Modernizing Medical Biotechnology Regulation

In its April 2025 report, the National Security Commission on Emerging Biotechnology (NSCEB) recommended creating simple pathways to market (Rec. 2.1a) and preparing regulatory agencies for novel products (Rec. 2.1b). Since the release of the report, the NSCEB conducted extensive stakeholder outreach to identify specific Congressional actions to achieve those outcomes. The NSCEB looks forward to working with Congress, federal agencies, and other stakeholders to implement these policy options, including through legislation, oversight activities, and other efforts.

The United States has been the global leader in medical biotechnology since the 1970s and must modernize its medical biotechnology regulations to maintain its leadership. China's share of the global drug development pipeline has risen to 30%, up from just 6% a decade ago.[1] Developers are increasingly shifting medical research, development, and manufacturing overseas, in part due to slow, unpredictable regulation in the United States. This weakens U.S. competitiveness and delays new treatments for American patients.

Opportunities to Modernize Medical Biotechnology Regulation

Preserving and strengthening American biotechnology leadership will require the Food and Drug Administration (FDA) to modernize its approach to cutting-edge medical products. The FDA divides oversight of medical products across three centers: the Center for Drug Evaluation and Research (CDER) regulates drugs, biosimilars, generics, and over-the-counter products; the Center for Biologics Evaluation and Research (CBER) regulates biologics, vaccines, cell and gene therapies, and blood; and the Center for Devices and Radiologic Health (CDRH) regulates medical devices and radiation-emitting products. This structure has largely been successful for traditional products but is increasingly inefficient when applied to innovative medical products.

Current regulatory frameworks were built for well-characterized, small-molecule drugs. The FDA struggles to adapt to biotechnology-enabled medical products, such as cell and gene therapies.[3]

The FDA's Hybrid Funding Model

In fiscal year 2024, the FDA received about $3.6B in annual appropriations and $3.3B from industry user fees.[2] This hybrid funding model balances stable appropriations with user fees that directly support product review.

- Annual appropriations are not tied to product review. Instead, they fund broader functions, such as outreach and interagency coordination, along with salaries, facilities, and information technology systems. Regular, sustained funding is essential for agency independence and for cross-cutting public-health functions.
- User fees are negotiated by the FDA and industry every five years, then codified by Congress. They establish specific industry fees and regulatory timelines for each process. User fees must be spent on work that is directly linked to product review, such as reviewer staffing, outside consults, and related infrastructure.

In addition, while the FDA's hybrid funding model provides flexibility, it also limits long-term, systemic regulatory improvements and modernization.

The United States now has advanced scientific and regulatory tools to evaluate innovative new medicines produced with biotechnology, but Congress needs to unlock them. Congress must act to reduce unnecessary regulatory burden for medical biotechnology products, and empower and resource regulators to work more efficiently. Adopting these policy options would speed medical product reviews, bolster U.S. competitiveness in global health innovation, and bring safe treatments to American patients faster.

Overview

Policy Options for Modernizing Medical Biotechnology Regulation

Building on the NSCEB's prior recommendations and extensive stakeholder input, this paper describes 22 policy options across five key areas to improve the regulation of biotechnology medical products: ensuring predictable and transparent reviews; conducting faster, fairer clinical trials; hiring, training, and retaining regulators; building a connected FDA; and promoting efficient manufacturing. These policy options should be considered alongside the NSCEB's overarching policy options for modernizing biotechnology product regulation. The NSCEB also developed detailed policy options for microbes, plants, and animals, which are presented in separate discussion papers.

Ensure Predictable and Transparent Reviews

1. Finalize and expand the platform technology designation.
2. Help developers meet data expectations.
3. Align evidentiary standards and review practices.
4. Establish a regulatory sandbox for medical biotechnology products.
5. Validate modern testing methods.

Conduct Faster, Fairer Clinical Trials

6. Require centralized review for multi-site trials.
7. Allow trial designs for small populations.
8. Align endpoints and biomarkers across the FDA.
9. Remove barriers to speedy Phase I trials.
10. Remove barriers to insurance cost sharing.

Hire, Train, and Retain Regulators

11. Target workforce gaps with existing tools.
12. Tie career progression to continuing education.
13. Rebuild the FDA's internal policy capacity.

Build a Connected FDA with Modern Integrated Systems

14. Build a single FDA enterprise system.
15. Leverage FDA data to support innovation and safety.
16. Implement AI-assisted review.
17. Harmonize terminology across agencies.
18. Support strong participation in international standard setting.

Promote Efficient Manufacturing

19. Expand risk-based inspections overseas.
20. Clarify manufacturing requirements.
21. Coordinate country-of-origin labeling.
22. Expand domestic manufacturing capacity and workforce.

Ensure Predictable and Transparent Reviews

Predictable review processes and clear regulatory milestones are essential for securing investment, scaling up manufacturing, and keeping trial sites and patients engaged. Biotechnology developers face uncertainty from uneven timelines, opaque decisions, shifting expectations, and inconsistent processes. These challenges raise the cost of product development, prolong the regulatory process, and delay patient access to new therapies.

1. **Finalize and expand the platform technology designation.**
   Biotechnology enables developers to rapidly build multiple therapies from the same well-characterized platform. However, inconsistency across the FDA limits the reuse of validated components, assays, and manufacturing methods. Additionally, current FDA policy restricts a developer from referencing their own previously submitted information for biologics, resulting in a full review even when a platform has already been evaluated. Congress should instruct the FDA to finalize its draft platform technology guidance and establish a cross-center platform technology designation with uniform criteria, explicit carryover of validated data, and shared standards. This would reduce repetitive testing and review, lower costs, and speed scale-up of medical biotechnology products.

2. **Help developers meet data expectations.**
   Developers lack clear guidance on data expectations and common deficiencies, which leads to delays and multiple rounds of revisions. The FDA has made some efforts to provide clarity, such as by releasing Complete Response Letters that describe why a submission was rejected. However, these letters are heavily redacted, which signals risk to investors while providing little usable guidance to developers. Congress should direct the FDA to publish aggregated, de-identified reports of common deficiencies and to standardize deficiency letters into a four-part structure: what was submitted, why it was insufficient, what is required, and the scientific rationale.[4] Clearer expectations would reduce back and forth between developers and reviewers and shorten the time to approval.

3. **Align evidentiary standards and review practices.**
   Varying standards of evidence and review practices across the FDA result in inconsistent timelines and decisions. Congress should require that the FDA develop uniform definitions for key terms, such as "reasonably likely to predict clinical benefit" and "serious condition." Congress should also require that the FDA develop cross-center guidance that applies these definitions consistently to standardize decisions and reduce uncertainty for developers.

4. **Establish a regulatory sandbox for medical biotechnology products.**
   FDA regulators lack a structured way to test new oversight approaches before applying them across the agency. Congress should direct the FDA to create a "regulatory sandbox" for time-limited trials of new regulatory processes for emerging biotechnology products. These efforts would allow the FDA to evaluate and refine updated workflows, guidance, and regulations before broader implementation. The FDA should then expedite final regulations or guidance based on the results. A regulatory sandbox would also encourage iterative experimentation with digital tools under regulatory supervision, while accelerating learning and de-risking innovation before broader adoption.[5]

5. **Validate modern testing methods.**
   New approach methodologies (NAMs), including predictive tools such as digital twins and organ-on-a-chip systems, can generate safety and efficacy data faster and at lower cost than traditional animal studies. However, these methods lack consistent validation and acceptance across the FDA, limiting their use in regulatory submissions. Congress should direct the FDA and the National Institutes of Health (NIH) to establish clear, science-based validation pathways for NAMs and other predictive tools. The FDA should consistently accept validated methods to reduce redundant studies and support innovation while maintaining a high bar for safety.

Conduct Faster, Fairer Clinical Trials

The FDA's clinical trial expectations are centered on large, randomized trials with thousands of patients. These expectations are increasingly out of step with the realities of modern medicine. Advances in diagnostics and genomics now allow researchers to define diseases more precisely, dividing patients into smaller groups. Large-scale clinical trials with thousands of patients are not possible for ultra-rare diseases that affect only a handful of people in the United States. Rigid regulatory standards that demand

traditional trial designs are unworkable in these contexts. This misalignment between regulatory expectations and clinical realities disproportionately affects rare disease communities and undercuts the very promise of precision medicine. Regulatory flexibility, including alternative endpoints, adaptive trial designs, and conditional approvals, is essential to ensure that scientific progress can translate into patient impact, even when the patient population is measured in dozens rather than thousands.

Operational constraints further undermine clinical trial efficiency. Layered bureaucracy significantly slows clinical trials in the United States and pushes developers to conduct clinical trials abroad. Stakeholders report that recruitment of trial participants continues to be a challenge, despite federal efforts. Other countries, such as Australia and China, are attracting developers due to investigator-initiated pathways and faster patient recruitment. Modern digital health tools can increase patient access to trials, but uptake is slow. Developers also report inconsistent application of standards for endpoints, biomarkers, and data requirements, despite FDA guidance on adaptive trial designs and alternative pathways. The United States needs risk-based, science-driven reforms to accelerate clinical trials and ensure that cutting-edge medical products are available to American patients first.

6. **Streamline multi-site trials.**
   Large, multi-site clinical trials are often delayed because each trial location must go through its own ethics review. This creates repetitive paperwork rather than improving patient protections. The Federal Policy for the Protection of Human Subjects, referred to as the Common Rule, encourages the use of a single Institutional Review Board (IRB) for multi-site studies, but provides exemptions for FDA-regulated trials.[7] Although the FDA has proposed alignment with the Common Rule, the lack of a clear mandate has resulted in inconsistent implementation, and stakeholders report that too few trials use a single IRB. Congress should instruct the Department of Health and Human Services (HHS) to require centralized IRB review for FDA-regulated multi-site trials, with limited exceptions. The FDA and the HHS Office for Human Research Protections (OHRP) should create a clear framework for designating one independent IRB of record for each multi-site trial, with modular consent language to address site-specific needs. Ancillary committees such as pharmacy, radiation safety, biosafety, and conflict of interest could remain local to each site, but run in parallel. Congress should also instruct the HHS to finalize its "Use of a Single Institutional Review Board for Cooperative Research" guidance, and the HHS should provide practical guidance on topics such as insurance, onboarding, and training. This framework and guidance would facilitate central contracting with trial sites to reduce administrative burden and decrease timelines. In addition, Medicaid patients often face barriers in receiving care across state lines. This can prevent eligible patients from enrolling in clinical trials, particularly when trials for rare diseases are offered only at a limited number of sites nationwide.

Clinical Trials Ensure Safety and Efficacy

Before a medical product can reach patients, it must go through a multi-step process to ensure it is safe and effective. Developers begin with early research and laboratory testing, followed by animal studies to assess safety. If results look promising, the product is tested in several phases of clinical trials. Each phase builds on the previous step to reduce risk, gather stronger evidence, and protect patients.[6]

1. **Early Testing:** Identifies promising compounds through laboratory and computational studies to assess basic function and feasibility.
2. **Animal Testing:** Evaluates safety, dosing, and potential side effects before testing in humans. This could take place in animals or new-approach methodologies.
3. **Phase 1:** Tests safety in a small group of patients or healthy volunteers (15 to 30).
4. **Phase 2:** Explores whether the product works and identifies appropriate dosage in a small group of patients (50 to 100).
5. **Phase 3:** Confirms safety and efficacy in a larger group of patients (hundreds or more), using a randomized controlled trial design in which participants are randomly assigned to receive either the new treatment or a comparison treatment.
6. **FDA Review:** The FDA evaluates all data to decide whether the developer can bring the product to market.
7. **Phase 4:** Post-market monitoring identifies rare or long-term effects.

State Medicaid programs should implement pathways, such as expedited or provisional enrollment for clinical trials, to allow residents to participate in out-of-state clinical trials.[8] Together, these actions would speed patient enrollment, reduce the administrative burden of standing up trial sites, and expand the geographic distribution of trials so more people can participate, even if they live far from a major medical center.

7. **Allow trial designs for small populations.** Promising therapies often stall not because they are unsafe or ineffective, but because the required trial structure is mathematically or logistically impossible when only a small number of patients exist. The FDA instituted Rare Disease Evidence Principles (RDEP) to support more flexible trial designs, but developers still face inconsistent acceptance by reviewers, unnecessary meetings, and additional paperwork. Congress should clarify that developers can meet the requirement for "substantial evidence" through other scientifically valid trial designs when large trials are not feasible. Congress should also require that the FDA use formal notice-and-comment rulemaking for the recently-announced Plausible Mechanism Pathway, and, if it proves to have merit, take steps to ensure consistent implementation. These changes would allow more rare disease treatments to become available faster.

8. **Align endpoints and biomarkers across the FDA.** Endpoints and biomarkers are the measurable outcomes and biological indicators used in clinical trials to determine whether a medical product is safe and effective. Inconsistent acceptance of endpoints and biomarkers across the FDA creates confusion for developers and delays clinical trials.[9] Congress should direct the FDA to create a cross-center process for issuing harmonized guidance and to convert relevant review frameworks into binding resources with uniform definitions and expectations for evidence.[10] These actions would standardize expectations and provide the necessary consistency and predictability to speed up trials.

9. **Remove barriers to speedy Phase I trials.** Some countries, including Australia, have a streamlined process for Phase I trials, in which the developer provides a 30-day notice to the regulator, then the trial proceeds unless the regulator objects.[11] In the United States, the Federal Food, Drug, and Cosmetic Act (FFDCA) similarly specifies that a trial may begin 30 days after notice to the FDA, and that the FDA may place a clinical hold if there are safety concerns.[12] However, stakeholders noted that delays in the initiation of Phase I trials are common in the United States, especially compared to some countries such as Australia and China. In fact, some stakeholders reported that they were unaware that current U.S. law already allows trials to begin 30 days after notification. Congress should direct the FDA to apply a risk-based approach to clinical holds for Phase I trials and to limit holds to cases where credible safety concerns are identified. The FDA should also provide clear information to developers about its 30-day notice for Phase I trials. This would enable timely initiation of Phase I trials while maintaining patient safety.

10. **Remove barriers to insurance cost sharing.** Current law requires insurers to pay for the routine costs of care for enrollees in clinical trials, though stakeholders reported that this is a challenge in practice. Specifically, when patients need a treatment and there is no standard of care for the disease, or the experimental treatment is not building upon a standard, insurers cannot easily assess if the treatment is routine or not. The result is that developers bear a disproportionate share of costs to care for trial enrollees. According to stakeholders, cost sharing is particularly important for early trials, when funding is tighter. Stakeholders suggested that other payment models may be more helpful for Phase I trials. Congress should consider new payment models as well as ways to ensure that the existing laws are being implemented to best serve patients and further innovation. Congress should also instruct the Office of the Inspector General (OIG) for the Centers for Medicare & Medicaid Services (CMS) to evaluate the ease of clinical trial enrollment for rare and chronic disease patients in a selection of state Children's Health Insurance Program (CHIP) and Medicaid programs, and challenges in paying for the costs of care for these patients. State Insurance Commissioners should also consider how state requirements may affect this issue. Together, these actions would lower costs for early-stage trials, improve predictability for developers, and support continued innovation in medical biotechnology.

Hire, Train, and Retain Regulators

Persistent staffing shortages and knowledge gaps limit the FDA's ability to review emerging technologies. Review teams often lack needed expertise in rare diseases, cell and gene therapy, and data science. High rates of staff turnover drain institutional knowledge and shift work to less experienced staff. The FDA has piloted training initiatives, such as Accelerating Rare disease Cures (ARC), Rare Disease Evidence Principles, and Support for clinical Trials

Advancing Rare disease Therapeutics (START), but these remain small in scale. Training opportunities are limited, workforce planning is opaque, and capacity for cross-functional policy has eroded.

11. **Target workforce gaps with existing tools.** Persistent staffing shortfalls limit the FDA's ability to review applications efficiently and keep pace with scientific advancements. Congress should direct the FDA to implement a workforce plan with detailed benchmarks and public dashboards that track vacancies, time-to-hire, and retention. The FDA should deploy existing authorities to strengthen its talent pipeline, such as direct-hire, special salary rates, and recruitment and retention incentives. Clear staffing targets would ensure that hiring efforts translate into increased review capacity.

12. **Tie career progression to continuing education.** The FDA struggles to compete with industry for talent, and reviewers often lack experience with the latest scientific advances. Congress should require that the FDA establish a continuing education framework, similar to Continuing Medical Education, that links verified learning credits to promotions, proficiency pay, and leadership eligibility. The FDA should set minimum annual requirements and define eligible activities, such as scientific conferences, workshops, certifications, and interagency rotations. The FDA should also evaluate and expand programs such as its Cell and Gene Therapy Interactive Site Tours and CDRH's Experiential Learning Program. A structured, incentivized training system would strengthen reviewer expertise, improve retention, and close knowledge gaps.

13. **Rebuild the FDA's internal policy capacity.** Critical policy development initiatives such as CDER's Office of New Drugs and the FDA's Rare Disease Council are under-resourced, despite their role in maintaining consistency across the FDA. Reductions in policy staff have slowed guidance updates and constrained activities such as stakeholder outreach and international harmonization efforts. Congress should restore and resource the FDA's policy offices and cross-center councils to accelerate guidance development and improve consistency across programs.

Build a Connected FDA with Modern, Integrated Systems

Fragmented information technology systems and manual workflows slow FDA review, create inconsistencies, and complicate coordination between CDER, CBER, and CDRH. Advances in artificial intelligence and machine learning (AI/ML) offer opportunities to automate routine tasks, strengthen data quality, and streamline review, but only if the FDA has modern, connected infrastructure. Terminology differences across the FDA and other agencies pose further barriers to consistent review. Without concerted efforts, legacy systems and fragmentation will continue to delay reviews and prevent the United States from using the FDA's clinical and manufacturing data as a strategic asset.

14. **Build a single FDA enterprise system.** The FDA has taken steps to standardize and consolidate submissions, but these initiatives remain siloed and incomplete. Congress should require the development of a single FDA enterprise system that unifies its cloud submission infrastructure and integrates AI/ML tools, shared application interfaces, consistent data access controls, and cross-Center analytics. The platform should support machine-readable standards and enable secure operations, such as audit trails and role-based access. A clear transition plan would include staff training, developer outreach, data sharing, timelines, and escalation procedures. By providing resources for an FDA enterprise system, Congress would accelerate reviews and enable data assets to be fully leveraged across the product life cycle.

15. **Leverage FDA data to support innovation and safety.** The FDA holds valuable troves of data from decades of regulatory reviews and post-market monitoring. Stakeholders proposed several ways to make better use of this information to improve oversight and support innovation, including fee-based access models to monetize certain data. For example, the FDA could expand academic access to Sentinel, its active surveillance system for post-market safety. The FDA could create a fee-based platform that allows industry, academics, and others to access aggregated and de-identified data from product submissions. In addition, combining data from the FDA and CMS could dramatically strengthen early detection of safety issues and help inform coverage decisions or label expansions for approved products.

16. **Implement AI-assisted review.**
The FDA is taking steps to adopt AI/ML tools, but capabilities are limited and uneven across the agency. AI could support tasks such as summarizing documents, validating data quality, and checking cross-submission consistency. For example, submissions often arrive as static PDFs, forcing manual processing that introduces errors and delays review, but AI could extract structured data and check for completion. Congress should instruct the FDA to implement AI-assisted review with human-in-the-loop controls, validated models, continuous monitoring, and regular audits. Congress should also establish a dedicated, well-resourced FDA AI task force to accelerate implementation, train FDA reviewers, and coordinate adoption across the FDA. Careful AI implementation would accelerate drug-approval timelines and make staff more efficient.

17. **Harmonize terminology across agencies.**
Center-specific definitions and data fields within the FDA make it difficult to combine and compare regulatory and medical data. For example, the terminology used to describe a cancer diagnosis can either facilitate or hinder comparison between patients.[13] Inconsistencies extend to the NIH and other agencies within the HHS. Congress should direct the HHS to develop a "common terminology service" to provide standardized, centralized definitions across systems, building on the NIH's efforts toward common data elements.[14] Harmonized terminology would support data sharing across the HHS and accelerate the translation of research into needed medical treatments.

18. **Support strong participation in international standard setting.**
Mismatched global standards complicate multi-country regulatory submissions, increasing costs and delaying patient access to new therapies. Congress should direct the FDA to strengthen participation in international standards development. Specifically, the FDA needs dedicated staff to lead International Council for Harmonisation (ICH) working groups. Because international regulatory agencies adopt ICH guidelines as binding, stronger participation would give the United States direct influence on regulatory requirements in other countries, including China. Shared international standards would also reduce duplicative trials and ease multi-country approvals.

Promote Efficient Manufacturing

Ensuring that novel products can be manufactured domestically is a matter of national strategic importance. Conventional, small-molecule medicines are shelf-stable and can be mass-produced overseas. In contrast, cell and gene therapies must be manufactured on-demand or in small batches to be delivered quickly. The United States must enact policies for modernized, domestic manufacturing to support American innovation and safeguard critical supply chains.

19. **Expand risk-based inspections overseas.**
The FDA has already implemented a risk-based approach to inspections, in which inspection history, safety signals, and other factors help the FDA prioritize inspections. The FDA often conducts domestic inspections with little advance notice, but surprise inspections of manufacturers overseas are all but impossible due to international agreements. This leaves domestic manufacturers at a disadvantage. Congress should instruct the FDA to consider options to enforce parity in inspection frequency between domestic and foreign facilities. The FDA should evaluate and consider expansion of its Foreign Unannounced Inspection program pilot to help level the playing field for U.S. manufacturers. Expanding mutual recognition agreements to cover pre-approval inspections would reduce duplication and accelerate approvals. In addition, domestic policy incentives such as fee waivers, exclusivity extensions, and priority inspections would help attract investment back to the United States and rebuild critical development and manufacturing capacity.

20. **Clarify manufacturing requirements.**
The FDA sets manufacturing requirements for products in development and on the market. These Current Good Manufacturing Practice (CGMP) regulations cover issues from the cleanliness of the workspace to potency and purity testing to record keeping. While the FDA does not require full compliance with Good Manufacturing Practice (GMP) for Phase I trials, many developers believe they must comply at this stage. Congress should instruct the FDA to clearly communicate manufacturing requirements and issue a roadmap so that developers are aware of validation requirements. This would help correct the widespread misconception that full GMP compliance is required prior to human trials.

21. Coordinate country of origin labeling.
Under existing law, all products that are imported into the United States must be marked with their country of origin, and the container that reaches the consumer must have this information.[15] U.S. Customs and Border Protection (CBP) is responsible for enforcement at the port. Many FDA-regulated products are shipped in large, multi-unit packages and individual products are not typically marked with their country of origin, even though each product typically includes FDA-approved labeling. Congress should instruct the FDA, CBP, and Federal Trade Commission (FTC) to coordinate enforcement and ensure each individual product is labelled appropriately. This would allow consumers to understand the sources of medical products and consider the country of origin when making purchasing decisions.

22. Expand domestic manufacturing capacity and workforce.
Particularly for emerging companies, the capital investment needed for a stand-alone manufacturing facility can be a major barrier in developing a viable therapy. Even when facilities are available, a fully-trained workforce is needed. Stakeholders discussed a variety of options to address these concerns. For example, Congress could consider opportunities to license private platforms to national labs and to enable entities such as academic medical centers to manufacture emerging products like personalized gene therapies. Stakeholders also discussed the potential for incentives, such as priority reviews, vouchers, or tax incentives, for products manufactured in the United States. These actions would enable more companies to manufacture advanced therapies in the United States and accelerate patient access to innovative treatments.

References

1. https://pmc.ncbi.nlm.nih.gov/articles/PMC12479303/; https://www.bloomberg.com/news/features/2025-07-13/china-drugmakers-catching-up-to-us-big-pharma-with-new-medicine-innovation
2. https://www.fda.gov/media/177540/download
3. https://www.genengnews.com/gen-edge/cell-and-gene-therapy-leaders-tell-fda-believe-in-american-solutions/
4. https://www.fda.gov/media/151712/download
5. https://milkeninstitute.org/content-hub/research-and-reports/reports/future-us-biomedical-research-and-innovation-recommendations-action
6. https://www.fda.gov/patients/learn-about-drug-and-device-approvals/drug-development-process
7. https://grants.nih.gov/policy-and-compliance/policy-topics/human-subjects/single-irb-policy-multi-site-research
8. https://ifp.org/the-case-for-clinical-trial-abundance/
9. https://doi.org/10.1007/s43441-025-00889-6
10. https://www.fda.gov/drugs/development-resources/surrogate-endpoint-resources-drug-and-biologic-development; https://www.fda.gov/drugs/development-resources/clinical-outcome-assessment-compendium
11. https://www.parexel.com/application/files/resources/assets/Australia%20Regulatory%20Market%20Access%20Article_Third%20in%20a%20three-part%20series%20(1).pdf
12. 21 U.S.C. § 355(i)(2)
13. https://www.sciencedirect.com/science/article/pii/S2352914819300450
14. https://cde.nlm.nih.gov/home
15. 19 USC 1304

Acronyms

- AI/ML: artificial intelligence and machine learning
- ARC: Accelerating Rare disease Cures
- CBER: Center for Biologics Evaluation and Research
- CBP: Customs and Border Protection
- CDER: Center for Drug Evaluation and Research
- CDRH: Center for Devices and Radiologic Health
- CGMP: Current Good Manufacturing Practice
- CHIP: Children's Health Insurance Program
- CMS: Centers for Medicare & Medicaid Services
- FDA: Food and Drug Administration
- FFDCA: the Federal Food, Drug, and Cosmetic Act (FFDCA)
- FTC: Federal Trade Commission
- GMP: Good Manufacturing Practice
- HHS: Department of Health and Human Services
- ICH: International Council for Harmonisation
- IRB: Institutional Review Board
- NAMs: new approach methodologies
- NIH: National Institutes of Health
- NSCEB: National Security Commission on Emerging Biotechnology
- OHRP: Office for Human Research Protections
- OIG: Office of the Inspector General
- RDEP: Rare Disease Evidence Principles
- START: Support for clinical Trials Advancing Rare disease Therapeutics

Staff at the National Security Commission on Emerging Biotechnology authored this paper with input from the NSCEB Commissioners. The content and recommendations of this paper do not necessarily represent positions officially adopted by the NSCEB.