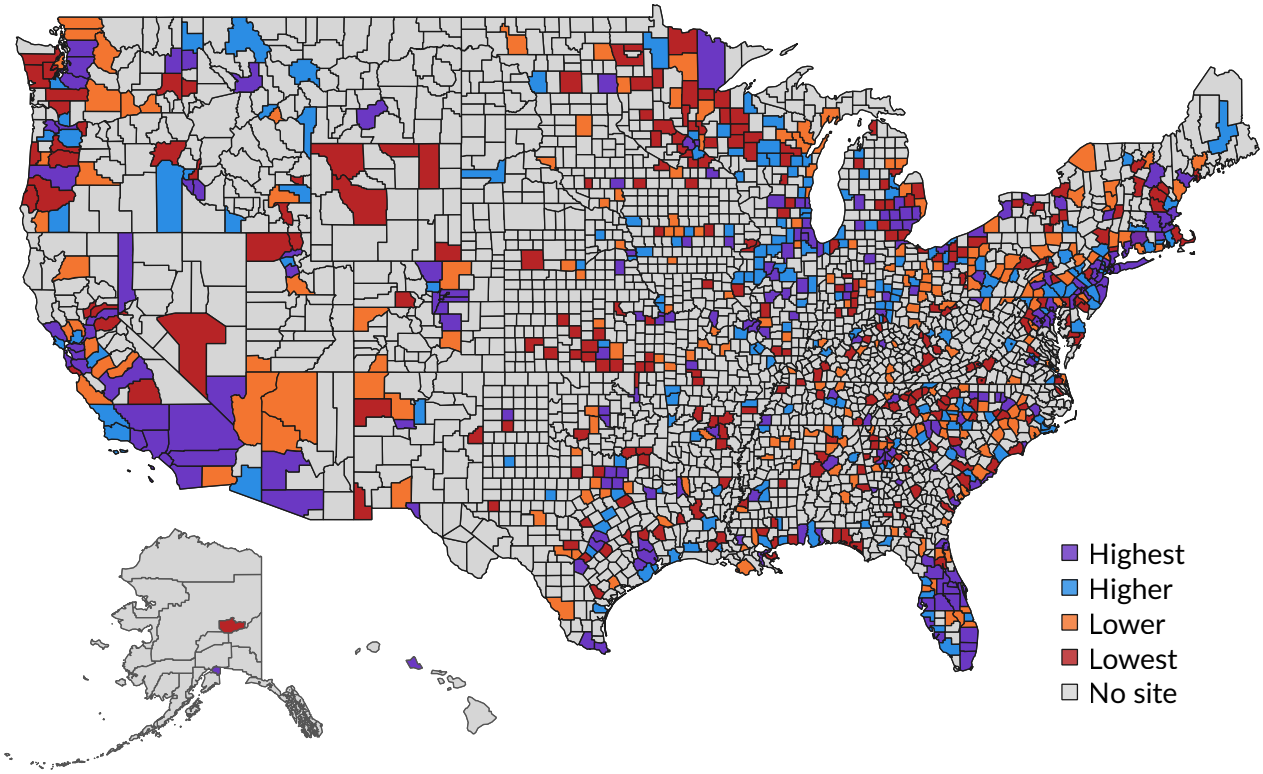
The US clinical trial system is undergoing a transition period, with strong competition worldwide, as other countries close the gap in attracting clinical trials. The COVID-19 pandemic demonstrated the power of tools that decentralize trials (e.g., telehealth, remote monitoring, lab collection in community-based settings, and home visits) in reducing travel burdens and bringing research closer to patients.

These approaches, reinforced by the FDA's 2024 guidance "Conducting Clinical Trials with Decentralized Elements," are increasingly seen as part of mainstream research. At the same time, the FDA advanced policies to improve informed consent and encourage the use of electronic consent, while also encouraging sponsors to broaden eligibility criteria to ensure that trial populations more closely reflect real-world patients.

The regulatory landscape for clinical research remains fragmented, despite progress. Oversight of Institutional Review Boards (IRBs) and informed consent is split between the FDA and the HHS Office for Human Research Protections (OHRP), creating overlapping rules that lead to duplication. While each serves its unique purpose, alignment and harmonization to create consistency and predictability in requirements are crucial to fostering efficiency. Inconsistent reporting, duplicative safety reviews, and lengthy contract negotiations further slow trials.

Despite federal efforts to promote reliance agreements and templates, many institutions insist on tailored legal language. These inefficiencies drive up costs and complexity. Large academic centers often face delays and administrative burdens, while smaller and community sites may find participation infeasible. As a result, trial opportunities remain concentrated in a few well-resourced institutions, limiting broader access (see Figure 7).

Figure 7: Density of Clinical Trial Sites, 2023



Sources: AACT Database (2025), Milken Institute calculations (2025)

Future

Clinical trials in the US must expand patient participation and address inefficiencies that hinder sponsors, sites, and the nation's capacity to run trials at scale. Traditional experimental trials, required for regulatory review of medical product safety and efficacy, remain essential for establishing causal evidence. The real opportunity for transformation, however, lies in expanding our understanding of how medical products work in the real world. The next generation of US clinical research will need to modernize both pillars: strengthening the traditional experimental model while unleashing the potential of large-scale real-world studies.

RECOMMENDATION 4A

Establish a National Agenda for Clinical Trials

To accelerate this transformation, the HHS secretary should establish a national agenda for clinical trials informed by a public-private partnership that brings together government, industry, academia, health systems, and patient groups. The agenda should set clear priorities for modernizing trial infrastructure and should address long-standing barriers that hinder patient participation and delay trial initiation.

Many of the barriers to clinical research have long been known, but progress has been slow, underscoring the need for a national strategy and coordinated action to move beyond incremental change.

The subsequent recommendations in this section outline key actions that should anchor this agenda.

RECOMMENDATION 4B

Invest in National Clinical Trial Infrastructure

It will require more than incremental fixes to strengthen the nation's clinical trial enterprise. It demands coordinated infrastructure that allows research to be conducted efficiently at scale. The following recommendations outline steps that Congress can take to improve coordination and build transparency into national research capacity.

- **Establish an Office of the National Clinical Trial and Research Coordinator (ONCTRC) within HHS.** Led by a national coordinator who reports directly to the HHS secretary, the office would serve as a central hub to coordinate efforts across federal agencies to improve the efficiency and effectiveness of clinical trials. Its responsibilities would include identifying evidence gaps in areas of high unmet need, developing national research challenges to address those gaps, and providing technical support and resources to new trial sites in community-based settings.
- **Integrate the ONCTRC with other agencies.** The ONCTRC must be tightly integrated with NIH and the FDA from the outset to avoid creating parallel structures. Careful design and sustained interagency collaboration will be essential to ensure that the office accelerates, rather than further fragments, the nation's clinical research ecosystem.
- **Build a national clinical trial and research network inventory.** Today, there is no comprehensive public understanding of domestic research capacity. Information on clinical trial sites is fragmented and inaccessible for coordinated planning. Much of the data are proprietary to private organizations. The ONCTRC would establish a national inventory through a public-private partnership that would identify key gaps, enabling resources to be directed strategically.

RECOMMENDATION 4C

Make It Easier for Patients and Clinicians to Participate in Clinical Trials

Technology is available that can connect Americans everywhere to cutting-edge research opportunities, and there is no reason to delay. The US should continue to build on actions already taken to further ensure that clinical trial participation is accessible to all patients.

- **Broaden eligibility criteria.** The FDA should build on recent guidance that requires exclusions to be scientifically justified rather than based on precedent. Through protocol reviews and exemplar trials, the FDA can encourage broadening participation.
- **Simplify informed consent.** The FDA's 2023 final guidance emphasizes plain language and accessibility. Building on this, the FDA and OHRP should promote standardized, plain-language templates and expand the use of electronic consent (eConsent), which allows for multimedia, multiple languages, and asynchronous review at a patient's own pace. Clear joint guidance can standardize expectations, reduce duplication, and give sponsors and IRBs confidence in eConsent.
- **Clarify reimbursement policies.** When participating in clinical trials, patients face costs related to travel, childcare, and lost wages, but current rules leave uncertainty around permissible reimbursement and routine care coverage, particularly for Medicare and Medicaid beneficiaries. Clear policies from CMS would also support broader adoption and acceptability in private insurance markets. The FDA and OHRP should issue clearer standards to reduce inconsistency, give sponsors greater certainty, and lower barriers for patients.

RECOMMENDATION 4D

Reduce the Administrative Burden of Conducting Clinical Trials

The cost of conducting trials in the US has risen steadily. For large and small trial sponsors and sites, the financial and administrative burden can delay or derail clinical trials, making them infeasible for new entrants, many of which are community-based. To ensure opportunities exist for patients who want to participate in clinical trials, burdens faced by sponsors and sites must be reduced.

- **Clarify overlapping FDA and OHRP requirements.** Joint guidance should eliminate duplication in oversight, standardize informed consent, align definitions of minimal risk, and explore single submissions that satisfy FDA safety reporting and OHRP continuing review.
- **Expand use of single IRBs and reliance agreements.** Although NIH requires single IRBs for most multisite studies and the FDA permits them, their adoption is uneven. The FDA and OHRP should standardize requirements and endorse reliance templates (e.g., the SMART IRB agreement, which is already used by more than 1,400 institutions). International models, such as Australia's National Mutual Acceptance program, show the efficiency gains of shared review.

- **Standardize contracts and liability frameworks.** Multisite trials often stall due to lengthy, customized contract negotiations. NIH- and FDA-endorsed standardized master contracts and template agreements could create a common, trusted framework for sponsors and sites.
- **Clarify Form FDA 1572.** Intended to list those making a "direct and significant contribution" to trial data, the form is now overapplied, forcing every local provider or lab to be included and disincentivizing community participation. To ease unnecessary documentation burdens, the FDA should clearly define who must be listed.
- **Simplify Medicare coverage analysis (MCA).** Each trial site repeats its own MCA to determine which services can be billed to Medicare as routine care. CMS should streamline the process by allowing a standardized MCA for multisite studies or issuing templates to reduce redundancy.

Australia's Ethics Review System

Governance: Human Research Ethics Committees (HRECs) are registered with Australia's National Health and Medical Research Council.

Scale: There are about 200 HRECs nationwide.

Integrated Interview: HRECs evaluate scientific merit and ethics in a single process.

Reliance Framework: Under Australia's National Mutual Acceptance scheme, a single HREC review is accepted across most states and territories for multisite clinical trials.

Additional Elements:

- Formal **risk-based review pathways** for low- versus high-risk studies
- Use of **shared standards and templates**

Impact: Trials launch in 6–12 weeks in Australia, compared to more than four months in the US.

5. The Federal Government Should Be an Attractive Destination for the Biomedical Workforce

Past

The federal government has long been a destination for top talent because of its ethos, stability, and the unique opportunities it offers to shape health and science at a national scale. Agencies like NIH, FDA, and CDC were seen as premier destinations for scientists, engineers, and analysts. Over time, however, structural challenges emerged: As the scientific enterprise continued to advance, the government's workforce struggled to evolve in parallel, particularly given demand for these skillsets in the private sector.

Present

Rapid developments in science and technology are exposing a widening gap between the skills needed to advance and regulate the life sciences sector and the federal government's current capabilities.

Biomanufacturing requires skilled engineers and technicians, genomics and drug discovery depend on data scientists who bridge biology and AI, and cell and gene therapies need regulatory experts, process engineers, and researchers to move discoveries into care. Yet across these and other technical fields, the federal workforce lacks the expertise needed to keep pace.

The shortage extends beyond technical experts. Biomedical progress also relies on program managers, policy analysts, ethicists, data specialists, and health economists who are in short supply.

Budget cuts, hiring freezes, and slow recruitment are driving retirements and deterring new entrants. Other countries are now recruiting US scientists and experts with significant incentives, and many are open to opportunities abroad (see Figure 8).

Figure 8: Percentage of US Researchers Considering Relocating to Another Country

Source: Nature poll (2025)

Future

Strengthening the federal biomedical workforce will require action on many fronts. To remain the leader in life sciences, the US needs reforms to STEM education that better prepare students for science and technology careers, immigration policies that keep the US open to global talent, and funding to support stable career pathways.

Much more can be done to strengthen the federal biomedical workforce; however, this report highlights two options to make the government a more attractive career destination for scientists, technical experts, and the broad range of professionals who support biomedical research and innovation.

RECOMMENDATION 5A

Create a National Biomedical Service Corps for Students to Receive Educational Support in Exchange for Federal Service Commitments in Critical Areas

Building on past precedents, including the National Health Service Corps, Congress should establish a Biomedical Workforce Corps supporting graduate students and professionals in areas identified as critical gaps in the current biomedical workforce.

A national biomedical service corps could be structured around the education-first, service-second model of the Reserve Officers' Training Corps, where participants receive scholarships, stipends, or loan repayment in exchange for committing to a defined period of public service. While addressing financial barriers that deter many from pursuing government careers, this approach creates a clear and predictable pathway into federal roles. Placement could be strategically targeted to fill areas of greatest national need. Recruitment should prioritize candidates from geographically diverse institutions to broaden participation in biomedical careers.

For such a program to succeed, it would need broad national commitment and steady support over time. A reliable funding model is essential for maintaining training pipelines and fostering confidence in the program's stability among participants.

Mentorship, professional growth opportunities, and clear career paths would help ensure that federal service is a fulfilling duty and an attractive choice for those who wish to stay beyond the initial term. With sustained support, a biomedical corps could become a lasting pipeline of talent that strengthens the federal workforce and accelerates the translation of discovery into health impact.

RECOMMENDATION 5B

Expand Hiring and Pay Flexibilities and Rotational Programs to Enable Greater Mobility Between Public and Private Sectors in the Biomedical Field

Agencies often lag in hiring for emerging fields because lengthy processes and rigid pay scales make it difficult to compete with industry. The federal government should create stronger mechanisms for career mobility between government and the private sector to accelerate knowledge transfer and innovation.

A recent example can be found in ARPA-H, which required the rapid recruitment of talent from the private sector to launch its programs. It drew on a wide array of special hiring and pay flexibilities to do so (see Table 1). While some have questioned the high salaries associated with these flexibilities, they underscore a larger challenge: Federal compensation systems are poorly aligned with today's scientific and technical labor market, forcing agencies to rely on exceptions.

To build the workforce of the future, agencies must proactively identify, adapt, and leverage the hiring tools available to them, ensuring that talent can move fluidly between sectors without losing career momentum.

Table 1: Hiring and Pay Flexibilities Used by ARPA-H to Hire Its Workforce

Hiring Authority	Description
Title 5 Hiring Authorities	Standard federal hiring mechanisms (e.g., direct hire for critical shortages, promotions, transfers, reinstatements)
Schedule A Excepted Service	Flexible appointments outside the competitive service, often used for fellowships, temporary positions, or targeted categories (e.g., individuals with disabilities)
Agency-Specific Authority (Consolidated Appropriations Act, 2022)	Unique ARPA-H authority to appoint scientific, medical, and professional staff without regard to Title 5 civil service rules, providing maximum hiring flexibility
Public Health Service Special Consultant Authority (42 U.S.C. § 209(f))	Enables temporary or limited-term appointments of outside experts as special consultants, bypassing standard civil service restrictions
Pay Flexibility	**Description**
Recruitment Incentives (5 U.S.C. § 5753)	One-time payments to attract highly qualified candidates to positions that are difficult to fill
Advanced Rates of Pay (5 U.S.C. § 5333)	Authority to set starting salaries above the minimum pay rate based on superior qualifications or special agency needs

Source: GAO (2024)

A rotation framework could also benefit both sectors. The government gains access to cutting-edge expertise, while industry gains clarity on priorities and partnership opportunities. These exchanges would build trust and enable more collaborative relationships over time. Options to structure such rotations include:

- Expanding exchange programs by broadening the Intergovernmental Personnel Act to include private entities alongside universities and nonprofits
- Scaling fellowship pathways by adapting existing models to support structured public-private rotations
- Modernizing incentives and benefits to ensure portability of retirement credits, health coverage, and leave and to narrow disparities between federal pensions and private 401(k) plans

6. America's Health Agencies Should Adapt to Meet Today's Challenges

RECOMMENDATION 6A

Clarify CDC's Mission and Role in the Biomedical Research Enterprise

Past

Since its founding, CDC's responsibilities expanded dramatically to include chronic disease prevention, health promotion, environmental health, occupational safety, and emergency preparedness, among other areas, often in response to congressional mandates or changes in national priorities. This expansion broadened CDC's scope and stretched its priorities across too many responsibilities relative to its limited resources.

Present

CDC plays a unique role in the US biomedical ecosystem as the nation's central source of public health surveillance data. CDC's surveillance systems supply essential population-level information that underpins public health interventions and biomedical research.

One notable example is CDC's Muscular Dystrophy Surveillance, Tracking, and Research Network, which enables longitudinal tracking of thousands of people with muscular dystrophies, creating a rich and unique research resource.

CDC's surveillance platforms are indispensable, generating data that few other entities are equipped to produce. At the same time, the agency navigates new pressures: rapidly evolving technologies, rising expectations for real-time data, and the need to integrate information across federal and state systems. These demands underscore the ongoing significance of CDC's surveillance responsibilities and the challenges of sustaining a modern data infrastructure.

Future

Balancing expanding expectations with CDC's core public health mission requires prioritization.

CDC's mission should be the subject of a deliberate review involving Congress, state and local partners, the public health community, the biomedical research community, and the broader public. The aim of the review should be to determine how best to organize and strengthen the nation's core public health functions for the future.

Part of this deliberation must include the country's capabilities in public health surveillance. CDC's surveillance data provide the foundational information on disease burden, prevalence, and disparities. This information guides public health interventions, prevention strategies, and biomedical research. Maintaining and strengthening this surveillance capacity should be a central component of any plan.

Questions should be explored, such as how surveillance data from CDC can inform national priority-setting for investments in biomedical research and innovation, how CDC data can better integrate and work alongside NIH data to monitor and address critical health gaps in the country, and how public health data and health-care data can better serve the public for rapid, real-time information on emerging health threats and unlock a rapid research response. How can CDC surveillance and analysis identify the underlying causes and potential interventions to address the relatively poor health status of the US relative to other high-income countries?

RECOMMENDATION 6B

Strengthen NIH to Drive Bold, Cross-Cutting Biomedical Research

Past

NIH, which began as a single institute, has evolved over decades into the world's largest funder of biomedical research, encompassing 27 institutes and centers. Much of the growth was guided by congressional priorities and the advocacy of patient- and disease-specific communities, which successfully mobilized dedicated attention and resources for many areas of health. This structure produced tremendous benefits but resulted in overlapping missions in some areas and left emerging interdisciplinary fields without a clear home.

Present

NIH is the largest and most influential funder of biomedical research, supporting discoveries that transform medicine and public health. NIH's 27 institutes and centers, each with a distinct focus, enable targeted investment in areas from cancer and cardiovascular disease to mental health, rare diseases, and infectious disease. NIH also supports generations of scientists through its grantmaking, training programs, and intramural research programs.

At the same time, NIH must continue to evolve. NIH's structural focus on diseases and populations reinforces silos that undermine cross-disciplinary collaboration. NIH's intramural program represents about 10 percent of NIH's budget and supports a wide range of research. Over time, however, its portfolio has expanded to areas that blur the distinction between research that is uniquely suited to government research labs and research that could be conducted elsewhere.

NIH's peer review system, while respected worldwide, is conservative. Rigid rules for the peer review process and lack of alignment between scientific review groups and funding institutes can lead to top-scoring applications that may not advance the kinds of innovative or mission-driven research that the funding institute seeks. As such, safe, incremental projects may be rewarded, while bold, high-risk ideas that could yield transformative breakthroughs are undervalued.

Future

Congress should reaffirm NIH's role as the nation's engine for bold and ambitious biomedical discovery.

NIH's intramural research program offers a unique platform for research that requires sustained, government-backed infrastructure, such as novel platform technologies to support broad-based commercial applications. The intramural research program should be reevaluated to ensure it is more fully aligned with this role. The NCATS Intramural Research Program offers a promising model. Instead of the traditional system where individual principal investigators run separate labs, NCATS organizes senior scientists into cross-disciplinary teams focused on tackling bottlenecks in translational science.

In NIH's extramural program, the peer review process should be recalibrated to prioritize transformative, interdisciplinary proposals and create clear pathways for investigators willing to pursue bold ideas. As a first step, study section chairs and reviewers should be oriented to the mission and programmatic context of the funding institute to ensure that decisions align with institute priorities.

The NIH director should have meaningful resources to advance cross-cutting initiatives, whether through a significantly expanded Common Fund or a new discretionary fund aligned with national health priorities.

Finally, Congress should work with NIH to assess any reorganization of the 27 institutes and centers in a transparent manner with full public engagement. Any future organization should align with biological systems, support scientific discovery and basic research, and sustain investments in translation and transformative breakthroughs—enabling NCATS and ARPA-H to achieve their full missions.

RECOMMENDATION 6C

Establish a Regulatory Sandbox at the FDA

Past

The FDA has grown by responding to crises, with each new law adding rules shaped by the science and technology of the time. This approach helped make the FDA the world's leading medical product regulator. However, it has also created a system that can be slow and rigid when new technologies do not fit traditional paradigms.

Present

The FDA leverages demonstration projects to bridge the gap between existing pathways and emerging technologies. It has funded Digital Health Technology for Drug Development pilots to explore digital endpoints, launched the Innovative Science and Technology Approaches for New Drugs program to qualify novel drug development tools outside existing programs, and completed its first AI-assisted scientific review pilot in 2025, with plans to expand generative AI across centers.

These pilots generate valuable lessons but face limits: They are constrained by statutory authority, often context-specific and resource-intensive and frequently fragmented across centers, making it difficult to scale successful models into policy.

Future

The FDA's pilots demonstrate how emerging technologies can be integrated into regulatory oversight, but they are hard to scale.

A regulatory sandbox, a controlled environment where high-promise but uncertain technologies can be tested under modified requirements with close oversight, could provide a more structured and sustainable framework for innovation (see Table 2).

Regulatory sandboxes are most commonly used in the financial sector, where they allow companies to test new payment systems or digital banking tools under temporary flexibilities. In the US, states like Utah and Texas have experimented with sandbox models. Utah offers a broad, cross-sector approach, and Texas focuses mainly on financial services to encourage innovation while maintaining oversight.

Singapore's Licensing Experimentation and Adaptation Programme (LEAP) is a health-sector example, launched in 2018 to support digital health innovation, starting with telemedicine. It allowed providers to operate under temporary, risk-managed flexibility with clear entry and exit criteria, while regulators monitored safety and data safeguards. LEAP generated evidence that accelerated the adoption of telemedicine in that country.

The FDA, in consultation with the HHS Office of the General Counsel, should review whether it has sufficient statutory authority to create and operate a regulatory sandbox. Such a sandbox would allow testing of select high-promise technologies under temporary, tailored requirements, with strict safeguards in place.

Participation would be time-limited, with clear entry criteria, defined graduation or exit pathways, and public reporting of results. Oversight would be risk-based and adaptive, ensuring patient safety while generating evidence to guide future regulation.

Table 2: Regulatory Sandbox Design Elements

Design Element	Description
Eligibility	Defines who can participate in the sandbox. Eligibility should be articulated clearly to ensure a level playing field across all market participants
Governance	Defines the internal operating structure of the sandbox, roles and responsibilities, and key operational processes
Timing	Includes: • Duration of the admission window • Duration of the test
Test Restrictions	Limits to the scope, scale, and/or conduct of the sandbox test to minimize potential harm
Exit	Includes: • Individual test outcomes (e.g., graduation, terminated test) • Program-level key performance indicators • Incorporation of insights and lessons learned into the broader regulatory agenda

Source: Adapted from World Bank (2020)

Conclusion

This report lays out recommendations for the work that lies ahead. Achieving its vision requires leadership, investment, and collaboration, grounded in the shared belief that the life sciences are not just another sector of the economy, but rather the foundation of a flourishing future. In these unprecedented times, the US must invest in health with urgency and ambition.

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